

DIRECTIONS

COMMUNITY NEWSLETTER | SPRING 2022





SHARE YOUR STORY

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



Is there a moment, anecdote, or story that has stood out in memory for you and your family? Share your SMA experiences and photos with us by emailing stories@curesma.org!

6 Page Community Mailbag

15 Page Original Articles

20 Page Community Spotlights

22 Page Cure SMA Updates

38 Page Event Updates

The production of this newsletter was supported
by grants from Biogen and Genentech.

As we enter a new season, we are optimistic about getting back to many of our in-person activities, which will help us build on our recent progress. In 2021, our community made measurable progress in expanding access to new treatments, implementing newborn screening, and establishing the Care Center Network, amongst other areas.

The Cure SMA Care Center Network is the centerpiece of our efforts to address the changing landscape of SMA and improve care and treatment access for all. Each of our Care Centers contributes valuable data to the SMA Clinical Data Registry, which help to identify the best care practices. Our goal is to continue to grow the Care Center Network both geographically and in the number of SMA patients at each center in the year ahead, enabling Cure SMA to capture real-world data that represents the SMA community and to then provide valuable information to SMA individuals and families.

Another key goal for this year and a cornerstone of Cure SMA is advancing new basic research. Cure SMA has been investing in basic research for decades, which has been the starting point for the treatments that we now have. But we are not done yet. This year, we are focusing on funding new early-stage research to help us learn more about non-SMN targeted therapies and combination treatment approaches. Without basic research, the SMA drug pipeline will not continue to grow and diversify. Basic research is our investment in continued drug development and future improvements for SMA.

Together, the expansion of the Care Center Network and funding new basic research will help us continue to meet the needs of our community.

Thank you for your support!



Kenneth Hobby
President, Cure SMA

MOVING AHEAD

Evrysdi
keeps working
so we can
keep reaching



What is Evrysdi?

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

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

Important Safety Information

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

- are pregnant or plan to become pregnant, as Evrysdi may harm your unborn baby. Ask your healthcare provider for advice before taking this medicine
- are a woman who can become pregnant:
 - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
- are an adult male. Evrysdi may affect a man's ability to have children (fertility). Ask a healthcare provider for advice before taking this medicine
- are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby

Tell your healthcare provider about all the medicines you take.

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

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

The most common side effects of Evrysdi include:

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

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

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

Please see accompanying brief summary for additional Important Safety Information.

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

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 **Evrysdi**[®]
risdiplam 60 mg
powder for oral solution

LIFE IN ACTION

Patient Information EVRYSDI® [ev-RIZ-dee] (risdiplam) for oral solution	
What is EVRYSDI? <ul style="list-style-type: none"> EVRYSDI is a prescription medicine used to treat spinal muscular atrophy (SMA) in adults and children 2 months of age and older. It is not known if EVRYSDI is safe and effective in children under 2 months of age. 	Reusable Oral Syringes <ul style="list-style-type: none"> Your pharmacist will provide you with the reusable oral syringes that are needed for taking your medicine and explain how to use them. Wash the syringes per instructions after use. Do not throw them away. Use the reusable oral syringes provided by your pharmacist (you should receive 2 identical oral syringes) to measure your or your child's dose of EVRYSDI, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringes are lost or damaged. Once 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.
Before taking EVRYSDI, tell your healthcare provider about all of your medical conditions, including if you: <ul style="list-style-type: none"> are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. EVRYSDI may harm your unborn baby. are a woman who can become pregnant: <ul style="list-style-type: none"> Before you start your treatment with EVRYSDI, your healthcare provider may test you for pregnancy. Because EVRYSDI may harm your unborn baby, you and your healthcare provider will decide if taking EVRYSDI is right for you during this time. Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping EVRYSDI. are an adult male planning to have children: EVRYSDI may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice. are breastfeeding or plan to breastfeed. It is not known if EVRYSDI passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with EVRYSDI. 	What are the possible side effects of EVRYSDI? The most common side effects of EVRYSDI include: <ul style="list-style-type: none"> For later-onset SMA: <ul style="list-style-type: none"> fever diarrhea rash For infantile-onset SMA: <ul style="list-style-type: none"> fever runny nose, sneezing, sore throat, and cough (upper respiratory infection) constipation diarrhea lung infection vomiting rash <p>These are not all of the possible side effects of EVRYSDI. For more information, ask your healthcare provider or pharmacist.</p> <p>Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.</p>
Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.	
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. <ul style="list-style-type: none"> 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. 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. 	How should I store EVRYSDI? <ul style="list-style-type: none"> Store EVRYSDI in the refrigerator between 36°F to 46°F (2°C to 8°C). Do not freeze. Keep EVRYSDI in an upright position in the original amber bottle to protect from light. Throw away (discard) any unused portion of EVRYSDI 64 days after it is mixed by the pharmacist (constitution). Please see the Discard After date written on the bottle label. (See the Instructions for Use that comes with EVRYSDI).
Taking EVRYSDI <ul style="list-style-type: none"> 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. <ul style="list-style-type: none"> 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: <ul style="list-style-type: none"> 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. 	Keep EVRYSDI and all medicines out of the reach of children.
	General information about the safe and effective use of EVRYSDI. Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use EVRYSDI for a condition for which it was not prescribed. Do not give EVRYSDI to other people, even if they have the same symptoms you have. It may harm them. You can ask your pharmacist or healthcare provider for information about EVRYSDI that is written for health professionals.
	What are the ingredients in EVRYSDI? Active ingredient: risdiplam Inactive ingredients: ascorbic acid, disodium edetate dihydrate, isomalt, mannitol, polyethylene glycol 6000, sodium benzoate, strawberry flavor, sucralose, and tartaric acid.
	 <p>Genentech A Member of the Roche Group</p> <p>EVRYSDI® (risdiplam)</p> <p>Distributed by:</p> <div> <div> Genentech, Inc. A Member of the Roche Group 1 DNA Way South San Francisco, CA 94080-4990 </div> <div> EVRYSDI is a registered trademark of Genentech, Inc. M-US-00007143(v2.0) ©2021 Genentech, Inc. All rights reserved. </div> </div> <p>For more information, go to www.EVRYSDI.com or call 1-833-387-9734.</p>

Letters from the Community



Thank you very much for the care package. Michaela loves all the toys. And we are really grateful to you for all your help and support!!

~ Blanka S.



Thank you very much for the care packages. I finally had a chance to open them and discovered all these lovely goodies for them. Thank you for all the info too.

~ MaryLou G.

Thank you so much for all the SMA gifts, I really appreciate it and Ryu will truly love this, I am so grateful for all support, this is the best ever that Ryu had received from his SMA journey.

~ John Michael K.



I wanted to thank you and the Biogen funders for these generous and helpful support packages and COVID package. These products have been so helpful especially the Amazon Echo and the power switch controls.

Last week, I was working and I leaned over to pick up a piece of paper when I lost my balance and could not get back up or reach my cell phone. Thank goodness for the Echo as I was able to call for help immediately using It.

Again, I just wanted to extend my thank you for the packages.

~ Krista F.



We received our care package late last week. It is amazing and heartwarming to think of all the people/families who have worked and donated to make this happen. We greatly appreciate this gesture, resources, and items.

~ The Newsom Family



We want to thank you because without Cure SMA, it wouldn't be possible for Josué to have all these nice things. I'm attaching a photo with all the gifts we received in the package. Josué will be very happy playing with everything! We are excited to see how this helps with his development!

Thank you Cure SMA so much again for the support you have provided and continue to provide. We appreciate everything you do for families like us!

Thank you so much for your support!!!

~ Cristina V.

Good afternoon!
I wanted to say thanks so much for the care packages and for the car bed for my son little Michael. I received them this afternoon and after opening up and looking through everything my eyes were filled with happy tears because I don't feel alone, and he has some wonderful things to come home to. You'll never understand how much this means to me and how grateful I am. May God continue to bless you as you have been a great blessing to us.

~ Kansas Kyle



We received the wonderful care package of the medical supplies, binder of information and also the wonderful box of toys and blankets for our baby (which was so very thoughtful).

Thank you so much for all the help and support you provided, and I look forward to becoming an active member with the SMA community.

~ Sarah S.

Your timing is impeccable, this welcome box arrived on a pretty rough day for my Mom and Dad. This box provided a welcome distraction for our family, especially my 3 year old brother! Thank you again for the warm welcome to the cure SMA family!

~ Quinton B. (Kerrie, Brian, & Brother Weston send a big THANK YOU too!)



