



# It's more than just a gift...

When you support Cure SMA, you are not just making a donation. You are making an investment in the lives of everyone with SMA and helping to build a hopeful future.

A photograph of a woman with dark hair, wearing a plaid shirt, kneeling and smiling next to a young boy with glasses sitting in a wheelchair. They are in a well-lit indoor space, possibly a conference hall, with a large 'cure SMA' logo in the background.

**\$100 – Funds One Telescoping Ramp**

**\$250 – Helps Send Care Packages to Newly Diagnosed Families, Teens, and Adults**

**\$500 – Provides Scholarships for our Annual SMA Conference**

**\$1,000 – Funds Ongoing Research Projects**



Questions about your donation, matching gifts, or looking for other ways to get involved?  
Please email us at [fundraising@curesma.org](mailto:fundraising@curesma.org)

**Make a donation today! Visit us at [www.curesma.org](http://www.curesma.org) and click the donate button.**

To make a gift by mail, send your donation to:  
Cure SMA 925 Busse Road, Elk Grove Village, IL 60007

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**One Community.** This was a key theme at the 2021 Virtual SMA Conference. Despite another year of not being together in-person for this week of engagement and community-building, it was great to still see individuals with SMA, their families, researchers, industry, and healthcare providers unite with the intent to learn and collaborate as one SMA community. The week hosted nearly 2,500 registrants from over 65 countries, attending more than 50 sessions and social events. We thank everyone who joined us to make the week so special.

We are looking to what is next for our community in the year ahead. This fall, Cure SMA is carefully starting to gather in-person for educational Summit of Strength programs and reuniting communities at Walk-n-Roll events across the country. We see our re-engagement of the community culmination at the 2022 Annual SMA Conference, set to take place in-person in Anaheim, Calif., June 16-19, 2022. We cannot wait!

In the meantime, we are continuing to build our networks that can provide more local support to ensure everyone has access to available treatments and the best possible care. This includes expanding the Cure SMA Care Center Network across the country to share data and insights and improve the standards of care and outcomes for all people with SMA.

Additionally, our next-generation research pipeline continues to advance. The focus is on our basic and translational work, developing symptomatic, muscle-targeting, and combination treatment options.

Finally, we are pushing through the final steps for full implementation of early diagnosis through newborn screening. With just 12 states left to implement for SMA screening, we are now at 85% of all babies born in the U.S. being screened. This is an amazing feat that would not be possible without our amazing local advocates.

We remain proud of the SMA community and all it continues to do together.



**Kenneth Hobby**  
President, Cure SMA

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The production of this newsletter was supported by grants from Biogen, Genentech, and Novartis Gene Therapies.

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FROM THIS  
MOMENT  
COMES  
FROM WITHIN

LAUREN // AGE 20  
LATER-ONSET SMA  
TREATED WITH SPINRAZA

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

## SPINRAZA IS APPROVED FOR CHILDREN AND ADULTS WITH SMA

### INDICATION

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

### IMPORTANT SAFETY INFORMATION

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

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

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

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

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

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

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





# SPINRAZA is the only FDA-approved therapy for all patients with SMA regardless of age\*

7+ years of clinical trial experience  
in early- and later-onset SMA

Scan the QR code to explore the data



\*Clinical studies of SPINRAZA included patients from 3 days to 16 years of age at first dose, but did not include sufficient numbers of subjects aged 65 and over to determine whether they respond differently from younger patients.

## IMPORTANT FACTS ABOUT SPINRAZA® (nusinersen)

### USES

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

### WARNINGS

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

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

### COMMON SIDE EFFECTS

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

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

### OTHER INFORMATION

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

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

### QUESTIONS?

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

### MANUFACTURED FOR

Biogen, Cambridge, MA 02142



# 2022 ANNUAL SMA CONFERENCE

THURSDAY, JUNE 16TH – SUNDAY, JUNE 19TH

Disneyland Hotel in Anaheim, California

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 researcher 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 and showing our support for others. As always, the Annual SMA Conference and the SMA Research & Clinical Care Meeting run alongside each other. This is the largest conference in the world for those 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 another great 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.

You must complete your conference registration with Cure SMA prior to reserving your hotel room to get the special room rates of \$279 per night at the Disneyland Hotel, \$259 per night at Disney's Paradise Pier Hotel, and \$349 per night at Disney's Grand Californian Hotel & Spa (plus tax on all rates).

## 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.

**TO REGISTER FOR**  
the Annual SMA Conference, please visit  
[www.curesma.org/annual-sma-conference/](http://www.curesma.org/annual-sma-conference/).

# Thank You to Our Presenting Sponsors



**Biogen**

**Genentech**  
A Member of the Roche Group



**NOVARTIS**



## Newly Diagnosed Conference Program

Thanks to the generous funding provided by the Erin Trainor Memorial Fund, Cure SMA covers the costs of up to four (4) family members' registration fees, as well as one (1) hotel room for three (3) nights of the Annual SMA Conference. This program is for all families newly diagnosed since the last in-person conference in 2019. The mission of the Erin Trainor Memorial Fund is to generate substantial funds to be able to provide conference scholarships, allowing newly diagnosed SMA families and individuals the opportunity to attend the Annual SMA Conference. Our goal is to allow as many newly diagnosed SMA families and individuals as possible to attend and experience the benefits of this amazing conference and community. The meeting gives everyone the opportunity to gather critical care and daily living information soon after diagnosis, learn directly from experienced SMA physicians, and network with changes in the community. This program is automatically offered to all newly diagnosed families that contact Cure SMA. Cure SMA will reach out to those who qualify once conference registration has launched. This program includes a Meet and Mingle with other newly diagnosed families, generously sponsored by Novartis.

## Adults with SMA Conference Scholarship

This scholarship offers adults with SMA ages 18 and older, as well as one caregiver, waived registration fees, a stipend for travel, and one hotel room for 3 nights for the Annual SMA Conference. This program is automatically offered to all adults with SMA ages 18 and older. Cure SMA will reach out to all qualified individuals once conference registration has launched.

## General Conference Scholarship

For families who are not newly diagnosed, we also have general conference scholarships available to help waive the registration fee costs to attend the conference. Families looking to receive a general conference scholarship are required to apply and can be placed on a scholarship waiting list. They will be notified if they are able to receive a scholarship.

### Contact Us:

Cure SMA  
925 Busse Road  
Elk Grove Village, IL 60007



Phone: (800) 886-1762



[conference@cureSMA.org](mailto:conference@cureSMA.org)



[www.cureSMA.org](http://www.cureSMA.org)



# Letters from the Community



You brought us to tears. Thanks so much for the incredible package and the feeling of pure love. We're so new to this journey and this certainly makes us feel the support knowing we aren't alone. We are beyond grateful. From the deepest part of our hearts, THANK YOU!

~ The Benz Family



We wanted to share our SMA hero Evelyn. Evelyn is 3 years-old, and has SMA Type 1. She loves books and unicorns! Thank you for the care packages. They really helped us out so much. We are so glad to have found an amazing and supportive community.

~ The Gonzalez Family

Thank you so much for this care package! It brightened our day! We appreciate the thought so much! Here is Briggs with some of his goodies!

~ Lindsay Schaffer



Thank you so much for the huge, amazing care package! We are so grateful.

~ Hegland Family



I got the information packet earlier this week and came home to the care package today. To say I'm in shock is an understatement. I am so thankful. From the bottom of our hearts, thank you for all you guys do. I cannot believe this! So many tools and resources. I'm just blown away!

~ Melissa Voss





We just wanted to shout out a **HUGE THANK YOU** to the Cure SMA organization!! This care package was so unbelievably thoughtful and heartwarming to receive! To have this kind of support is absolutely amazing and we cannot thank you all enough!

~ Lissa Jones and Family



We would like to say a big **THANK YOU** for the care package and wagon that were sent to us. Madelyn is doing great! Kaden, who also has SMA, loved the toys and asked, “What’s next?”. Your team is a blessing! Thank you so much for all you do!

~ Wilson and Elva Martin



Brantley would like to send a huge **THANK YOU** to the Cure SMA team! He absolutely loves all the items in the care package he received. His favorite item is the O-ball, which he can grasp with both hands and bring it to his mouth.

~ The Dennison Family



Thank you, for all the goodies! The kids were so excited to get them!

~ Brenda Loritz



I am a parent to Odai Ahmed. He wants to say “Hi” with all this good stuff we really like from our care package. Thank you!

~ Alaa Mahmoud



We just received our care package and **WOW!** I can’t believe how amazing this is. It made me cry. I didn’t know what to expect in the package at all! I am so excited for her to play with everything in this box! Thank you from the bottom of our hearts; we are so beyond thankful.

~ Natasha Jones

# GRATITUDE FOR TEEN & ADULT CARE PACKAGE

I just learned about the new Independence Assistance Package and sent in my application for it. However, I never gave feedback on the previous support packages I received. The first Teen & Adult Support Package included an Amazon Echo and a moldable gel pillow, both of which have been amazing, and I've used on a regular daily basis. These care packages make a big difference for those of us who need some assistance. These packages give us the opportunity to try items without risk, and in many cases, it has turned out to be amazing.

~ Holly VonWald

Please accept my most sincere appreciation for your lovely Teen & Adult Support Package. Everything is so thoughtfully helpful and/or comforting. I am using several items right now and they are easing the difficulty of being on extended holds with insurance and provider/procedure scheduling. You truly brightened my rather cloudy outlook. Thank you for all you do!

~ Robbin Brabander

I received my support package today and I was so excited to see all the helpful items that will allow me to be more independent at home! I know that the kitchen utensils will be used often since I love to cook but struggle to open items. I also loved the smart switches so that I can turn things on and off with just the touch of my phone. How amazing!!! Thank you for all you do for me and all of us with SMA!

~ Jonna Simcox

I am a happy recipient of the various support packages you have offered over the course of this past year, including most recently the LifeVac and Independence Assistance Package. I wanted to write again to express my sincere gratitude. Many of the tech items in the Independence Assistance Package will or have already come in handy. Please know I am not only aware of the crucial role Cure SMA plays in spreading awareness, as well as advocating for breakthroughs in treatment and care for the SMA community, but I am also very grateful for all the efforts it makes.

~ Kareen

# Donations



Cure SMA received a special donation of lightweight rattles for our Newly Diagnosed Care Packages. A special thank you to Karime Garza for this donation!

Thank you to Debbie and Richard Butler for collecting these wonderful toys for the Newly Diagnosed Care Packages in honor of Liv Harlow McDonald's 4th birthday! Liv loved anything with unicorns, so they were excited to include fun new unicorn books in her honor!



Cure SMA would like to give a special thanks to Andrew and Jayanna Capite for collecting toys for the Newly Diagnosed Care Packages in honor of their daughter Amelia's 1st birthday. These toys were donated in honor of their friend Anna Hehman, who has SMA.

Special thank you to Holly Sontag for donating playdoh for the Newly Diagnosed Care Packages. This was donated in honor of Mila Thomas's 4th birthday!



Cure SMA would like to thank Kathy Philips for knitting these awesome beanies! Kathy donated them for the Newly Diagnosed Care Packages.

Cure SMA received a special delivery of handmade quilts to include in the Newly Diagnosed Care Packages! These beautiful quilts were made by Louise Chisholm in honor of Bodhi Bhattarai. Thank you, Louise!





Sydney Hegland



Aderion Merritt



Alexandria Ellis



Braedon DeVay



Davy Harris



Emily Pantier



Giavanna Moore



Weston Cook



Adelia Ollie Martinez



Ella Christopher



Luka Vega



Mya Cala



Payton Lavoie



Sean Johnson



Christopher Pantier



Grayson Hoskin



Emelie James



Tierney McKown



Paisley Brooks

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



# How Meditation Can Reduce Your Stress... Without Trying

By Tara Davenport, SMA Mother

Meditation is the current buzz word for stress relief or stress reduction, but many people have a misunderstanding of what meditation truly is and how it can help. To begin, let me tell you what meditation is not. Meditation is not trying to stop your thoughts. Meditation is not trying to clear your mind. Meditation is not trying to relax. Meditation is not trying to reduce your stress.

Is this surprising to you? In fact, meditation is simply focusing your attention on one sensation in your body (known as an anchor) for a period, even just 2 to 3 minutes. Let's say you choose the feeling of your breath as your anchor. During meditation—which can happen anywhere at any time—you practice focusing on the feeling of your breath. The way your chest moves, the way your belly moves, or the way your breath feels in your nose. For this period, you practice simply focusing on those sensations.

And guess what? Your attention will wander away from those sensations. Your attention will move to thinking about your grocery list or that doctor's appointment you have tomorrow. No worries. That's what our mind does—it wanders to other things. No need to try to keep this from happening. In the practice of meditation, when you become aware that your mind has wandered, you simply choose to bring your attention back to your anchor, or that feeling of your breath as it moves in and out of your body. When you again notice your mind has wandered, you choose again to bring it back to the anchor sensations.

As you continue this dance of the mind—wandering, back to the anchor—you are developing the ability to have a more stable mind. You are realizing you can choose where you put your attention. You don't have to follow all your thoughts, especially ones that aren't benefitting you.

Often, we are unaware that we have this power to move our attention and have a more stable mind. By engaging in a meditation practice, we not only practice this movement of our attention to a sensation in our body, but we also become more aware of how our thoughts jump around. Every one of us has a “monkey mind” that jumps from thing to thing! With practice, you can have more control of that “monkey mind” rather than it controlling you.

Training our mind is no different than training our bodies. We go to the gym or to physical therapy to train our bodies. We go to meditation to train our minds. It takes some practice, but in time we become aware of how our mind can hijack us and cause us unnecessary stress. When we practice intentionally paying attention to how our minds do this during our meditation practice, then when it happens as we are out living our lives, we are more likely to be able to stop and shift our attention to something that brings us more benefit than letting our minds run amok. And this can result in a more clear, relaxed mind and less stress.

For ideas and guided meditations, visit <https://insighttimer.com>.

# 2021 SUMMIT OF STRENGTH PROGRAM



Cure SMA has continued the Summit of Strength Program throughout 2021, offering a hybrid between original in-person events alongside the webinar series that was launched in 2020.

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

As of July 31, 2021, Cure SMA has hosted 44 Summits of Strength across the United States, reaching almost 3,100 registrants, and 22 Summit of Strength Webinars, bringing together 1,635 live viewers virtually! Almost 150 local and national experts have shared their knowledge on numerous highly rated and unique topics.

We have been happy to reintroduce these opportunities for networking and building community in person. This local engagement highlights Cure SMA's core values and further strengthens the bonds that make our communities so vibrant! We would also like to make our attendees aware that Cure SMA will continue to execute necessary COVID-19 precautions per guidelines from the U.S Centers for Disease Control and Prevention (CDC) at these in-person events moving forward.

The safety and well-being of all our attendees is our top priority. Everyone in attendance over the age of two will be required to wear a mask at all times, unless actively eating or drinking. Registration numbers will be monitored and limited to ensure the program remains at a safe attendance. Seating will be spaced out to provide proper social distancing. All attendees will also be provided with sanitizing wipes and gel hand sanitizer. The Dallas Summit of Strength offered six presentations from leading SMA experts to the 30 socially distanced attendees.

**In-person Summits of Strength will continue through the rest of 2021 – Be sure to register today and join in!**

**“Really enjoyed all of it. Great agenda. Loved all the speakers attended the conference as well. It was nice to have them there to interact with.”**

**“Loved seeing people and meeting local folks in the SMA community. We didn't know anyone before.”**

**“The Cure SMA network and community is an amazing resource!”**

**“Talking one to one with others and the presenters were great.”**

## This year's event dates and locations include:

May 1 – Dallas, TX	September 11 – Boston, MA
May 15 – Raleigh, NC	September 25 – NYC, NY
June 26 – Philadelphia, PA	October 2 – Anaheim, CA
July 10 – Indianapolis, IN	October 16 – San Francisco, CA
July 31 – Kansas City, MO	November 13 – Louisville, KY
August 7 – Orlando, FL	November 20 – Jacksonville, FL
August 21 – Cincinnati, OH	December 4 – New Orleans, LA
August 28 – Minneapolis, MN	

*This is a free program that includes breakfast, lunch, and parking for all attendees. If you have any questions or comments, please contact [communitysupport@curesma.org](mailto:communitysupport@curesma.org). Also, surveys are offered at every event; Cure SMA greatly values attendee feedback which helps us continue to build upon the value of this program.*

*Thank you to the National Presenting Sponsors for the 2021 Summit of Strength Program: Biogen and Genentech. We would also like to recognize Platinum Sponsor, Accredo, and supporting sponsor, Scholar Rock, for their support of the 2021 event series. The health, safety, and well-being of the entire SMA community is important to us. Cure SMA is continually monitoring and evaluating the impact of COVID-19 in the U.S. and in-person summit events are subject to change.*



# 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](mailto:stories@curesma.org)!



# Fertility, Pregnancy, and SMA

Contributors: Ahmed Ahmed, MD; Snigdha Alur-Gupta, MD; Debra Guntrum, MS, FNP, ANCC

Planning for and starting a family can be an exciting time in many people's lives. With continuing advances in nutritional, medicinal, respiratory, and physio therapies, more individuals with SMA can maintain independent living and, in many cases, safely navigate pregnancy. Planning is key to maximize the chance for a healthy pregnancy, particularly for individuals with SMA who have unique preconception and prenatal considerations in addition to routine obstetrical care.

## Fertility and SMA

Current reviews of human data have not suggested that SMA has a negative impact on female fertility, and many women with SMA have more than one pregnancy. Women with SMA do not have a higher risk for miscarriage, ectopic pregnancy, or stillbirth. However, SMA may impact male fertility as animal studies have shown impaired sperm production and testicular anatomy. Human data has found that some men who initially present for evaluation of infertility are later diagnosed with mild SMA. It is therefore possible that a diagnosis of SMA in men may increase the chance for lower testicular function and/or infertility.

## Preconception Care with SMA

To allow for personalized assessment of potential maternal risk factors associated with SMA and pregnancy, preconception consultation with a perinatologist (high-risk obstetrician) in Maternal Fetal Medicine is recommended. This will allow for anticipatory guidance specific to a women's SMA symptoms, including optimization of medications and ongoing guidance with a multidisciplinary team of healthcare providers (e.g., obstetrics, pulmonary, cardiovascular, neuromuscular, anesthesia, and supportive care).

## Pregnancy with SMA

Women with SMA can anticipate some differences in their prenatal care compared to what is considered "routine" management of pregnancy. Depending on her specific medical factors, this may include more frequent medical visits (e.g., prenatal check-ups, fetal monitoring with ultrasound and/or non-stress tests, or neurology visits). For some, pregnancy may worsen certain symptoms associated with SMA, and sometimes these changes may be permanent. Pregnancy-associated weight gain and change in center of gravity may lead to changes in how one transfers or increase the risk of falls and precipitate loss of ambulation. If this increased weakness or change in functional abilities persists after delivery, changes in adaptive equipment may be necessary. The later stages of pregnancy can result in reduced lung capacity and increased risk for pneumonia and other lung diseases. Pregnant women with SMA may also have a higher risk for urinary tract infections and/or chronic lower back pain. Maternal SMA does not appear to increase the risk for pregnancy complications such as gestational diabetes, gestational hypertension, and/or pre-eclampsia, poor growth in the fetus, or excessive amniotic fluid (polyhydramnios).

## Giving Birth with SMA

As is seen with other neuromuscular conditions, pregnant women with SMA have a higher chance for pre-term delivery and for operative (forceps, vacuum) or cesarean delivery. Multidisciplinary team planning can optimize delivery for both mother and baby. Anesthesia plays a critical role in the care of women with SMA during delivery, not only for maternal pain management, but to address potential issues from SMA-related skeletal changes (i.e., scoliosis, prior back surgeries) and respiratory care (i.e., airway management, cough assist).

## Life with Baby and SMA

Caring for an infant is an activity of daily living, so individuals with SMA may need a home evaluation by a physical or occupational therapist to assess for adaptations needed to care for the baby at home. For women who desire to breastfeed, consultation and ongoing support from a lactation specialist can be very beneficial. All new mothers need to be monitored for post-partum depression, and this is even more important for women with SMA, as studies have shown that there is a higher incidence of depression and/or stress in women with three or more functional limitations.