I have to tell you how very nice your packages were that you sent, I especially love the power outlets that come with a remote, I never thought easy it could be to turn something on and off. You have sent some very considerate products. The Wi-Fi plugins will also come in handy in my woodworking shop when I want to turn tools on and off. I hope you're all staying safe, happy holidays and happy new year and thank you again.

~ Jacobi W.

Thanks, for the new support package you sent me. The microphone will be handy for all these virtual visits we have now due to covid. The smart plugs make it easier for me to turn on my lamps and the kitchen utensils are handy also! You guys are awesome.

~ Jeff V.



We were welcomed by two huge boxes when we got home from church last night. I have two excited and very thankful kids. Thanks so much for this sweet gesture.

~ Machael C.

Thank you all so much for our care package! This means so much to our family after such a difficult year. Regan loves her play sink! Her brother loved playing with her too. We're still exploring & enjoying the package & it's truly made our holidays brighter. I continue to be amazed at the support in this wonderful community. Thank you, Cure SMA, & God bless!

~ Rachel G.



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

Recognize the symptoms and save a life!



EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

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



SMArtMoves.CureSMA.org

Donations



Special thank you to Monica and Tony Perez for collecting all of these wonderful toys to donate to the Newly Diagnosed Care Packages in honor of Bellamay Perez!

Thank you to Michael and Miriam Galas for donating these handmade quilts for our Newly Diagnosed Care Packages in honor of their granddaughter, Tilly.



Thank you to the US Medical Affairs team at Novartis Gene Therapy for donating these awesome items for the Newly Diagnosed Care Packages.

Cure SMA was thrilled to receive a recent donation of Cabbage Patch Fantasy Dolls to our Newly Diagnosed Care Packages in honor of what would be Liv Harlow McDonald's 5th birthday on March 7th. This generous donation was made by Liv's grandparents Debbie and Richard Butler.



Cure SMA was thrilled to receive a shipment of Play-Doh play kits donated in honor of Everett Wigington's 1st birthday! While celebrating her 1st birthday in February, Everett's family asked for these Play-Doh kits in lieu of gifts so they could be donated to our Newly Diagnosed Care Packages in her honor! Special thanks to the Wigington family and their friends for this wonderful donation!



The McIntosh Family



Weston Cook



The Copeland Family



The Johnson Family



The Vaudry Family



The Bird Family



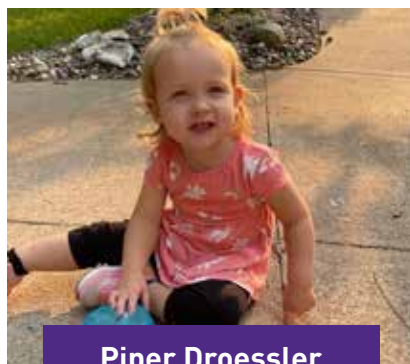
Jack Resendez



Seda Karakaya



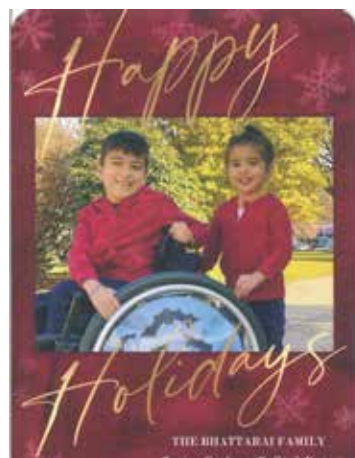
Aniya Porter



Piper Droessler



The McHale Family



Bodhi and Devi Bhattari



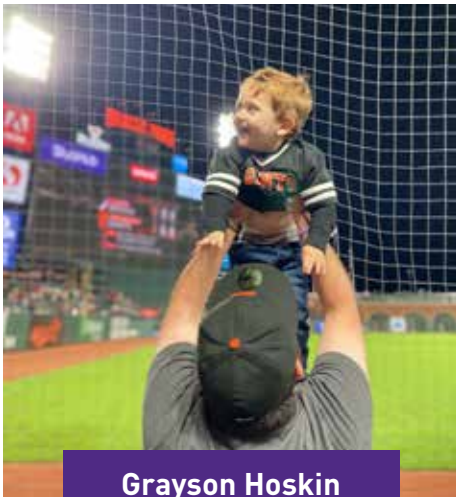
Jonathan Lasko



Blanka, Vanessa, and
Michaela Stiborkova



Joey Smith



Grayson Hoskin



The Eide Family



The Guagliardo Family



The Turner Family



The Tomko Family



SHARE

YOUR PAST CONFERENCE EXPERIENCE

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

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



Tag us on social #2022SMAConference



ANNUAL SMA CONFERENCE

JUNE 16-19, 2022 | ANAHEIM, CA

Every year, Cure SMA sponsors a conference to bring together the leading SMA researchers and clinicians, as well as individuals with SMA and their families. Cure SMA has been hosting the Annual SMA Conference since 1988. The weekend is filled with a wide variety of workshops, a family friendly SMA research poster session, a memorable children's program, a meet and greet with a family fun fest, teen and adult social activities, a Family Movie Night, and an Evening at the Park. There are so many opportunities to connect, interact, and receive first-hand updates from SMA researchers.

We look forward to reuniting as a community at this conference. As always, the Annual SMA Conference and the SMA Research & Clinical Care Meeting run alongside each other. This is the largest conference in the world for those with SMA, those involved in providing support, and those who care for people with SMA. There is no other program like it!

The interactions between the families, adults with SMA, researchers, and clinicians at this conference are extremely special. The Annual SMA Conference also provides children an opportunity to make new friends and have a great time. We are expecting record attendance of well over 2,500 attendees. Cure SMA will also be offering an array of workshops as well as networking and social events for adults with SMA. These will include an adults with SMA social as well as an adults with SMA lounge, which will provide a relaxed space to connect with friends away from the busy conference. Both of these programs have been generously sponsored by Biogen. Disneyland Hotel, Disney's Paradise Pier Hotel, and Disney's Grand Californian Hotel & Spa in Anaheim, California have been carefully selected to meet the needs of the SMA Community for the 2022 Annual SMA Conference.

For any questions about the Annual SMA Conference, please email conference@curesma.org.

Annual SMA Conference Goals

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

Thank You to Our Presenting Sponsors





CURE SMA TEEN AND ADULT INDEPENDENCE ASSISTANCE PACKAGE

This new Teen & Adult Independence Assistance Package—created based on feedback from those who received the first Teen & Adult Support Package—is filled with additional helpful items that were not previously sent to teens and adults in the SMA community.

New items include:

- Logitech Blue Snowball Microphone
- Wi-Fi Smart Plugs
- Travel UV Sanitizing Wand
- Universal Cup Holder
- Telescopic Metal Straws
- Jar Opener with Base Pad
- Multi Kitchen Tools Set



If you have not already received this package, please visit
www.curesma.org/IndependencePackage to request one at no charge.

Cure SMA wishes to thank our donors and supporters who make programs like this possible. Special thanks to Biogen for funding a generous grant which supported this effort.

Please note, this is a supplemental package from the original Teen & Adult Support Package that was launched in 2018, so please apply if you have not yet received the items listed above.



HOW THE SMA COMMUNITY UTILIZES ASSISTIVE TECHNOLOGY

Assistive Technology (AT) devices and technologies help an individual with SMA maintain or improve everyday function that is critical to their independence and community living.

In addition to the recent advocacy issue survey in which the SMA community identified technology as a top priority, Cure SMA also conducted the “SMA Teen and Young Adult Clinical Meaningfulness Survey”. Teens and young adults with SMA created videos and shared their strategies in navigating challenges. Challenges with socialization due to accessibility concerns and activity limitations was an area of meaningfulness described by many. With this in mind, Cure SMA began a series of support packages aimed at providing tools for independent living to our teen and adult community.

Because I can only use my left thumb and a switch to control my computer, I must utilize assistive technology to do everything. My workplace bought me a GoPro camera for when my team is out in the field after a disaster so I can go with them. I bought a Beam+ Robot before I actually started working there and now it lives in my office so I can attend meetings and such. If you haven't seen one, it's like a big FaceTime on wheels. ~ Kim Hill



Below are some common Assistive Technology devices, technologies, and platforms utilized by the SMA community. They provide home modification options to help make a residence more accessible. Logitech Blue Snowball Microphone: Amplifies soft voices and offers crystal clear audio for communicating, creating, and recording, including for Skype, Messages, FaceTime, etc. Creates studio-quality recordings with Blue Microphone's Snowball, a USB microphone with exceptional sound-capture capabilities. Also includes Plug and Play for PC and Mac. Snowball iCE requires no drivers.