## Reproductive Genetics and SMA

The American Congress of Obstetrics and Gynecology recommends that SMA carrier screening be offered to all women who are thinking about getting pregnant or who are already pregnant. Genetic counseling is very important, both for individuals or partners who are diagnosed with SMA, as well as for individuals or partners who are found to be only carriers of SMA. For both groups, reproductive options when choosing to have a child include:

- Conceiving and carrying a pregnancy without doing any genetic testing.
- Conceiving a pregnancy with donor sperm and/or donor eggs to minimize the chance of having a baby with SMA.
- Conceiving a pregnancy and having prenatal genetic testing to determine whether a baby will have SMA.
- Pursuing IVF with pre-implantation genetic testing to try to minimize the chance that an embryo with SMA is transferred.
- Building a family through adoption.



## Get to know **EVRYSDI**

### An SMA medication for infants, children, and adults

In people 2 months and older

#### 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. 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, your healthcare provider will decide if taking Evrysdi is right for you during this time
    - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
  - are an adult male planning to have children: Evrysdi may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice
  - are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with Evrysdi
- **Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine
- 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
- 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, sore throat, and cough (upper respiratory infection), 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 side effects to Genentech at **1-888-835-2555**.

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

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

**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 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.

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

- are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. EVRYSDI may harm your unborn baby.
- are a woman who can become pregnant:
  - Before you start your treatment with EVRYSDI, your healthcare provider may test you for pregnancy. Because EVRYSDI may harm your unborn baby, you and your healthcare provider will decide if taking EVRYSDI is right for you during this time.
  - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping EVRYSDI.
- are an adult male planning to have children: EVRYSDI may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice.
- are breastfeeding or plan to breastfeed. It is not known if EVRYSDI passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with EVRYSDI.

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

- 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.

*Taking EVRYSDI*

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

*Reusable Oral Syringes*

- Your pharmacist will provide you with the reusable oral syringes that are needed for taking your medicine and explain how to use them. Wash the syringes per instructions after use. Do not throw them away.
- Use the reusable oral syringes provided by your pharmacist (you should receive 2 identical oral syringes) to measure your or your child's dose of EVRYSDI, as they are designed to protect the medicine from light. Contact your healthcare provider or pharmacist if your oral syringes are lost or damaged.
- 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.

**What are the possible side effects of EVRYSDI?**

**The most common side effects of EVRYSDI include:**

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

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

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

**How should I store EVRYSDI?**

- Store EVRYSDI in the refrigerator between 36°F to 46°F (2°C to 8°C). Do not freeze.
- Keep EVRYSDI in an upright position in the original amber bottle to protect from light.
- Throw away (discard) any unused portion of EVRYSDI 64 days after it is mixed by the pharmacist (constitution). Please see the Discard After date written on the bottle label. (See the **Instructions for Use** that comes with EVRYSDI).

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

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

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

**What are the ingredients in EVRYSDI?**

**Active ingredient:** risdiplam

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

**Genentech**

*A Member of the Roche Group*

EVRYSDI® (risdiplam)

Distributed by:

**Genentech, Inc.**  
A Member of the Roche Group  
1 DNA Way  
South San Francisco, CA  
94080-4990

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



# SPOTLIGHT *on* The Menke Family's SMA Diagnosis

September was Newborn Screening Awareness Month, a chance to talk about the progress we have made in getting SMA added to newborn screening panels across the country and the work still to be done to ensure 100% of all babies are screened for SMA at birth.

Carrie Menke is a South Dakota native who resides in Sioux Falls with her husband Tony and their four children—Xavier, Colette, Avila, and Rose. Carrie is one of the many advocates across the country who has supported Cure SMA's efforts to have SMA added to her state's newborn screening panel. Her passion is strong on this issue, as both Xavier and Rose have SMA.

Xavier was born in November 2013. "He met all his early developmental milestones and was walking independently shortly after his first birthday," said Carrie. "Xavier was also very verbal, saying his first words at 8 months of age and, later, talking in sentences. He was consistently on-track until he was about 18 months of age."

It was between the ages of 2 and 3 years that Carrie and Tony noticed Xavier's most stark regression. Xavier saw his pediatrician, a physical therapist and then a neurologist. The neurologist immediately suspected SMA. Both Xavier and his younger sister, Colette, were tested. Colette tested negative, while Xavier was diagnosed with SMA at the age of 3 years.

*Learning that Xavier would only get worse over time, given the degenerative nature of the disease, was devastating," recalled Carrie. Yet, the whole family felt hopeful when they learned that a treatment was recently approved that could help prevent further muscle weakness.*

Xavier is now 6 years old and enjoys playing with his younger sisters, Colette, Avila, and Rose. Rose also has SMA. She was diagnosed early and was treated pre-symptomatically, just after she turned 1 month old.

*"When we told Xavier that his baby sister would also have SMA, his response was sweet," recalls Carrie. "He said, 'Now there will be someone like me.'" Xavier also started treatment, once one became available.*

Since starting treatment, Xavier's endurance has improved. "He can now jump with both feet off the ground, walk upstairs while holding the railing, and he no longer exhibits tremors. He still is significantly behind his peers with gross motor skills, but he is determined," says Carrie.

Now that her children are bit older, Carrie reflects on their great sibling relationship. "The girls keep Xavier physically active and working on achieving new gross motor skills. He gets frustrated at times that he cannot keep up, but Colette has an awareness of Xavier's limitations and often will keep pace with him when she notices Xavier getting discouraged. He always does his best and finds ways to keep up with his sisters and friends," shared Colette.

Like Carrie and Tony, more than 17,733 South Dakota residents are SMA carriers. In June 2021, South Dakota began screening babies for SMA as part of its newborn screening panel. Carrie knows that her advocacy efforts—and the decisions made by law makers in her state—around newborn screening of SMA will help give parents the information they need to make important decisions about treatment and care of their child with SMA.

Despite the progress in screening newborns for SMA, the U.S. remains well short of the goal of 100 percent universal newborn screening for SMA. With 1 in 3 babies in the U.S. still at risk of a delayed SMA diagnosis, now is the time to encourage non-screening states to act on newborn screening for SMA! Check out [www.curesma.org/advocacy/](http://www.curesma.org/advocacy/) to learn more on what you can do.

# Recognize the symptoms and save a life!



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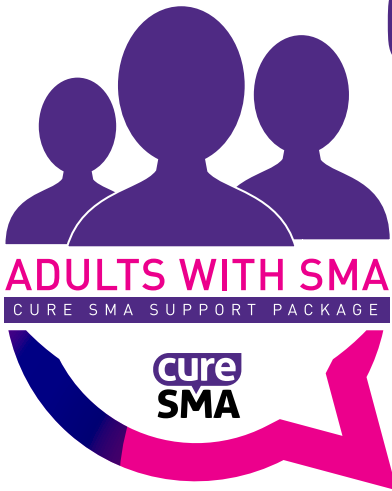
EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

Motor delays or missed milestones may  
signal serious conditions like SMA.

There is new hope, but it's important to act  
swiftly and make SMArt Moves.



[SMArtMoves.CureSMA.org](http://SMArtMoves.CureSMA.org)



# 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  
<http://events.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.\*\**

# Community SPOTLIGHT



## Sparking an Internal Movement with LaMondre Pough

When speaking with LaMondre Pough, CEO of Billion Strong, a few things are immediately evident: his confidence, his passion, his heart, and his ability to spark empowerment.

**“It’s not that I’m intentionally speaking to motivate, but to enlighten. I’m trying to educate; I’m trying to inform – and I’m really passionate about that, it drives me.”**

LaMondre is a natural communicator, something he developed early on in childhood through singing and feeling comfortable on stage. He has extensive experience working in both for profit and non-profit organizations, which began with teaching advocacy skills to people with disabilities and working with companies to ensure accessibility in built and digital environments. He’s also an entrepreneur, content developer and the Chairman of Arts Access South Carolina.

The intersection of communications work and his dedication toward disability inclusion is where we currently find him, as the CEO of Billion Strong, A Global Disability Movement. Created out of his work with Ruh Global Impact, a disability inclusion consulting firm, LaMondre wanted to build a global movement to unite, educate, and elevate people with disabilities.

“[I wanted] a movement that really addressed the identity of people with disabilities. When I say identity, I’m talking about it in two ways. Number one – it’s about how the world sees people with disabilities. And there’s a flip side to it – how people with disabilities see themselves.”

This is what LaMondre calls an “internal movement,” one that asks people to advocate for themselves and feel empowered to show up authentically. He realizes that this is something everyone needs, no matter what abilities they have.

“I found people who were not necessarily part of the disability community that still needed to know how to advocate for themselves. They still had to start understanding they weren’t broken, that they were not less than. That their intrinsic value was not diminished because of a situation or a circumstance.”

Although it may already be obvious, it must be said; LaMondre is bold and strong in his convictions. He certainly is in no way diminished by his circumstance. When asked about spinal muscular atrophy (SMA), his answer is direct: “it’s simply part of who I am ... just like the fact that I’m male, just like the fact that I’m Black. I have spinal muscular atrophy”.

His diagnosis story is familiar. Doctors delivered a bleak message to his mother and listed out the things he “wouldn’t be able to do.” She saw through it.

“My mother loved me for real. Her thinking was ‘it doesn’t matter how much time I have with him; I’m going to raise him to be whatever it is he’s going to be,’” shared LaMondre.

She encouraged him to go out and create his own path, making clear other people were not going to create it for him. She empowered him to go after what he wanted, to seek out accommodations, and get familiar with speaking up for himself. She would tell him to get it as far as he could and then she’d help with the rest.

“She made me feel so good about being exactly what I was.” Feeling good about exactly who you are is part of Billion Strong’s mission and amplifying the voices of people with disabilities is imperative not only to LaMondre’s work but to society.

“Stop thinking about it as the ‘right’ thing to do and start thinking about it as the ‘smart thing’ to do,” says LaMondre.