Wi-Fi Smart Plugs: Using the app on your smartphone, instantly controls devices connected to a smart plug wherever you have internet. It can be controlled with voice command through Alexa or Google Home Assistant.

Etekcity Remote Control Outlet Switch Kit: Remote control outlets that work from as far as 100 feet and great for switching on/off hard-to-reach appliances and devices. Operates household appliances from across rooms and is a great assistant for those with SMA.

Eyegaze technology: Generate speech by using eye movement. Allows people to talk with friends on social media, check email, listen to music, podcasts, etc. This technology also integrates with email systems, social media, entertainment systems, and more via Bluetooth or wifi connectivity.

Responder PHR (Personal Health Record) package: In emergency situations, assistive technology can be lifesaving. Through a Responder PHR (Personal Health Record) package, medical personnel will have immediate access to a person's medical records stored online, whether the person is able to communicate for themselves or not. The Responder PHR system also serves as an organizational tool and allows the user to input personal information and previous health records into categories.

LOCAL TODDLER'S FAMILY ADVOCATES RARE DISEASE, NOW ON OKLA. NEWBORN SCREENING LIST

CADDO, Okla. (KXII) - A local 3-year-old is learning to take a stand, on his own, as an advocate of the rare disease he's battled since he was born.

Caddo's Brady Clark was diagnosed with Spinal Muscular Atrophy at 13-months-old. He just turned 3 in June and he's got all the spunk and personality to fit in his little shoes. And his walker certainly doesn't hold him back from running and playing with his friends.

"When Brady was 9-months-old he had met all of his typical milestones to that point. We crawled on time, we sat up on time, we were doing everything perfectly on time," said Nicole Clark, mother of Brady.

Around 10 months, his parents Nicole and Tyler found him progressing less.

"His crawl had turned into more of a swing. He was dragging his legs when he was crawling," said Clark.

He was diagnosed at 13 months with Spinal Muscular Atrophy type 2, meaning his fine motor skills typically learned at his age weren't developing. The Clarks chose a treatment plan a day after diagnosis, and Brady was treated a month later.

"It does not mean SMA went away it just means all the regression that we were seeing and were continuing to see every day was going to stop," said Clark.

"My name is Brady Dean," said Brady, proudly. Brady goes to therapy regularly to bounce back from the disease's regression, and he notices his improvement.

"We're starting to see him making movements and doing things that, even though he's 3, he's like, 'look mom I'm walking,' or, 'look mom I'm standing on my own.' And he notices his friends, he's like, 'my friends can walk on their own, so can I,'" said Clark.

The Clarks share Brady's story as a way to raise awareness of the disease for new parents, and as a way to teach others that Brady just needs a little extra help to get around.

"His friends, like if he can't get to his walker they bring it to him. If he can't get in his chair at church his little 3-year-old buddy will help him get to his chair. It's amazing to see the acceptance," said Clark.

The Oklahoma state mandatory list for newborn screenings recently added Spinal Muscular Atrophy to now be able to catch it within days of birth to prevent the regression from ever starting.

SMA is also on the Texas mandatory newborn screening list.

"So they don't have to say what's wrong with my baby, I don't know what's wrong. They can say at the hospital your child has this, but it's okay, there's treatments, we're going to move forward," said Clark.

September is Newborn Screening Awareness Month and while the month may almost be over, Brady is always excited to show everyone how fast he can run with his walker, just like any other kid.

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Originally Published by KXII and Emily Tabar, on Sep. 29, 2021:

www.kxii.com/2021/09/30/local-toddlers-family-advocates-rare-disease-now-okla-newborn-screening-list/



LifeVac Home Kit

The LifeVac is a non-powered, non-invasive, single-use only airway clearance device developed for resuscitating a victim with an airway obstruction when current choking protocols have been followed without success.

This support program is offered to all affected individuals with SMA to help in the event of a choking emergency. Through this program, a LifeVac Home Kit is provided to any individual with SMA who requests one.

The LifeVac Home Kit includes:

- (1) LifeVac non-powered, non-invasive airway clearance device
- (1) Adult mask
- (1) Pediatric mask
- (1) Practice mask

If you are an individual with SMA or a parent/legal guardian of a child with SMA, and are interested in receiving the LifeVac Home Kit, please visit www.curesma.org/LifeVac to request one at no charge.

Please note, you must have a confirmed SMA diagnosis, live within the U.S., and have not already received a LifeVac Home Kit through this program to qualify.



Donor Spotlight:

Robin and Kevin Mussler

Robin and Kevin Mussler have been dedicated supporters of Cure SMA since 2019 and became members of The Purple Circle, Cure SMA's giving society, in 2021. Their SMA journey began in 2018 when their niece, Lucy, was diagnosed at 10 weeks old. No one in their family had ever heard of SMA and they quickly tried to learn as much as possible. They were unsure what the future held for Lucy, but fast forward four years and today Robin says, "With the combination of advanced medicine, therapies, an amazing doctor, community of family and friends, and two amazing parents, Lucy is thriving! Thankfully, Lucy is making amazing strides physically and mentally."

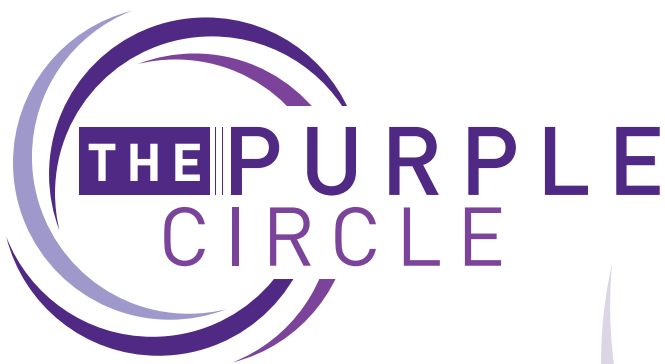
Lucy is not only smart, hilarious, and beautiful, she is a fighter! Nothing stops her.

~ Robin Mussler, Aunt of Lucy

Robin and Kevin have been blessed over the years to donate to various charities and foundations. In reading and learning more about SMA, they knew right away they wanted to support Cure SMA's mission. They felt the research completed in the last two decades has been truly remarkable. Robin says, "We could not be happier to support and give back to this organization."

In addition to giving monetarily, they were able to participate in the 2019 Walk-N-Roll in Louisville, KY. It was a great opportunity for them to meet other dedicated members of the SMA community and spread awareness for SMA.

Robin and Kevin would like to challenge you to get involved with Cure SMA. Robin states, "Whether it's through donating financially, helping raise awareness, or participating in annual events, it will be a truly rewarding experience to know you are a part of something special."



GIVE GENEROUSLY. AND CHANGE THE FUTURE OF SMA



“

Our Purple Circle members are those who join us actively as we identify and aggressively seek new treatments, care options and support services to benefit people with SMA. Their unwavering focus and active participation will allow us to deliver the best quality of life for all those living with SMA.

~ Kenneth Hobby
President, Cure SMA

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Members of The Purple Circle are helping lead Cure SMA's mission to fund critical SMA research and community support programs.

- This group of dedicated supporters help accelerate our progress and create exciting new opportunities that will improve the quality of life for people with SMA, and their families.
- Membership includes unique benefits, such as exclusive quarterly updates on SMA progress, invitations to special events, and more.
- Purple Circle donors lead the SMA community by annually giving \$1,000 or more.

Become a Purple Circle member by donating today!



Online: www.curesma.org/donate

By Mail: 925 Busse Road, Elk Grove Village, IL 60007

Questions about The Purple Circle or your donation? Please email us at ThePurpleCircle@curesma.org.

Advocacy Update

ADVOCATING FOR THE TECHNOLOGY NEEDS OF PEOPLE WITH SMA

Assistive technology (AT) devices and services, such as a power wheelchair or a high-tech communications device that tracks eye gaze, are improving the lives of children and adults with SMA. AT devices and technologies help an individual with SMA maintain or improve everyday function that is critical to their independence and community living. The SMA community identified technology as a top priority in Cure SMA's recent advocacy issue survey. Cure SMA advocates with and for the SMA community on technologies issues as part of the organization's Greater Independence agenda.

Access to Assistive Technology:

Cure SMA is working in Congress to extend and fully fund the AT State Grant Program that helps people with disabilities in every state to access and acquire AT. Cure SMA supports bipartisan legislation (21st Century Assistive Technology Act) that would extend the AT State Grant Program through 2026, providing certainty that the state AT expertise and assistance will continue for people with SMA.

Research and Coverage of Assistive Technology:

Cure SMA is urging federal decisionmakers, including Congress and the Centers for Medicare & Medicaid Services (CMS), to invest in research into new technologies to assist people with disabilities and rare disease and to ensure these emerging technologies and devices are recognized and covered through Medicare and Medicaid.

OUR ADVOCACY PARTNERS ON TECHNOLOGY

Cure SMA works on technology issues on the federal level through key national coalitions, including the Consortium for Citizens with Disabilities and the ITEM Coalition.



The Consortium for Citizens with Disabilities (CCD) is the largest coalition of national organizations that advocate for children and adults with disabilities. CCD member organizations include Cure SMA, American Association of People with Disabilities, Christopher and Dana Reeve Foundation, and Paralyzed Veterans of America.



The ITEM Coalition is devoted to raising awareness and building support for policies that will enhance access to assistive devices, technologies, and related services for people with disabilities and chronic conditions. ITEM Coalition member organizations include Cure SMA, The ALS Association, American Council of the Blind, and the Spina Bifida Association.

DID YOU KNOW?

- Access to technology was identified by the SMA community as a top priority in Cure SMA's 2021 Advocacy Issues Survey.
- Assistive technology (AT) is defined in federal law as any item, piece of equipment, or product system that is used to increase, maintain, or improve functional capabilities of individuals with disabilities. Examples include mobility aides, modified driving controls, voice recognition programs, screen readers, and adaptive pencils and utensils.
- Every state and U.S. territory operates a statewide AT program to help individuals with disabilities access and acquire AT devices through device demonstration, device lending, state financing, and device reuse activities. Find the AT program in your state through the National Assistive Technology Act Technical Assistance and Training Center (www.at3center.net/).
- Federal law, including the Americans with Disabilities Act and Section 508 of the Rehabilitation Act, require federal agencies and certain businesses to make their websites and web content accessible for people with disabilities.



CURE SMA'S GREATER INDEPENDENCE LEGISLATIVE AGENDA

From research into SMA treatments to newborn screening of SMA, Cure SMA has advocated with and for the SMA community on issues important to children and adults with SMA. Our new legislative agenda is focused on helping individuals with SMA access the technology, supports, and programs they may need to excel at school, thrive in the workplace, and participate in the community.

NEWBORN SCREENING PROGRESS CONTINUES IN 2022!

2022 is off to a strong start for state implementation of newborn screening of SMA. Since January 1, 2022, six more states have started to screen for SMA, including Louisiana (January 1), Arizona (January 3), New Jersey (February 1), Idaho (February 1), Alabama (February 14), and Virginia (March 16). As of the printing of this publication, 44 states screen for SMA, covering just under 95% of all babies born in the United States. This progress is the result of Cure SMA advocates participating in meetings, telling their stories, and urging action. Thank you for your great advocacy!

THE 5 PILLARS OF CURE SMA'S GREATER INDEPENDENCE AGENDA ARE:

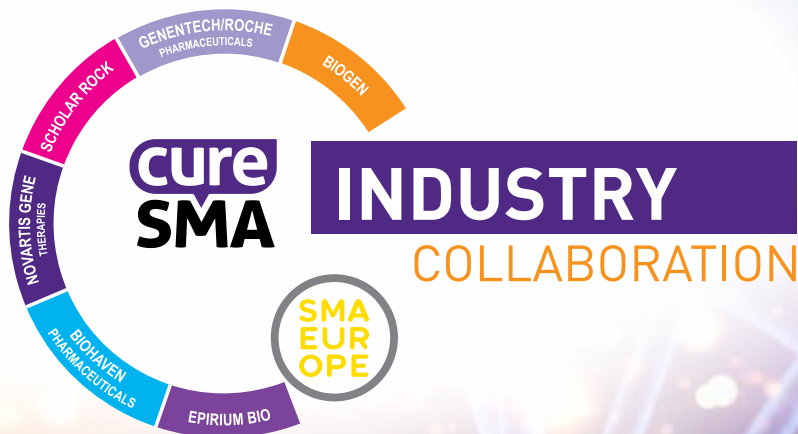
1. **Access to Healthcare and Treatment:** Promote the development of and timely access to SMA treatments, equipment, and other health-related services.
2. **Transition to Adulthood:** Improve the transition from youth to adulthood for individuals with SMA and their families.
3. **Community Living:**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



NEW CURE SMA ADVOCACY ACTION CENTER

Cure SMA recently launched a new, user-friendly technology tool to help individuals with SMA and their families advocate for assistive technology and other issues important to the SMA community. Cure SMA upgraded its online action alert system that allows Cure SMA advocates and supporters to easily contact their Members of Congress and other key decision makers about SMA community priorities. Check out the new action center and sign up to be an advocate at: www.curesma.org/advocacy/actioncenter.

Learn more about Cure SMA Advocacy and ways you can help advance the Greater Independence agenda at www.curesma.org/advocacy/.



Research Update

UPDATE ON THE CURE SMA INDUSTRY COLLABORATION

Cure SMA is pleased to announce the launch of an expanded Phase 7 of our SMA Industry Collaboration. The SMA Industry Collaboration is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations, to share information, ideas, and data to benefit the broader SMA community. **The goals for this next phase of the SMA Industry Collaboration include:**

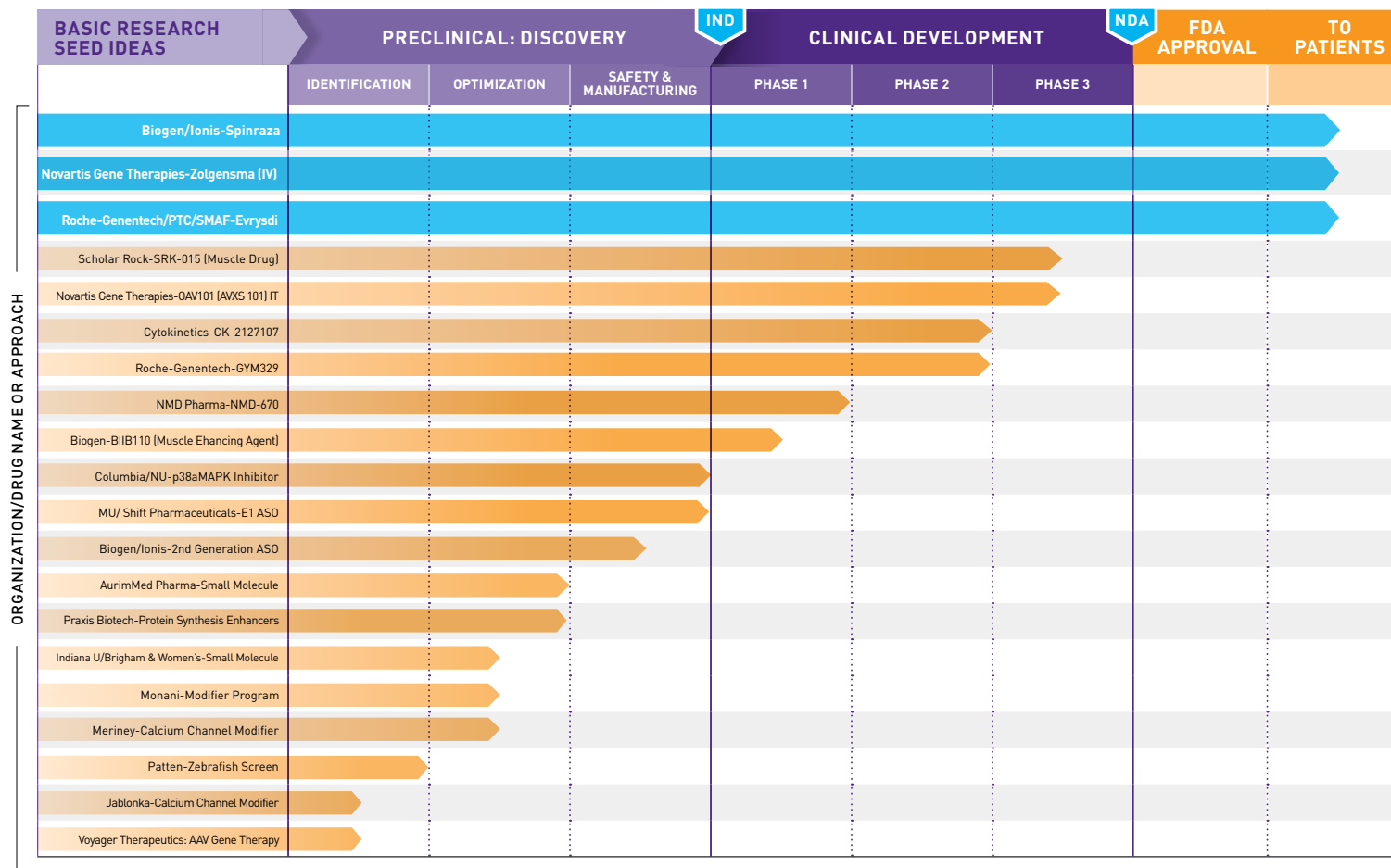
- Distribution of a benefit risk survey to assess the specific risks affected individuals and families would be willing to accept in exchange for certain treatment benefits
- Development and publication of an updated Voice of the Patient report
- Development and refinement of outcome measures
- Distribution of a survey to acquire demographic information from individuals and families affected by SMA
- Organizing focus groups to understand and identify disparities and perceived gaps in SMA care, treatment, and clinical trial research access