Empowering future generations to come is something that is also dear to LaMondre’s heart, as the cousin to Ariana, a 12-year-old who has SMA. After hearing her exclaim she could be anything she wanted, he was overcome with emotions because he realized she saw herself and her potential within.

“This is a part of my purpose. It’s the reason I breathe.”



# COVID-19 UPDATE: WHERE ARE WE NOW?

Since June 20, 2021, when the U.S. had the lowest number of new COVID-19 cases across the country, new infection rates have increased rapidly, driven by a more easily transmitted virus variant, called Delta. COVID-19 variants arise due to continued transmission of the virus to susceptible people. With every new case of COVID-19 infection, the virus can change and mutate to improve its survival in humans. Some variants arise and disappear while others—like the Delta variant, which has become the dominant COVID-19 strain and is found today in >99% of COVID-19 infections—emerge and continue to spread. To date, four variants have been classified as “variants of concern,” as defined by the U.S. Centers for Disease and Prevention (CDC): Alpha, Beta, Gamma, and Delta. The Delta variant is expected to represent 99% of all COVID-19 infections in the next few weeks. The community transmission rates across the U.S. are “high” and some states in the southeast and south are seeing an even greater number of people infected daily now, compared to the prior COVID-19 peak that occurred in January 2021.

## Who is Being Hospitalized?

U.S. hospitalizations for COVID-19 in the past two months has been predominantly people who have not received the COVID-19 vaccine, representing up to more than 95% of those hospitalized. Dr. Rochelle Walensky, Director of the CDC, states that, “[COVID-19] is becoming a pandemic of the unvaccinated.” In addition, the hospitalization rate of non-Hispanic Black people has increased faster and has risen higher than other racial and ethnic groups and is twice the rate for non-Hispanic White people. The age range of those hospitalized has dropped and is predominantly people 18-49 years old. New hospitalization rates for COVID-19 are at their highest levels since the start of the pandemic in several states, including Alabama, Florida, Louisiana, Mississippi, Oregon, and Washington State. Similarly, deaths have been predominantly among people who are not vaccinated.

## COVID Vaccination

As of August 26, 2021, 365.8 million COVID-19 vaccine doses have been administered in the U.S., meaning 61% of the U.S. population has received one vaccine dose and 52% of the total U.S. population has been fully vaccinated. Among people over the age of 65 years, 92% have received at least one dose and 81% are fully vaccinated.



This statistic may explain the shift down in the ages of those hospitalized. Some vaccinated people will become infected as no vaccine is 100% effective; however, the risk for severe disease, hospitalization, and death are decreased for those who are vaccinated.

The U.S. currently has three vaccines available. The Pfizer BioNTech was given full approval by the U.S. Food and Drug Administration on August 23, 2021, for people 16 years of age and older and 2 doses are recommended for full vaccination. The Pfizer BioNTech vaccine continues to be available for children 12 to 16-year-old under the Emergency Use Authorization (EUA). Two other vaccines are available under the EUA—the Moderna mRNA-based vaccine (2 doses recommended) and the Johnson & Johnson vaccine (1 dose recommended) for people 18 years of age and older. The Pfizer vaccine is also available for children 12 to 16-year-old under the EUA.

These vaccines are effective against the Delta variant. However, due to high virus transmission rates, suboptimal and variable vaccination rates across the U.S., knowing vaccines are not 100% effective, and the risk for new COVID-19 variants to develop, continued vigilant prevention is required to get COVID-19 under control.





COVID-19 vaccines are in clinical trials for children 2 years to 12 years of age. Information from clinical trials is anticipated in late fall 2021 for review and consideration of EUA or approval in late 2021 or early 2022. The CDC's Advisory Committee on Immunization Practices (ACIP) and the FDA are considering recommending a COVID-19 vaccine booster to begin this fall. The proposal is to provide the booster eight months after the 2nd dose of the mRNA vaccines, (Pfizer-BioNTech or Moderna). The FDA is conducting an independent evaluation to determine the safety and effectiveness of a booster dose. The ACIP will decide whether to issue a booster dose recommendation based on a thorough review of the evidence.

## Impact on People with SMA

Based on Cure SMA surveys of the SMA community about their experience with COVID-19, approximately 21% of people with SMA have had or been exposed to COVID-19, and 0.6% of people with SMA were hospitalized. Severity has been variable and has ranged from fatigue and loss of smell and taste to having severe difficulty breathing and requiring hospitalization with invasive breathing support in an intensive care unit. Currently, there are not data on tolerability of the COVID-19 vaccine in people with SMA. The risk for complications due to COVID-19 infection is high for those with SMA due to the underlying muscle weakness that may be made worse with COVID-19 infection.

## How to Protect Yourself and Your Family

Most importantly, get a COVID-19 vaccine if you are eligible. Getting a vaccine will help protect others who are not eligible to receive the vaccine. Wearing a mask over the nose and mouth is highly recommended when indoors at public spaces or in crowded outdoor spaces. Physical distancing of 6 feet should be practiced, and you should avoid interacting with other who are ill or who don't live with you. Ensure general hygiene practices like frequent hand washing, covering coughs and sneezes, and disinfecting high touch surfaces daily. Finally, as you monitor your health, stay home if not feeling well and be aware of suspicious symptoms such as fever, cough, and loss of smell or taste.

As it relates to education, keep in mind that in-school transmission of COVID-19 reflects community transmission. Thus, if community transmission is high and community vaccination level is low, then students and staff are more likely to come to school while infected and introduce COVID-19 into the schools. To mitigate risk of COVID-19 in school, all adults and children 12 years of age and older should be vaccinated. Everyone who is able should wear a mask over the nose and mouth and/or a face shield, practice physical distancing, and stay in small groups. If your school situation does not seem safe, consider home schooling.

### Resources:

CDC website:

[www.cdc.gov/coronavirus/2019-ncov/index.html](https://www.cdc.gov/coronavirus/2019-ncov/index.html)

Cure SMA website:

[www.curesma.org/covid19/](https://www.curesma.org/covid19/)



# THE IMPORTANCE OF ADVOCACY

Individuals with SMA and their families advocate every day in doctor's offices, classrooms, and over the phone with their insurance companies. These same skills can be used to create larger, community-wide changes and improvements by advocating for policies and legislation at the federal and state level.

State and federal policies impact nearly all aspects of life for those with SMA. Advocacy is critical in advancing SMA-related policies—from insurance coverage to employment opportunities to everyday accessibility. The voices of individuals with SMA and their families are instrumental to policy advancement when reaching out to lawmakers who can have an impact on the community.

## Federal and State Advocacy: Why Both are Important

With an increased focus on advancing policies that directly impact people with SMA, it is important to first understand the legislative structure and how the legislative process works within your home state. The structures of state legislatures can vary from full-time, part-time, or a hybrid of the two. Additionally, each state legislature has committees that oversee issues related to children's health and other topics important to children and adults with SMA. Legislative committees typically hold hearings and markups on legislative bills before they are considered by full state legislative bodies.

Though we think of federal advocacy as relating to Washington D.C., all persons elected to Congress have district offices in their respective states. Typically, Congress creates or extends major legislative bills 4 to 6 years at a time but collect improvements or revisions every year. This is where you can really make an impact.

Congressional champions, or those who support the advocacy goals of the SMA community, are always looking for opportunities to add provisions to any relevant active legislation. An example of this is the recent legislation that supports an expansion of The ABLA Age Adjustment Act, a bill that would ensure all individuals with SMA, including those with SMA Type 4, would be eligible to save for their futures without jeopardizing their eligibility for Medicaid and other essential public services.

It's important to stay active in your advocacy throughout the year by setting up an educational call with your representative or acting on specific alerts Cure SMA provides so that state or congressional offices are familiar with you once an urgent issue or opportunity arises. In its most basic sense, advocacy is about relationship building. Therefore, it's important to build rapport with your state or federal government officials year-round.

**NOTE: Your advocacy may matter more if you live in the district and state of the chairperson responsible for the issue/bill revision.**



## Things to Consider in Legislative Advocacy

Not every action ends with an immediate win, but every action has an impact and leads to increased awareness about the issue. In addition, big ideas or major overhauls rarely happen in one single action, but rather through incremental changes from multiple actions over years (i.e., provisions of a large bill advancing rather than the entire comprehensive bill).

SMA Community legislative wins—such as the Americans with Disabilities Act, 21st Century Cures Act, and the Newborn Screening Saves Lives Act—did not happen overnight. These successes were the result of years of advocacy and thousands of advocacy engagements, from letters and action alerts to in-person meetings and testimony at public forums. This is why it's important to stay active throughout the year regarding advocacy efforts.

## Your Activism, Your Stories, and Your Dedication Continue to Pave the Way

Tomorrow's successes and opportunities are the result of today's actions and community engagements. Everyone in the SMA community can engage to create meaningful change. Every action matters and no action is too small. Visit the advocacy page on our website to get involved today: [www.curesma.org/advocacy](http://www.curesma.org/advocacy).

## ADVOCACY TIPS

### Federal Advocacy

Remember that federal elected officials have two types of offices. They have their congressional office in Washington, D.C., and what's called a district office in their home state. It's often easier to get a meeting in the district office.

### State Advocacy

Always reach out to the legislator from your district first when advocating. It may seem more effective to reach out to the chairman of a certain committee, but always start with your legislator. It's considered a breach of etiquette for a legislator to respond to a colleague's constituent, so you'll just be referred to the person who represents you.





ZOLGENSMA® (onasemnogene abeparvovec-xioi) is a one-time-only gene therapy that stops the progression of spinal muscular atrophy (SMA), helping children experience memorable moments in life. From speaking their first words to trying new foods to standing on their own, these children are achieving milestones big and small.

**Results and outcomes vary among children based on several factors, including how far their SMA symptoms progressed prior to receiving treatment.**



Natalie, treated at ~14 months and pictured at 1½ years, was diagnosed with SMA Type 2.

Follow us on **Facebook @zolgensmausa** and **Instagram @zolgensma\_us** to see how children treated with ZOLGENSMA are doing today.

## Indication and Important Safety Information

### What is ZOLGENSMA?

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA). ZOLGENSMA is given as a one-time infusion into a vein. ZOLGENSMA was not evaluated in patients with advanced SMA.