- Evaluation of five years of data captured via the Annual Community Update Survey to better understand changes in the SMA disease experience and patient needs
- Development of educational materials to highlight the impact of copy number and disease status at time of treatment, on health outcomes for affected individuals

About the Cure SMA Industry Collaboration

The Cure SMA Industry Collaboration was established in 2016 to leverage the experience, expertise, and resources of pharmaceutical, biotechnology companies, and other nonprofit organizations involved in the development of spinal muscular atrophy (SMA) therapeutics to more effectively address a range of scientific, clinical, and regulatory challenges. It is currently comprised of our partners at Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, and SMA Europe. Additionally, the work of the Collaboration is supported by funds provided by Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, and Epirium Bio.

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



IND = Investigational New Drug NDA = New Drug Application Last updated: November 2021

CURRENTLY RECRUITING SMA CLINICAL TRIALS:

SMART: A Phase 3b, Open-label, Single-arm, Single-dose, Multicenter Study to Evaluate the Safety, Tolerability and Efficacy of Gene Replacement Therapy With Intravenous OAV101 (AVXS-101) in Pediatric Patients With Spinal Muscular Atrophy (SMA)

Ages Eligible: Up to 17 years

ClinicalTrials.gov Identifier: NCT04851873

ASCEND: A Phase 3b Study to Evaluate Higher Dose Nusinersen (BIIB058) in Patients With Spinal Muscular Atrophy Previously Treated With Risdiplam

Ages Eligible for Study: 5 Years to 39 Years

ClinicalTrials.gov Identifier: NCT05067790

STEER: A Randomized, Sham-controlled, Double-blind Study to Evaluate the Efficacy and Safety of Intrathecal OAV101 in Patients Type 2 Spinal Muscular Atrophy (SMA) Who Are ≥ 2 to < 18 Years of Age, Treatment Naive, Sitting, and Never Ambulatory

Ages Eligible for Study: 2 Years to 17 Years

ClinicalTrials.gov Identifier: NCT05089656

DEVOTE: Escalating Dose and Randomized, Controlled Study of Nusinersen (BIIB058) in Participants With Spinal Muscular Atrophy

Ages Eligible for Study: Child, Adult, Older Adult

ClinicalTrials.gov Identifier: NCT04089566

RESPOND: A Phase 4 Study of Nusinersen (BIIB058) Among Patients With Spinal Muscular Atrophy Who Received Onasemnogene Aporvovec

Ages Eligible for Study: 2 Months to 36 Months

ClinicalTrials.gov Identifier: NCT04488133

RAINBOWFISH: An Open-Label Study of Risdiplam in Infants With Genetically Diagnosed and Presymptomatic Spinal Muscular Atrophy

Ages Eligible for Study: Up to 6 weeks

ClinicalTrials.gov Identifier: NCT03779334

*Trials recruiting as of 3/7/2022

Clinical Care Update

We are excited to share that the Cure SMA Care Center Network is expanding!

The Care Center Network includes 19 pediatric and adult centers across the country. These SMA Care Centers contribute real-world data to the SMA Clinical Data Registry. The Network's goal is to collect diverse SMA patient data in the SMA Registry to produce new evidence-based standards of care for SMA. Currently, 725 individuals with SMA across 19 centers contribute to the SMA Clinical Data Registry.

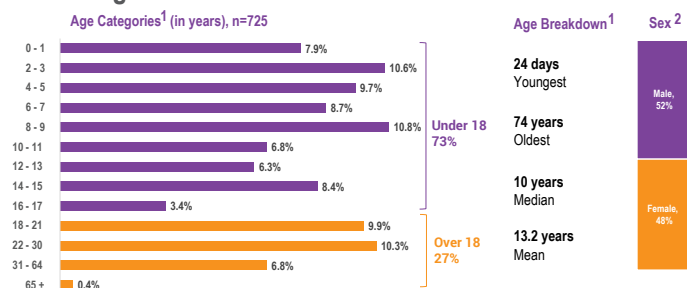
The first Cure SMA Care Center submitted data to the SMA Clinical Data Registry in October 2018. The SMA Clinical Data Registry has grown at a steady pace despite the challenges of COVID-19 pandemic. In 2021, the number of new patients in the SMA Registry increased by 74%.

Care Center Network and Clinical Data Registry Patient Accrual



Cure SMA Clinical Data Registry Demographics

Patient Age Distribution



Data pulled as of 04/20/2022

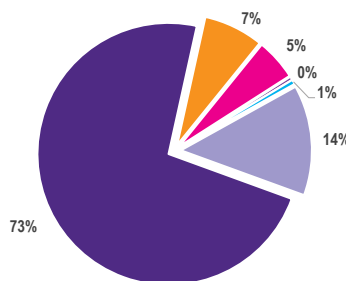
¹ Age calculated at date of data pull

² Sex assigned at birth

Patient Race and Ethnicity

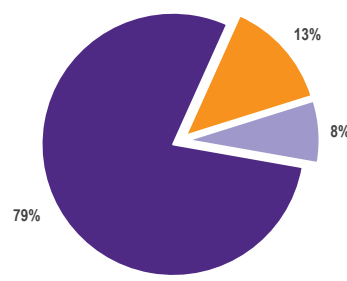
Race Categories, n=705

Note: n= 20 with missing race



Ethnicity, n=722

Note: n= 3 with missing ethnicity



Currently, the SMA Clinical Data Registry has 73% pediatric and 27% adult representation. The SMA Care Center Network will be expanding to 29 centers in 2022. These additional centers will allow for increased adult representation with a target of 50% adults by 2025. The Cure SMA Care Center Network overall goal is to have 50 Care Centers and 2,500 patients with SMA represented.

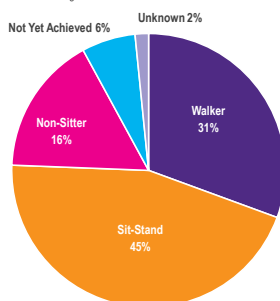
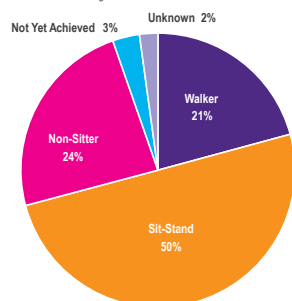
Functional Status¹

Current Functional Status², n=693

Maximum Functional Status, n=693

Note: n= 32 with missing functional status

Note: n= 32 with missing functional status



1 Functional status was pulled from electronic case report forms (eCRF) filled out by clinicians
2 Current at the time of eCRF completion.

Work is underway to update guidelines and standards of care for SMA diagnosis and treatment considerations. As you see in the above graphs, how we talk about SMA type and how we classify SMA is an evolving landscape. Cure SMA has convened healthcare professionals from the US and Europe to discuss updates to SMA diagnosis standards and how they are thinking about SMA treatment when considering the FDA-approved treatments for their patients. Publication is planned for later this year with community focused companion documents to follow.

We thank the SMA community for your support and participation in the SMA Clinical Data Registry. We thank the Care Center Network for their commitment and contributions. We acknowledge the Oscar G. and Elsa S. Mayer Family Foundation and an endowment from Bill and Susan Orr and the Tyler William Orr Memorial Fund for their support. We acknowledge the Cure SMA Real World Evidence Collaboration for support of this work.