### What is the most important information I should know about ZOLGENSMA?

- ZOLGENSMA can cause acute serious liver injury. Liver enzymes could become elevated and may reflect acute serious liver injury in children who receive ZOLGENSMA.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of the corticosteroid or vomits it up.

### What should I watch for before and after infusion with ZOLGENSMA?

- Viral respiratory infections before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if you see signs of a possible viral respiratory infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Decreased platelet counts could occur following infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.

- Thrombotic microangiopathy (TMA) has been reported to occur approximately one week after ZOLGENSMA infusion. Caregivers should seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.

### What do I need to know about vaccinations and ZOLGENSMA?

- Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed to accommodate treatment with a corticosteroid.
- Protection against respiratory syncytial virus (RSV) is recommended.

### Do I need to take precautions with the patient's bodily waste?

Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool. Use good hand hygiene when coming into direct contact with bodily waste for 1 month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

### What are the possible or likely side effects of ZOLGENSMA?

The most common side effects that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

**The safety information provided here is not comprehensive. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.**

**You are encouraged to report suspected side effects by contacting the FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch), or Novartis Gene Therapies, Inc. at 833-828-3947.**

**Please see the Brief Summary of the Full Prescribing Information on the next page.**



## IMPORTANT FACTS ABOUT ZOLGENSMA® (onasemnogene abeparvovec-xioi)

### USE

ZOLGENSMA is a prescription gene therapy used to treat children less than 2 years old with spinal muscular atrophy (SMA).

- ZOLGENSMA is given as a one-time infusion into a vein.
- ZOLGENSMA was not evaluated in patients with advanced SMA.

### WARNINGS

#### Acute Serious Liver Injury and Elevated Liver Enzymes

- ZOLGENSMA can cause acute serious liver injury. Liver enzymes could become elevated and may reflect acute serious liver injury in children who receive ZOLGENSMA.
- Patients will receive an oral corticosteroid before and after infusion with ZOLGENSMA and will undergo regular blood tests to monitor liver function.
- Contact the patient's doctor immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of the corticosteroid or vomits it up.

**Decreased platelet counts** could occur following infusion with ZOLGENSMA. Seek immediate medical attention if the patient experiences unexpected bleeding or bruising.

**Thrombotic microangiopathy (TMA)** has been reported to occur approximately one week after ZOLGENSMA infusion. Seek immediate medical attention if the patient experiences any signs or symptoms of TMA, such as unexpected bruising or bleeding, seizures, or decreased urine output.

### OTHER IMPORTANT INFORMATION

**Patients should be tested for the presence of anti-AAV9 antibodies** prior to infusion with ZOLGENSMA.

**Vaccination** schedule should be adjusted where possible to accommodate treatment with an oral corticosteroid. Talk with the patient's doctor to decide if adjustments to the vaccination schedule are needed during corticosteroid use. Protection against respiratory syncytial virus (RSV) is recommended.

**Viral respiratory infections** before or after ZOLGENSMA infusion can lead to more serious complications. Contact the patient's doctor immediately if you see signs of a possible viral respiratory infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.

**Temporarily, small amounts of ZOLGENSMA may be found in the patient's stool.** Use good hand hygiene when coming into direct contact with bodily waste for 1 month after infusion with ZOLGENSMA. Disposable diapers should be sealed in disposable trash bags and thrown out with regular trash.

### COMMON SIDE EFFECTS

**The most common side effects** that occurred in patients treated with ZOLGENSMA were elevated liver enzymes and vomiting.

**These are not all the possible side effects. Talk to the patient's doctor about any side effects that bother the patient or that don't go away.**

### QUESTIONS?

To learn more, talk to your doctor and visit [www.ZOLGENSMA.com](http://www.ZOLGENSMA.com) for Full Prescribing Information.

### MANUFACTURED, PACKED, DISTRIBUTED by

AveXis, Inc.

Bannockburn, IL 60015

04/2021 US-ZOL-20-0121 V2

# Donor Spotlight:



Kevin Pearce with granddaughter, Neely, a 5-year-old with SMA Type 2.

## Kevin and Tracy Pearce

Kevin and Tracy Pearce became involved with Cure SMA when their granddaughter, Neely, was diagnosed with SMA at the age of 2 months, after she began missing key motor milestones. As grandparents, Kevin and Tracy felt helpless. But after being introduced to Cure SMA, they had hope and knew that the best way they could support Neely was to donate to Cure SMA to help find a treatment.

Kevin and Tracy are dedicated monthly donors to Cure SMA and have supported the San Diego Walk-n-Roll for the last three years. Through their gifts, they show their love for Neely. They know that when they give to Cure SMA, they are doing their part in making sure she and others have the best quality of life, now and in the future. They make a monthly donation a priority. Kevin says, “We make our month donation a priority. Circumstances in life can sometimes get in the way and an automatic payment ensures that it is never overlooked.” They encourage others to become monthly donors because it offers consistent income that Cure SMA can depend on while continuing to expand critical research and life-changing support services. Neely is now 5 years old and just started kindergarten. Kevin and Tracy are excited to watch her blossom and grow as the spunky, energetic little girl she’s become.

## Joe Scully

Joe Scully is a proud uncle. He was first introduced to SMA when his cousin and her husband lost their son, Mark, at 6 months old. Seven years later, their daughter, Lauren, was diagnosed with SMA Type 3 at the age of 4 years.

Joe wanted to get involved with Cure SMA because he wanted to help people with SMA, like Mark and Lauren, and their families. He started attending fundraisers and Walk-n-Rolls 31 years ago, and eventually began his own golf tournament. “I enjoyed taking the lead and running my own fundraiser because the Cure SMA team was extremely helpful and there was such a feeling of community when everyone got together. Donating and fundraising is the least I can do,” said Joe.

Joe understands how scary an SMA diagnosis can be, and he feels thankful to Cure SMA and the SMA community that significant progress has been made through treatments and support services. He knows that early intervention has changed the course of SMA for future generations, but that there is still a lot to do. Joe also knows that with everyone’s continued support, we will be able to find a cure one day. Joe states, “Sometimes you choose the cause you want to support, and other times it chooses you. SMA chose us, and we will continue to support Cure SMA and the SMA community!”



Joe Scully with niece, Lauren Rogowicz, a 28-year-old with SMA Type 3.



**JOIN**



**As a member of The Purple Circle, you are helping to lead Cure SMA's mission to fund critical SMA research and community support programs.**

Membership in this society is based on an annual, non-event, general gift of \$1,000 or greater. Cure SMA's annual giving cycle operates concurrent to our fiscal year (July 1-June 30).

## **BENEFITS INCLUDE:**

### **\$10,000+**

- Welcome letter from Kenneth Hobby, President of Cure SMA, and a welcome call from a member of Cure SMA Leadership.
- Exclusive quarterly newsletter providing updates from strategic areas of focus such as research, clinical care, community support, and advocacy.
- Bi-annual, personal update on organization's progress from member of Cure SMA Leadership.
- One free Cure SMA merchandise item of your choice, plus 10% discount on any additional items.
- Your name listed on The Purple Circle donor honor roll on the Cure SMA website.

### **\$1,000 - \$9,999**

- Welcome letter from Kenneth Hobby, President of Cure SMA.
- Exclusive quarterly newsletter providing updates from strategic areas of focus such as research, clinical care, community support, and advocacy.
- 10% discount on Cure SMA merchandise.
- Your name listed on The Purple Circle donor honor roll on the Cure SMA website.

Questions about The Purple Circle or your donation? Please email us at [ThePurpleCircle@curesma.org](mailto:ThePurpleCircle@curesma.org).



**Make a donation today at: [www.curesma.org/donate](http://www.curesma.org/donate).**

To donate by mail, send your donation to: Cure SMA, 925 Busse Road, Elk Grove Village, IL 60007.

# Clinical Care Update

## Why Know Your SMN2 Copy Number

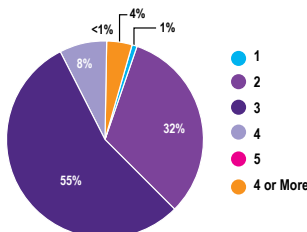
With the availability of SMA treatments, the conversation on how we talk about SMA is changing. Prior to identifying the gene for SMA and the availability of SMA treatments, SMA was described clinically by SMA “Type,” from Type 0 to 4. SMA type was based on age of onset of SMA symptoms and maximum motor function achieved (i.e., non-sitter, sitter, or walker). For example, Type 1 has been described as the most severe form of SMA, with symptoms appearing in the first six months of life and unable to sit independently.

In 1995, it was discovered that SMA is caused by the loss or mutation of the survivor of motor neuron (SMN) 1 gene. A near duplicate of the SMN1 gene, called the SMN2 gene, was identified to encode the same protein as SMN1, but although it is not a replacement for the SMN1 gene, research has shown that it modified the severity of SMA. These discoveries have led to several SMN-enhancing treatments. The use of SMN-enhancing treatments has resulted in motor function achievements that have exceeded expectations for SMA based on the SMA Type someone was diagnosed with. For example, those diagnosed with SMA Type 1 are now reporting to sit independently. Furthermore, infants identified with SMA through statewide newborn screening and subsequently treated prior to symptom onset fall outside the criteria for describing SMA by Type.

### So How Do We Describe SMA?

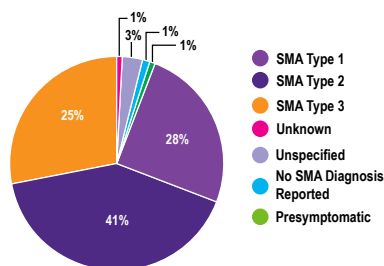
As we continue to learn more and talk about SMA in this era of disease modifying treatments, one aspect of the disease that does not change is the number of copies of SMN2 a person has. People with SMA are born with a specific number of SMN1 genes and a set number of SMN2 copies. If you have had complete genetic testing for SMA, your SMN1 copy number (typically 0 copies and occasionally 1 copy) and your SMN2 copy number (typically between 1 and 4 and some with 4-plus or more) is known. In general, the higher the SMN2 copy number you have, the less severe the symptoms of SMA. We encourage you to be familiar with these numbers. Clinical trials for SMA-specific treatments have shown that one’s disease trajectory is influenced not only by the age they began treatment but also by the number of SMN2 copies one has. Knowing your SMN2 copy number can help manage expectations of treatment outcomes. Conversations to describe SMA and prognosis are shifting to add consideration of SMN2 copy number and what SMN2 copy number may mean for your future and the SMA community’s understanding of SMA.