About the Cure SMA Real World Evidence Collaboration

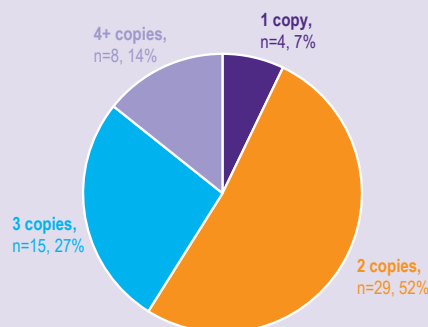
The Cure SMA Real World Evidence Collaboration was established to leverage the experience, expertise and resources of pharmaceutical and biotechnology companies and nonprofit organizations involved in development of SMA therapeutics to guide the future direction of real world evidence collection and use in SMA. Current members include Biogen, Novartis Gene Therapies, and Genentech, Inc.

SMA NEWBORN SCREENING REGISTRY UPDATE

The SMA Newborn screening registry is an opportunity for families to share information about the impact of SMA newborn screening. Currently, 57 infants are included.

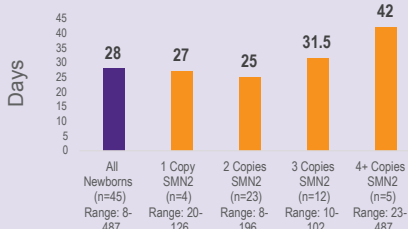
What we have learned from this registry:

SMN2 Copy Number Distribution, n=56



- Over half of infants diagnosed with SMA after newborn screening are reported to have 2 copies of SMN2
- The median time from diagnosis confirmation to receiving the first treatment was 19 days per parent reporting

Median Age at First Treatment, n=45



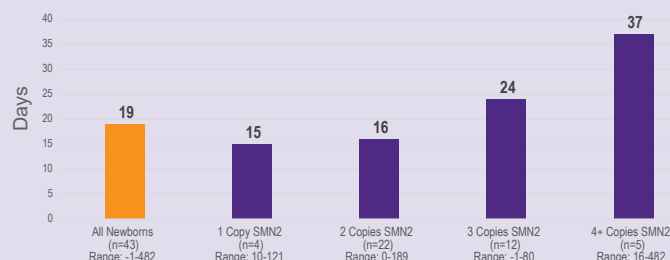
Median age at diagnosis confirmation:

7 days
(range 1-22 days)

Median age at 1st treatment:

28 days
(range 8-487 days)

Median Time from Diagnosis to First Treatment, n=43



Thank you to the SMA community! We acknowledge the Cure SMA Newborn Screening Coalition for support of this work. Members include Novartis Gene Therapies, Genentech, and Biogen.

We invite you to participate in the SMA Newborn Screening Registry so that we can grow our understanding of early diagnosis and treatment impact on newborns with SMA. Thank you!
<https://curesma-enrollment.rexdb.net/caregiver>



Unique New Ways to Support Cure SMA

At Cure SMA, we understand every SMA experience is unique and a diverse SMA community makes us strong. From families who are recently diagnosed through newborn screening and just starting their journey, to adults with SMA who have years of experience navigating the challenges of this disease, our goal is to help everyone with SMA thrive. Also, we have a diverse group of supporters that include healthcare professionals, researchers, sponsors, donors, and many others. They come from all over the country to help move our mission forward and ensure every individual with SMA has the support they need.

No matter who you are or where you live, you have the power to help. This is why Cure SMA offers a wide range of ways for people to contribute their time and money to our cause. Many people already know about our exceptional Walk-n-Roll program, but did you know that planned giving options and monthly donations are other ways you can make an impact? Also, our new Purple Circle giving society is highlighting leaders in our community who provide significant support to further advance progress in SMA.

Please read more about these unique ways to support our mission below:

Purple Circle Giving Society

In 2021, Cure SMA launched The Purple Circle annual giving society, a diverse group of leadership donors who are helping accelerate progress by giving \$1,000 or more. High level support is needed to fund basic research and explore new pathways for SMA treatments, expand the Cure SMA Care Center Network and gather the data needed to develop an evidence-based standard of care for SMA, and make new strategic investments in advocacy at federal, state, and local levels. These dedicated individuals are helping ensure we exceed these goals. Plus, members of The Purple Circle receive great benefits, such as exclusive quarterly updates on our progress, opportunities to connect with Cure SMA Leadership, invitations to special events, and more!

[Learn more at: www.curesma.org/the-purple-circle](http://www.curesma.org/the-purple-circle)

Monthly Giving

Many donors choose to support Cure SMA by making monthly contributions. This giving option allows people to spread their contributions out and provide a higher level of support over a period of time. On average, monthly donors give more than twice as much as one-time donors. By providing recurring support,

you can have a greater impact, such as helping expand our support programs that improve the quality of life for everyone with SMA. Also, monthly giving makes it easy to provide a consistent, predictable source of support that helps our organization plan for the future.

Legacy Giving

Legacy giving, or planned giving, is a great way to make a transformational gift that helps Cure SMA plan ahead and navigate future challenges. These gifts help create new opportunities that otherwise might not have been possible and accelerate progress to new levels. Because of their size and impact, planned gifts provide the funding needed to launch new local programs or expand research projects. Also, there are donor benefits to planned giving, such as financial flexibility and tax incentives. Speak with your financial advisor and Cure SMA about how you can create a legacy for you and your family that makes a lasting impact in the SMA community.

[Learn more at: www.curesma.givingplan.net](http://www.curesma.givingplan.net)

As treatments continue to change the SMA experience, we need your help to ensure that the diverse, evolving needs of the SMA community are met. We all can help change the future of SMA, and by exploring new giving options, you can find unique ways to make a greater impact. The next breakthroughs in SMA are on the horizon and together, we can create a bright future for everyone with SMA. Explore how you can help move our mission forward at:

www.curesma.org/ways-to-give

Questions? Please reach out to one of our dedicated team members:

Brett Kinley | Director, Development – Individual Giving
brett.kinley@curesma.org
(847) 709-6314

Anna Parker | Manager, Development – Individual Giving
anna.parker@curesma.org
(847) 264-1160

Cure SMA continues to be a global leader in groundbreaking research, high-level clinical care, and providing support programs that improve the daily lives of those with SMA. See how you can have direct impact in the SMA community and shape a better future for everyone with SMA.

\$5,000

provides one Panthera Pediatric or Adult Wheelchair for our Equipment Pool. Offering additional mobility for people with SMA, at no cost to the individual.



\$1,000

funds a SMA research project for one week. These projects investigate the biology and cause of SMA to identify new pathways for drug discovery.



\$500

provides one Teen and Adult Independence Assistance Package specifically designed to increase independence with certain tasks they might not have been able to do on their own.



\$100

funds one LifeVac Home Kit, a practical and life-saving item for individuals with SMA. This non-invasive, single-use airway clearance device was developed to resuscitate a victim with an airway obstruction.



\$65

per day provides our Advocacy Action Alert Tool. This allows Cure SMA advocates across the country to easily connect with their elected officials on SMA community policy priorities.



Note: The figures above are taken from the 2020 fiscal year (07/01/2020-06/30/2021). Our complete financial information can be found at www.curesma.org/annual-reports-financials/.

Community Support Update

In-Person Engagement Events

After a year and a half of uncertainty, Cure SMA launched a new program, the Adults with SMA Social, sponsored by Biogen. This is a free program that provided a space for adults with SMA to gather and connect in person. The in-person socials were held at a few locations in 2021:

To see if additional socials will be available in your area, please check out the link: www.curesma.org/virtual-community-engagement-events/

Virtual Engagement Events

Over the past two years, Cure SMA hosted a variety of virtual community events, which allowed the SMA Community to stay engaged, connected, and provided a space to come together. From the always entertaining Adults with SMA Virtual Trivia Nights, to the helpful Wellness Webinars, these virtual programs have been widely attended. If you are interested in learning more about our virtual engagement events and would like to join in, please visit our webpage to register and learn more: www.curesma.org/virtual-community-engagement-events/

ADULTS WITH SMA



Adults with SMA Virtual Evening & Morning Socials

These social events, sponsored by Biogen (Evening Socials) and Genentech (Morning Socials), continue to offer adults with SMA a space to connect with others in the community. If you are looking for a quick catch-up with friends or a brief mental break before a busy day, check out our 30-minute morning socials! Meanwhile, our evening socials offer a longer 90-minute chance to network and meet new adults with SMA in the community.



Adults with SMA Virtual Therapy Program

This program, funded by Genentech and the Dhont Family Foundation, continues to be available to adults with SMA. In this continued time of uncertainty, this program makes virtual therapy more accessible. To get started, visit our virtual engagement website to fill out a request form to determine your eligibility to receive up to three (3) 30-minute live video sessions with a licensed therapist, through the online therapy platform, TalkSpace.