### SMN2 Copy Number as Reported in SMA Clinical Data Registry



1 Copy	6
2 Copies	147
3 Copies	256
4 Copies	35
4+ Copies	18
5 Copies	1

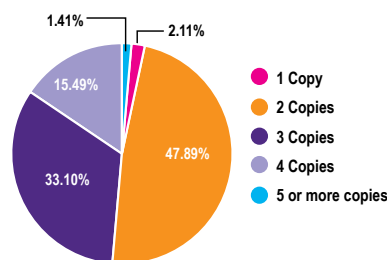
### SMA Type as Reported in SMA Clinical Data Registry



Type 1	162
Type 2	241
Type 3	143
Unspecified	8
Presymptomatic	17
Unknown	5
No SMA Diagnosis Reported	6

The above graphics represent data collected in the SMA Clinical Data Registry from the Care Center Network. The most common SMN2 copy number is 3 copies and the most common type is Type 2. These numbers identify the prevalence of SMN2 copy number and SMA type. **Prevalence** describes people currently living with SMA.

### SMN2 Copy Number of Infants with SMA Identified by Newborn Screening as Reported in Cure SMA Database n=142



1 Copy	3
2 Copies	68
3 Copies	47
4 Copies	22
5 or more copies	2

In contrast, SMN2 copy number of newly diagnosed infants in the Cure SMA database shows that the most common SMN2 copy number is 2 copies. This preliminary data represents the incidence of SMN2 copy number in SMA. **Incidence** describes all people born with SMA. SMA type is not provided for newborn screening-identified infants because diagnosis of SMA occurred before symptom onset.

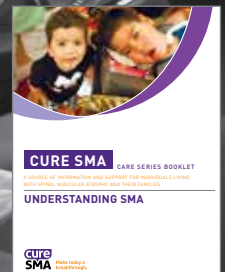
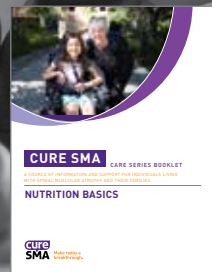
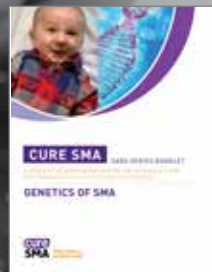
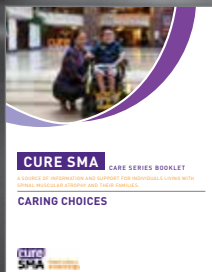
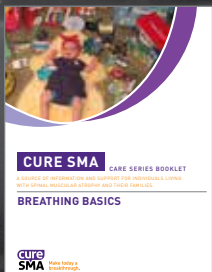
**Thank you to the SMA community.**





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

*\*Some books are available in other languages*



# Advocacy Update

## Cure SMA Unveils Online Advocacy Toolkit to Assist You in Your Advocacy

State and federal policies impact nearly all aspects of life for those with SMA. Advocacy is critical in advancing SMA-related policies, and the voices of individuals with SMA and their families are incredibly important when reaching out to lawmakers who can have an impact on the community.

To promote SMA community advocacy, Cure SMA has launched an online Advocacy Toolkit that includes shareable and printable one-page fact sheets to help you educate Members of Congress and other key policymakers about issues important to the SMA community.

In a 2020 Cure SMA survey, adults with SMA self-reported great interest in advocacy, but also expressed their limited experience in reaching out to elected leaders in their states. Cure SMA's Advocacy Team created this toolkit as a resource to foster greater advocacy impact and participation by adults with SMA, families of people with SMA, and other supporters of the SMA community.

The toolkit features advocacy fact sheets on healthcare, employment and financial security, transportation, community living, emergency disaster response, and other key SMA community priorities. Each fact sheet contains a "Did You Know" section with key facts and background on the topic to inform Cure SMA advocates and educate policymakers about the topic. The remainder of each fact sheet is messaged to the policymaker and their staff, highlighting Cure SMA's position on the issue.

The online toolkit and advocacy fact sheets are great tools to increase your impact in your personal advocacy around legislative issues important to the SMA community. The fact sheets can be easily downloaded or printed to share in advance of or as a follow up to a call, meeting, or other correspondence with a Member of Congress, congressional staff, or other key decisionmaker.

TO ACCESS THE CURE SMA ADVOCACY TOOLKIT,  
go to [www.curesma.org/advocacy/#advocacy-toolkit](http://www.curesma.org/advocacy/#advocacy-toolkit)

OR CONTACT CURE SMA ADVOCACY  
at [advocacy@curesma.org](mailto:advocacy@curesma.org).

# THE POWER OF YOU!

## Cure SMA Advocates Making a Difference Through Advocacy

Fiscal Year 2021 (July 1, 2020 through June 30, 2021)

2,400+

separate Cure SMA Advocate actions

5,800+

Cure SMA Advocate messages sent to key federal and state legislators

53%

of Cure SMA Advocate actions supported newborn screening for SMA

38%

of Cure SMA Advocate actions supported adult and young adult SMA issues

9%

of Cure SMA Advocate actions supported SMA treatment access and coverage issues

### INTERESTED IN ADVOCACY? WANT TO HELP SUPPORT CURE SMA ADVOCACY PRIORITIES?

Go to [www.curesma.org/advocacy/](http://www.curesma.org/advocacy/) to sign up to be an advocate and take action in support of SMA community priorities.

### 3-Years-In: Newborn Screening Moves Closer to Universal SMA Screening Goal



In early July 2021, Cure SMA celebrated the three-year anniversary of SMA being added to the federal Recommended Uniform Screening Panel (RUSP)—the list of suggested conditions that states should screen for within their statewide universal newborn screening programs. We project that more than 9 in every 10 babies born in the U.S. will be screened for SMA by the end of 2021. So far this year, six states have already implemented statewide screening of SMA, including Texas, which represents 10 percent of all U.S. births.

Another four or more states are expected to start screening for SMA before the end of 2021, pushing the percentage of U.S. babies screened for SMA at birth to well past 90 percent. Thanks to effective advocacy efforts of Cure SMA supporters and partners, we are inching closer to Cure SMA's goal of 100 percent universal newborn screening for SMA. Thank you to everyone who has contributed to this success. Together, we can reach this goal!



# Fundraising Update

## The Benefits of Planned Giving

Estate planning is not a frequent topic of conversation for most people, but it is an important part of Cure SMA's strategy to fund our mission and accelerate progress in research, care, and support. By discussing your options and planning for the future, you can create a legacy for yourself and for your family that makes a lasting impact in the SMA community. Together with Cure SMA, you can learn about the benefits of planned giving and explore the different opportunities to support our life-changing programs. There are many options to choose from—such as retirement plan gifts, wills and trusts, charitable gift annuities, and many more. Each allows you to make larger, more impactful gifts that support the daily lives and needs of those with SMA.

For donors, there are many benefits to making a planned gift to Cure SMA. Most importantly, they offer additional avenues to make larger, transformational gifts that accelerate progress to new levels. These alternative giving vehicles offer greater flexibility for your charitable decisions by allowing you to give gifts of securities and other non-cash gifts. Also, there are planned gifts that can provide tax incentives, income options, and greater financial security to you and your loved ones, such as charitable gift annuities and charitable remainder trusts. By working with your financial advisors and Cure SMA development staff, you can explore the many benefits of planned gifts and create a legacy with Cure SMA.

Not only do planned gifts offer benefits to the donor, but they also play an important role for Cure SMA. They help sustain long-term funding and ensure future stability that our organization can rely on as we continue to grow to meet the changing needs of the entire SMA community. As COVID-19 has taught us over the past 18 months, we can't always predict the future. Your planned gift can help us diversify our revenue and provide a consistent, long-term source of funding that will help us navigate future challenges.

But planned gifts don't just help us sustain challenges. They create new opportunities that otherwise might not have been possible. Due to their size and impact, they have the capability of creating transformational change at an organization.



*Cure SMA provides support and hope to families across the United States that weren't available during our son's life. In 2019, we named Cure SMA as a major beneficiary of our estate plan and created The Tyler William Orr Memorial Fund. Also, we contribute yearly IRA distributions that allow Cure SMA to realize the benefits today.*

*~ Susan Orr – Susan and Bill Orr's son Tyler, lived with SMA and passed away at 3 years old in 1982.*

They help provide the funding needed to launch new local programs, expand research projects to create the next breakthroughs in SMA, and help spread awareness to bring new stakeholders to our cause. Simply put, by making a planned gift, you can help change the future of SMA.

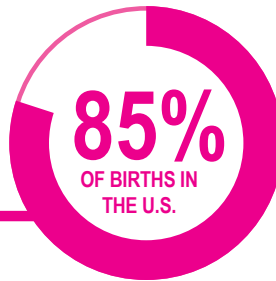
*Are you interested in making a planned gift to Cure SMA or want to learn more? Contact Brett Kinley, Director of Development – Individual Giving, at [brett.kinley@curesma.org](mailto:brett.kinley@curesma.org) or (847) 709-6314.*

Check out Cure SMAs' new Planned Giving website at [curesma.givingplan.net](http://curesma.givingplan.net)

Over the last 12 months, Cure SMA continued to make progress for all individuals with SMA and their families by advancing our research, advocacy, care, and support programs. These efforts were made possible by the hard work and dedication of the entire SMA community. We believe that no challenge is too large and together we will build a hopeful tomorrow for future generations of people with SMA. Below are just a few highlights and outcomes from last year.

## ADVOCACY:

Increased the number of states screening newborns for SMA to 38, accounting for



**2,400+** advocacy actions taken and **5,800+** messages delivered to federal and state legislators in support of SMA community advocacy priorities.

## RESEARCH:

Cure SMA-funded research led to the publishing of **22 journal articles.**

**1,028**  
RECEIVED

responses for the 2020 SMA Community Update Survey, which helps inform Cure SMA's agenda in support of people with SMA and their families.

## COMMUNITY SUPPORT

**2,457** registrants for 2021 Virtual SMA Conference

**115** virtual programs held, with 22,000+ views of programs to date

**253** Newly Diagnosed Care Packages sent

**520** of original Teen & Adult Support Packages sent

**504** of new Teen & Adult Independence Assistance Packages sent

**327** pieces of equipment sent from our equipment pool

**1,095** COVID-19 PPE Packages sent

## CLINICAL CARE:



Expanded the numbers of participants in the SMA Clinical Data Registry to

**nearly 600,**

including both children and adults with SMA.

**INVESTED**  
**\$2.4 million**

in extending our connected network of U.S. research and care sites through the Cure SMA Care Center Network, Pediatric Neuromuscular Clinical Research Network, and SMA Clinical Data Registry.

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/](http://www.curesma.org/annual-reports-financials/).

# Research Update

2021 VIRTUAL

SMA RESEARCHER

MEETING

## New Clinical Trial Data Released at the 2021 Virtual Researcher Meeting

The 2021 Virtual SMA Researcher Meeting welcomed hundreds of research scientists in the SMA community to two days of sharing, collaboration, and celebration. The goal of the meeting—which included a special session, basic research session, and a session on clinical drug development—was to create open communication of scientific data and accelerate the pace of research. Even in the virtual setting, attendees were able to build productive engagement—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.

This year's Special Session, entitled "Rehabilitation and Regenerative Therapies/Technologies," looked at why and how motor neurons degenerate within the context of low levels of SMN protein and how developmental factors influence this degeneration, both critical for better understanding the pathology of SMA. Furthermore, understanding degeneration may lead to potential therapies aimed at preventing or halting this degeneration, giving insight into therapies aimed at regeneration. The goal of this session was to explore what is known about degeneration and regeneration within the context of neuromuscular diseases and discuss how this knowledge may impact future therapeutic development.

We were also excited to see that, at this year's virtual meeting, several companies released new data from their SMA research programs. The data released covers the entire SMA population—from infants to adults—and includes different therapeutic approaches, including those that target the underlying genetic cause of SMA and those that work on the muscles affected by SMA. Clinical trial updates included:

- Scholar Rock shared an oral presentation of its TOPAZ Phase 2 trial results, showing that treatment with apitegromab in conjunction with nusinersen in patients with Types 2 and 3 SMA led to meaningful motor function improvements of up to 20 points as measured by the Hammersmith Functional Motor Scale Expanded (HFMSSE). A muscle-directed approach such as apitegromab, a selective inhibitor of myostatin activation, has the potential to complement SMN up-regulators and address motor function impairments in patients with SMA.

- Data updates from Genentech included preliminary efficacy data from RAINBOWFISH, a study evaluating Evrysdi in babies from birth to 6 weeks with pre-symptomatic SMA, showed that infants treated for 12 months achieved age-appropriate motor milestones of sitting, standing, and walking, and improvements in motor function. Then, data from JEWELFISH, evaluating the safety of Evrysdi in people aged 1 to 60 years who have been previously treated with another SMA-targeting therapy, showed the safety profile of Evrysdi and increase in SMN protein levels are consistent with those observed in other Evrysdi studies.
- Biogen shared new research supporting the continued development of an investigational higher dose of Spinraza and additional data reinforcing the strength of the drug's clinical profile for people with SMA over time. Data from two studies in children with infantile-onset SMA suggest a higher dose of Spinraza may lead to a clinically meaningful increase in the CHOP INTEND score beyond that of the 12mg approved dose. Another analysis of data from the NURTURE study showed that 92 percent of patients who initiated Spinraza treatment as pre-symptomatic infants maintained the ability to swallow after a median of 3.8 years.
- Novartis shared Zolgensma data from the completed two-copy cohort of the Phase 3 SPRiNT clinical trial, which demonstrated age-appropriate milestone development in presymptomatic children with SMA without respiratory or nutritional support of any kind, and with no serious, treatment-related adverse events. The completed Phase 3 STRiVE-EU trial demonstrated rapid improvements in motor function following treatment with Zolgensma, and most patients achieved motor milestones not observed in the natural history of SMA Type 1.

*Cure SMA appreciates the sponsors that made the 2021 Virtual SMA Researcher Meeting possible, including Presenting Sponsors, Biogen, Genentech, and Novartis Gene Therapies, as well as Platinum Sponsors, Cytokinetics and Scholar Rock.*



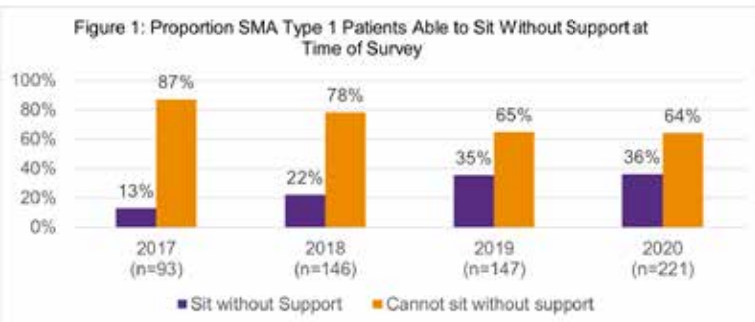
# Cure SMA Launches 5th Annual Community Update Survey

For the past five years, Cure SMA has been working to collect data and information on the SMA community's experiences and daily challenges through our Annual Community Update Survey. Each year, every individual with SMA—as well as their families, loved ones, and caregivers—are invited to complete a survey that will help Cure SMA understand the evolving landscape and unmet needs of the SMA community so we can help to best address them. The questions in the survey cover a range of topics, including demographic information (e.g., sex, age at survey, vital status, educational level, employment), clinical disease characteristics (e.g., age at diagnosis and symptom onset, SMN<sub>2</sub> gene copy number, etc.), family/medical history, respiratory interventions, current motor function, surgeries, hospitalizations, participation in clinical trials, treatment, and quality of life assessments.

Data from the survey informs Cure SMA's advocacy agenda and has been featured in state fact sheets, legislative support statements, and educational outreach to federal and state leaders to showcase the priorities and needs of the SMA community related to newborn screening, employment, transportation, community living, and healthcare. Results from the survey were also included in the Critical Path Innovation Meeting package, which was used as part of a virtual meeting with the U.S. Food and Drug Administration (FDA) in the summer of 2020, helping to enhance the FDA's understanding of the most significant unaddressed needs for treating children and adults with SMA. This included data on both an increase over time in the ability of Type 1 patients to sit unsupported (Figure 1) and a decrease in hospitalizations across all SMA types, along with treatment updates from over the past several years.

The results from the 2017 and 2018 Community Update Surveys were published in a manuscript entitled, "The Cure SMA Membership Surveys: Highlights of Key Demographic and Clinical Characteristics of Individuals with Spinal Muscular Atrophy" in the *Journal of Neuromuscular Diseases* in 2020. The purpose of this paper was to summarize the results the demographic and clinical data from 1,522 surveys collectively received from the first two years. A second manuscript, also published in 2020, in the *Orphanet Journal of Rare Diseases*, entitled, "SMA: A baseline dataset from the Cure SMA Community Update Survey," highlighted the patient reported outcomes (PROs) that were collected in the 2019 Community Update Survey with the goal of providing baseline quality of life data for use in future studies that can measure year-over-year changes from new therapies and improved care.

On April 19, 2021, the 5th Annual Community Update Survey was launched and closed on June 18, 2021. Data is currently undergoing analysis and is planned to be presented at upcoming congresses.



**Acknowledgement:**

Funding for the 2019, 2020, and 2021 Community Update Survey was provided by members of the 2020 Cure SMA Industry Collaboration (SMA-IC), which included, Genentech/Roche, Novartis Gene Therapies, Biogen, and Scholar Rock. The SMA-IC was established in 2016 to leverage the experience, expertise, and resources of pharmaceutical and biotechnology companies, as well as other nonprofit organizations involved in the development SMA therapeutics, to address a range of scientific, clinical, and regulatory challenges more effectively. It is currently comprised of our partners at Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, and SMA Europe. Additionally, the work of the Collaboration is supported by funds provided by Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, and Scholar Rock.

# Community Support Update

## Cure SMA Virtual Engagement Events Continue this Fall

Over the past year and a half, Cure SMA has held a variety of virtual community engagement events, which have allowed the SMA community to stay connected and provided a space to come together. We are happy to share that many of these virtual programs will continue into the fall and winter months. If you have not attended a virtual engagement event yet, please visit [www.curesma.org/virtual-community-engagement-events/](http://www.curesma.org/virtual-community-engagement-events/) to register and learn more.

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 new program provides personal care assistants (PCAs) of adults with SMA three complementary 30-minute live video sessions with a licensed therapist through the online therapy company, Talkspace. 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, and register today to learn more!





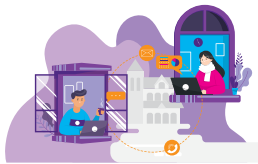
## Physical Therapy Webinar Series

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 will continue to host our Wellness Webinar Series, sponsored by Genentech. 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. Register today to learn 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!

## You Tube

*If you missed any of our virtual education events over this past year—including wellness, physical therapy, and career webinars—you can catch the recorded presentations on the Cure SMA YouTube Channel.*



## Get a LifeVac Home Kit

LifeVac is a non-powered, non-invasive, single-use only airway clearance device developed for resuscitating a victim with an airway obstruction. Cure SMA is offering this support program to all individuals with SMA to help in the event of a choking emergency. 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 <http://events.curesma.org/LifeVac> to request one at no charge.