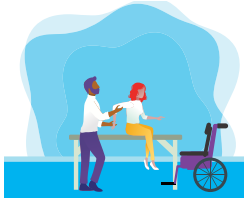
Virtual Therapy Program for Personal Care Assistants of Adults with SMA

In September, Cure SMA launched our new Virtual Therapy Program for Personal Care Assistants of Adults with SMA, sponsored by Genentech. This program provides personal care assistants (PCAs) of adults with SMA three complementary 30-minute live video sessions with a licensed therapist through the online therapy company, Talkspace. The initial request form must be completed by the adult with SMA who is receiving care; to learn more and get started, visit our virtual engagement website!



Career Panel Webinar Series

Interested in learning about career paths, experiences, accommodations, and more from adults with SMA? Check out the next installments of our successful webinar series where panelists discuss their career journeys, experiences, and answer questions from attendees. Thanks to our generous sponsor, Biogen.



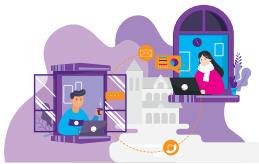
Physical Therapy Webinar Series

This ongoing series, sponsored by Biogen, offers a 1-hour webinar led by physical therapists who are experienced in providing care for both adults and children with SMA. These webinars provide information for exercises at home, and attendees receive a free therapy item upon registration, which the therapists will demonstrate exercises using the item.



Wellness Webinar Series

Cure SMA continued to host our Wellness Webinar, sponsored by Gentech. This series allows the community to take time and focus on our mental health and wellness. These 1-hour webinars include topics on therapeutic yoga, effective communication, and mindfulness that provides valuable tools and strategies to best care for oneself and others. Visit Cure SMA's engagement page to view past sessions and register for future webinars!



College Panel Webinar Series

Another new program Cure SMA is excited to launch is our College Panel Webinar Series! Thanks to the generous sponsorship from Genentech, this series will provide 1-hour webinars in which adults with SMA will share their wide array of college experiences, discussing important topics like independence, support systems, accessibility, travel, living arrangements, and much more.



Teens with SMA Virtual Hangout

Cure SMA has launched a new program specifically for teens with SMA. With the sponsorship of Genentech, these 90-minute sessions invite teens with SMA to gather online in a friendly setting. It is an opportunity to share about their lives, offer support for one another, make new friends, and catch up with old friends online! Visit our website to register!

If you missed any of our webinars over the past year, you can always catch the recorded presentations on the Cure SMA YouTube Channel:



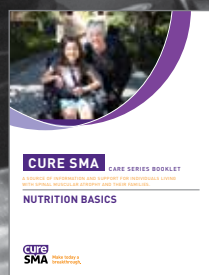
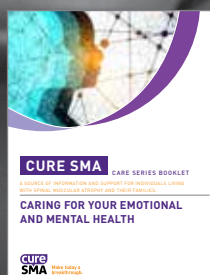
- Wellness Webinar Recordings
- Physical Therapy Recordings
- Career Panel Recordings
- College Panel Recordings
- Virtual Summit of Strength Recordings
- 2021 Virtual SMA Conference Recordings

And more! Visit <https://www.youtube.com/channel/UCECjEl49gy32nz8wWeb2AFQ>



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

Check out the updated Cure SMA Care Series Booklets.



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

**Some books are available in other languages*

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

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

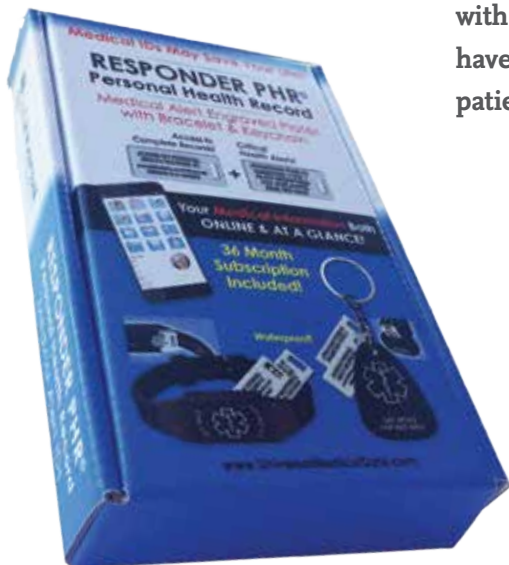


Medical Alert Bracelet Support Program

This support program is offered to all individuals living with spinal muscular atrophy (SMA) in the U.S. to help identify medical needs in case of a medical emergency. Through this program, a Responder PHR (Personal Health Record) with Medical Alert Bracelet and Keychain 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.

The package includes:

- One (1) adjustable 5.5" - 8.5" unisex medical alert bracelet.
- One (1) medical alert keychain.
- Two (2) engraved acrylic plates that contain the URL access and a unique 8-digit member ID number, allowing medical responders access to PHR stored online.
- Two (2) custom engraved acrylic plates that contain the person's most critical medical information to be easily read in any emergency.
- One (1) emergency medical card that can be completed, updated, edited, and printed either online or manually.



In addition to serving to identify medical needs in case of an emergency, the device can also be an organizational tool, allowing the user to input personal information and previous health records.

If you are interested in receiving this package, please visit www.curesma.org/ResponderPHR to request one at no charge.

Please note, you must have a confirmed SMA diagnosis, live within the U.S., and have not already received a Responder PHR with Medical Alert Bracelet and Keychain through this program to qualify. Parents can request on behalf of their child if they are not old enough to apply.

This program is generously funded by a grant from Genentech.



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

These half-day mini conferences host a wide variety of experts who cover topics that are valuable to individuals of all ages and types of SMA, and their caregivers. Each Summit offers a unique agenda of topics ranging from standard of care, research and clinical trials, navigating the special education process, physical therapy, stress management, and much more! Presenters are experts in their fields, with years of experience specific to SMA, and attendees have the opportunity to have their questions answered by these specialists in an intimate setting.

These events provide a tremendous opportunity for networking and building community locally while learning about the latest advances in treatment, care, advocacy, and support. This local engagement highlights Cure SMA's core values and further strengthens the bonds that make our communities so vibrant!

In-person Summits of Strength are now happening.
Be sure to register today and join in!

2022 will be the program's fifth year, after having brought together over 3,000 attendees from across the U.S. at our 54 in-person events, and over 1,600 live viewers virtually through the 22 installments of the Summit of Strength Webinar Series!

For everyone's safety at in-person events, Cure SMA will be executing necessary COVID-19 precautions, per guidelines set forth by the Centers for Disease Control and Prevention (CDC).

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

2022 Summit of Strength Schedule

- February 5th – Atlanta, Georgia Summit of Strength
- February 19th – Orlando, Florida Summit of Strength
- March 5th – Seattle, Washington Summit of Strength
- March 19th – Nashville, Tennessee Summit of Strength
- May 7th – Charleston, South Carolina Summit of Strength
- May 14th – Denver, Colorado Summit of Strength
- July 23rd – Boston Summit of Strength
- July 30th – Minneapolis Summit of Strength
- August 6th – New York City Summit of Strength
- August 13th – Philadelphia Summit of Strength
- August 20th – Austin Summit of Strength
- September 10th – San Francisco Summit of Strength
- September 24th – Baltimore Summit of Strength
- October 15th – Chicago Summit of Strength
- October 22nd – Phoenix Summit of Strength
- October 29th – Hartford Summit of Strength
- November 5th – Miami Summit of Strength
- November 19th – Anaheim Summit of Strength
- December 3rd – New Orleans Summit of Strength
- December 10th – San Antonio Summit of Strength



2021 Tee It Up to Cure SMA Golf Outing in Honor of Ava and Hailey



On September 23, 2021, the Finelli Family successfully hosted their 2021 Tee it Up to Cure SMA Golf Outing in Honor of Ava and Hailey. After having to cancel their 2020 event due to COVID, over 110 golfers and more than 50 dinner guests came out to support Cure SMA at their second event in New Jersey!



Players enjoyed 18 holes of golf with exciting course games, lunch and refreshments provided by generous sponsors, a program with dinner, and over twenty silent auction packages. Thanks to the generosity of the sponsors, players, and community, the 2021 event raised over **\$100,000** for Cure SMA!

Thank you to the Finelli Family for their hard work and dedication to Cure SMA!





WALK-N-ROLL

REGISTER TODAY

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

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

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



Thank You to Our National Premier Sponsors



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

Chapter Updates Section



Southern California & Southern Florida Chapters

Members of the Southern California & Southern Florida Chapters got into the holiday spirit and brought tidings of good cheer to everyone at their annual Holiday Social. Attendees from both chapters had an afternoon filled with tasty holiday treats, some goodies from Santa, and enjoyed getting to see old friends, and make some new ones as well!

Thank you to Fiorenna Stark & Jennifer Miller Smith for planning the South Florida Social and thank you to Autumn Montoya for planning the Southern California Social!



Michigan Chapter

Congratulations to Ian Zurawski, who was awarded his Eagle Scout rank through Boy Scouts of America on December 29, 2020. Ian has been active in Scouting since the first grade.

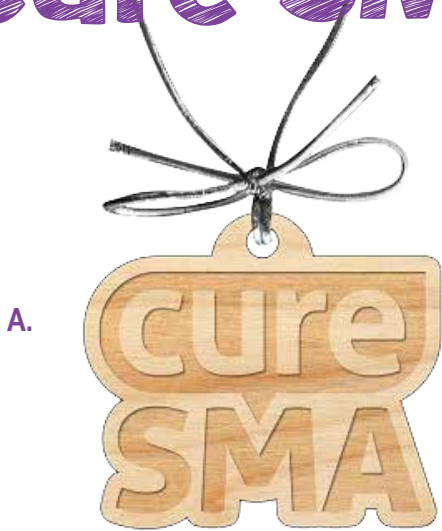
To obtain the rank of Eagle, scouts must complete various other scout ranks, earn a minimum of 21 different merit badges (13 of which are required), demonstrate Scout spirit and leadership within their troop, and complete a community project.

Ian's Eagle Scout project was making different size medical corn bags for Camp Cavell in Lexington, Michigan – where he attends MDA camp each year.

He is also very conscientious and volunteers regularly in the community. These include environmental community cleanups, assists at church with Sunday School program, and Student Government activities.

NEW!

Cure SMA Merchandise



- A. CURE SMA CUSTOM BIRCH ORNAMENT – \$15
- B. CURE SMA LUNCH COOLER – \$20
- C. CURE SMA PUSH POP FIDGET GAME – \$8

- D. CURE SMA STAINLESS STEEL WINE TUMBLER – \$20
- E. CURE SMA LICENSE PLATE FRAME – \$20

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

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



Walk-n-Roll Highlights

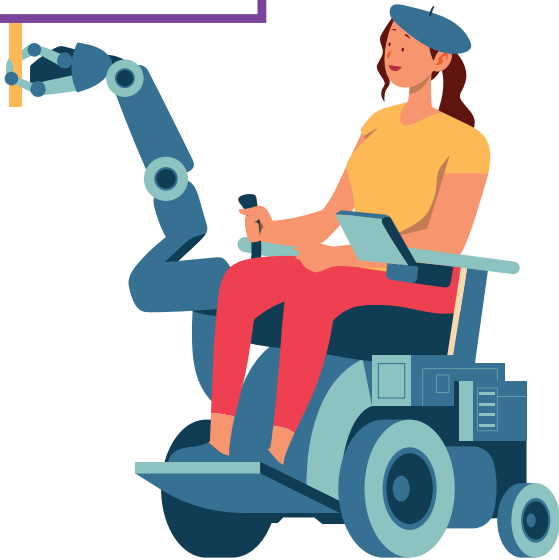


HIGHLIGHTS

of Participants – 1,127

of Virtual Events – 8

of In Person Events – 12





Champions Club

We would also like to thank and congratulate the following Fall Walk-n-Roll participants for being inducted into the 2021 Cure SMA Walk-n-Roll Champions Club! This program recognizes and rewards individual participants who raise at least \$1,000 at their respective Walk-n-Roll. These individuals participants who raise at least \$1,000 at their respective Walk-n-Roll event and are helping to lead the way for fundraising for Cure SMA.

In order to become a Spring 2022 Cure SMA Walk-n-Roll Champions Club member, participate in one of our Spring Walk-n-Roll events that can be found at www.curesma.org/walk-n-roll-program/. Fundraising for this season ends on June 30, 2022.

Cure SMA Walk-n-Roll Champions Club Members

Kevin Schaefer	Danielle Galan	Cathy Barsotti	Hans Eide
Kim Sykora	Erin Courteau	Jean Talburt	Christine Jobanputra
Mitul Patel	Cyndy Henderson	Angel Wolff	Kelly Madsen
Allie Wolfe	Claire Venezia	Kevin Sullivan	Bradley Morgan
Dennis Geissler	Kyle Derkowski	Pam Swenk	James Holmes
Christopher Jankowski	Craig Couture	Peter Koch	

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



twitter.com/cureSMA



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



facebook.com/cureSMA



youtube.com/user/FamiliesofSMA1



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



cure SMA DO IT YOUR WAY FUNDRAISING

is the perfect way to host your own event

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

DIY FUNDRAISING CATEGORIES:



GATHERING FOR GOOD:
COMMUNITY EVENTS



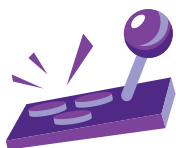
YOUTH FUNDRAISING
INITIATIVES



SLAM DUNK SMA:
ATHLETIC EVENTS



MEANING TO YOUR
MOMENTS: LIFE EVENTS



SUPPORT THROUGH STREAMING:
ESPORTS EVENTS



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

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

Cure SMA's Coast to Coast Challenge is more than a challenge, it's an adventure and community celebration all rolled into one. As always, all are welcome to join every step of the way, literally!

Join Cure SMA's



Challenge!

Start your adventure with Cure SMA, where you can collectively log miles towards our team goal of completing 2,790 miles. That's the distance from New York to California!

Join Cure SMA's Coast to Coast Challenge and help us tackle 2,790 miles as a community. Choose to run, bike, walk, or roll with us and make a difference in the SMA community!



INTERESTED IN SIGNING UP OR LEARNING MORE?

**Reach out to Alyssa Zavislak, Senior Development Manager, Endurance,
alyssa.zavislak@curesma.org**



United for Good – Cure SMA Launches New Fundraising Challenge with Good United

Cure SMA recently partnered with GoodUnited, an organization that helps nonprofits build and execute campaigns to attract new donors, to launch the ‘Run 30 Miles in February Challenge.’ One of Cure SMA’s objectives is to connect with people affected by SMA who are not currently involved with the organization. In partnering with GoodUnited on this campaign, Cure SMA was able to introduce new people to the organization, while also raising important funds to further our mission and vision.

Through the ‘Run 30 Miles in February Challenge,’ more than 2,000 new individuals were made aware of Cure SMA and more than 1,400 became challenge members, including runners and walkers hailing from New York to Hawaii. There was amazing level of camaraderie and encouragement among the fundraisers. Everyone supported one another and cheered each other on through social media. Cure SMA, via GoodUnited, responded to every post and comment, creating a level of trust that encouraged ongoing participant and fundraiser engagement. The runners and walkers put their hearts into this challenge and really pounded the pavement. The total miles run by the group was enough to circle the U.S. nearly three times.

More than 150 of the challenge members became fundraisers and those individuals raised \$42,490 in just one month! To put that in perspective, the total funds raised by the challenge could equate to one year of funding for a patient care coordinator at a Care Center Network site (\$10,000), two Panthera adult wheelchairs (\$5,000 each), 2 Panthera pediatric wheelchairs (\$5,000 each), 12 weeks of SMA research (\$1,000 each), and one flight for an adult with SMA to the Annual SMA Conference (\$500).

TOP CITIES	
Houston, TX	23
New York, NY	22
Chicago, IL	14
Los Angeles, CA	10
Stroudsburg, PA	10

Cure SMA is already gearing up to partner with GoodUnited on additional challenges and campaigns to bring even more new connections and fundraisers to the organization. Thank you to all our challenge participants!

2022 National Partners

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

National Premier Partners



National Platinum Partner



National Gold Partners



National Silver Partners



VISION AND MISSION STATEMENT

Cure SMA leads the way to a world without SMA, the number one genetic cause of death of infants. We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide families the support they need for today.

OUR VALUES

Innovation

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

Balance

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

Collaboration

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

Respect

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

Compassion

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

Determination

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



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

Submissions

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

Change of Address

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

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

Cover photo: Hunter and Gunnar Rhodes, Oakland, MD



Cure SMA
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1.800.886.1762

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Elgin, IL

August is

SMA Awareness Month



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