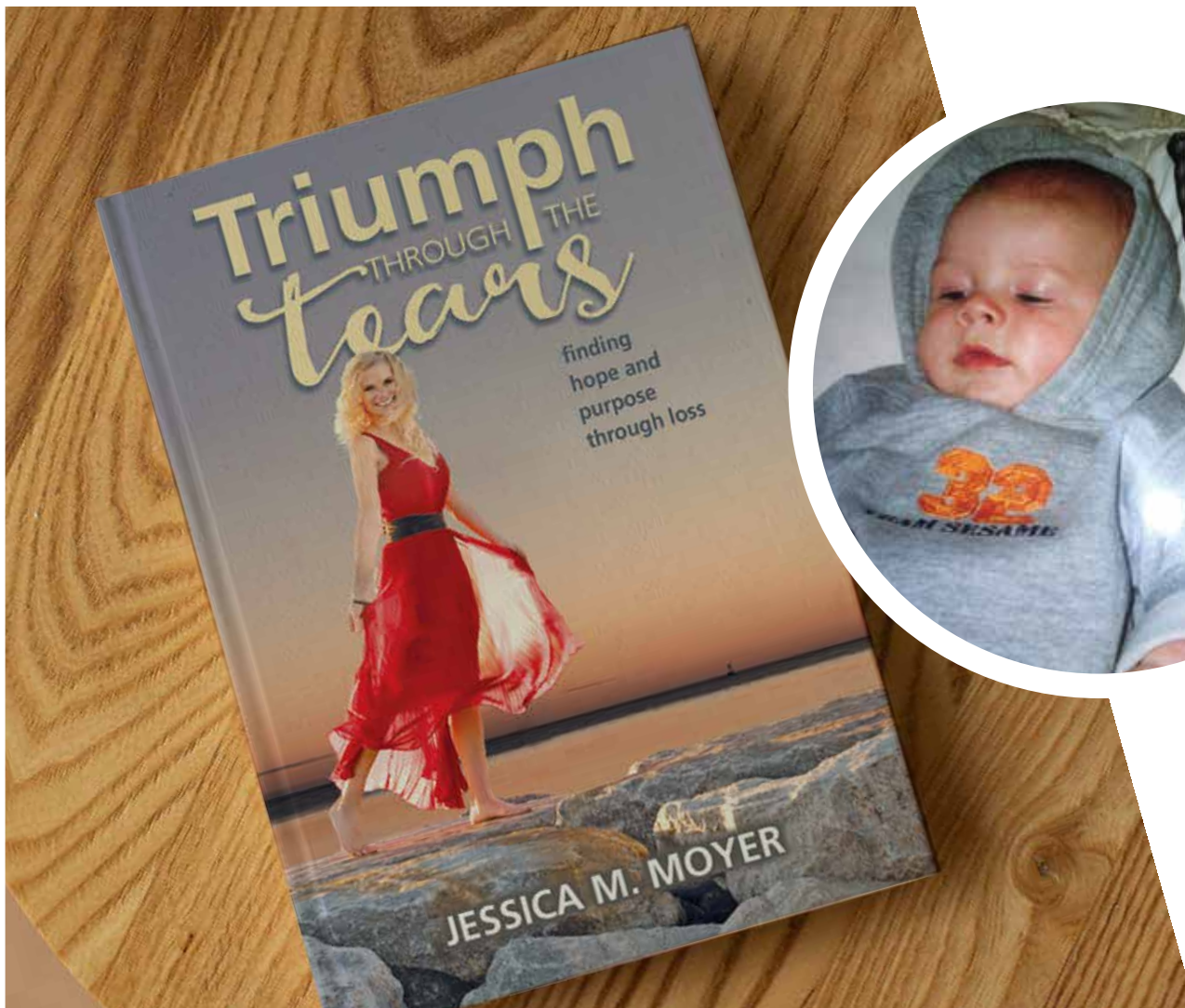
**cure**  
**SMA**

Make today a  
breakthrough.

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

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

*Photo of Tonya Willingham and her daughter, Hanna*



“Triumph through the Tears: Finding Hope and Purpose through Loss,” is a compelling and true story of an ordinary woman who experienced extraordinary traumas and losses in her life. Through the pain and darkness, she was able to rise above and turn the devastation into her life’s purpose. This book is for anyone who is searching for hope despite the tears, trials, and sorrows they may be experiencing.

Jessica Moyer offers a candid look into the use of the “five intrinsic gifts (or 5G framework) to improve your mindset and tangible ways to turn your pain into purpose and seek the light. She will guide you in improving your mental and physical health, while also navigating the difficult and seemingly lonely times of grief. She offers hope you will need for bright days before you. This is your opportunity to open the pages and be inspired to live purposefully triumphant!

“Triumph through the Tears: Finding Hope and Purpose through Loss” is available on Amazon.

“After several years of just getting by in life after losing my son Steven to SMA type 1, and then after two in utero losses, I needed to share my story of hope through loss and growth through grief. This book is my personal journey of the world of grief after losing my son, and how over time I found ways to implement change using my ‘5G framework.’ The use of our five intrinsic gifts of gratitude, generosity, growth, grit, and grace can turn our world from suffering and pain into hope and purpose. Open the book and be inspired that you, too, can find triumph through your tears.”

**About the Author**

Jessica Moyer is a heartfelt national speaker, coach, and author. Her passion and purpose to help women live fully through embracing their health (mentally, physically, emotionally, spiritually) has led her to open a boutique wellness studio in Delaware. In addition, she is the owner of Purposeful Life & Wellness Coaching to help people find hope through loss and grief. Her passion to help her community is evident in her involvement in many different charities, including President of Cure SMA Delaware/South Jersey Chapter, participant of Dancing with Delaware Stars to benefit Mom’s house and Boys & Girls Club, among others.

## SMA Community Supports Another Successful Virtual SMA Conference

Thank you to everyone who attended the recent 2021 Virtual SMA Conference. It was an impactful week of workshops, networking, and SMA community engagement.

This year's virtual conference week—the second virtual conference during these pandemic times—hosted nearly 2,500 registrants from over 65 countries. Cure SMA offered more than 50 sessions, including the SMA Research & Clinical Care Meeting, which received almost 7,800 live household views, a number which grew to nearly 10,000 views in the weeks following the meeting. Although we were unable to come together in-person, it was such an honor to have individuals with SMA, their families, clinicians, and researchers virtually attend our events from around the world.

If you couldn't tune in for the live events of the Virtual SMA Conference, we've got you covered! Many of our sessions were recorded and are now available to view on Cure SMA's YouTube channel. Check out the latest on the "2021 Virtual SMA Conference" playlist.

### Conference Overview

The 2021 Virtual SMA Conference kicked off on Monday, June 7, with the Opening Session and Newly Diagnosed Virtual Session. That evening, we hosted three of our 13 social events offered throughout the week – the Newly Diagnosed Mingle, Grandparents Networking Social, and Adults with SMA Trivia Night. This set the stage for a week of education and engagement.

Family workshops kicked off on Tuesday, June 8, and ran through Friday, June 11, covering topics such as Reproductive Options for SMA Families, Sleep Problems in SMA, Mental Health and SMA, Optimizing Telehealth, and much more!

We also hosted an array of additional socials throughout the week, including Mom's Night In, Dads' Night In, Teens with SMA Trivia Night, Adults with SMA Evening Social, Kids & Teens Talk It Outs, and a Newborn Screening Social. These special events provided opportunities for attendees to gather in a friendly online setting to socialize with their peers.

The 2021 Virtual SMA Conference concluded with an SMA Community Conference Closing Social, which brought together the whole SMA community—individuals with SMA, families, researchers, and healthcare providers—to celebrate the culmination of a valuable week of education and connection. We had almost 100 attendees celebrate the week's success.

This year's Virtual SMA Conference would not be possible without the support from our outstanding conference speakers, whose dedication helped to make this online event a success. Thank you, all!

*We would also like to thank our National Presenting Sponsors for the 2021 Virtual SMA Conference: Biogen, Genentech, and Novartis Gene Therapies. Additional recognition goes out Platinum Sponsors, Cytokinetics and Scholar Rock, Gold Sponsor, Accredo, and Silver Sponsor, Orsini Specialty Pharmacy, for their support.*



**We look forward to coming back together for the 2022 Annual SMA Conference in Anaheim, California, June 16-19, 2022, at the Disneyland Resort, and hope to see you all there!**



**WE LOOK FORWARD TO SEEING YOU  
IN PERSON AGAIN SOON!**





# Why Volunteer for Cure SMA

With more than 6,400 volunteers and over 32,000 hours of service, volunteers are the backbone of Cure SMA. Each year, Cure SMA hosts more than 400 local events, the Annual SMA Conference, and sends out thousands of resources including information packets, care packages, and equipment. Without our wonderful volunteers—made up of chapter leaders, event organizers, conference volunteers, office volunteers, and families—the success of these events and programs would not be possible. We are grateful for our SMA community members and the passion that you give every day through their unwavering dedication to Cure SMA, and there are lots of ways to get more involved and help the community.

If you are interested in learning more about volunteer opportunities with Cure SMA, reach out to us at [volunteer@curesma.org](mailto:volunteer@curesma.org).

Together, we can make a difference in the lives of individuals with SMA and their families. Volunteer today!



## Volunteer Spotlight Nick Farrell

means we are not alone. SMA can be scary and may feel isolating. But knowing there are others out there like us—and drawing upon their support, knowledge, and experience—is truly life changing. I don't like to think about what our lives would be like without Cure SMA."

Nick has been volunteering as a member of the Cure SMA Board for three years. In 2020, he became the Chairperson. "We've got big plans, and the three approved treatments we now have are only the beginning," shares Nick. Emphasizing the importance Cure SMA volunteers have had on the organization, Nick adds "they are directly responsible for the accomplishments our organization has enjoyed, and we could not survive without them."

Nick credits our volunteers for the progress Cure SMA has made in having three FDA-approved treatments, which is uncommon for a disability as rare as SMA. "Each person who gives themselves to our mission multiplies our chances of achievement. And our successes have been nothing short of miraculous, and we only expect them to grow," stated Nick. And yet, there is still much work to be done. Nick shares that while the stakes and expectations are very high, resources are finite. "There really is a direct correlation between our volunteers and the outcomes the organization can generate."

You might know Nick Farrell as the newest Chairperson of the Cure SMA Board of Directors. But he is also an SMA Dad. Nick and his wife Kacey have three daughters ages 6, 10, and 12 years. Nick became part of the SMA community when his middle daughter, Blake, was diagnosed with SMA Type 2 at 14 months of age. Soon after her diagnosis, a local family introduced the Farrells to Cure SMA, and within days, they received a newly diagnosed care package. It was not too long before the Farrell family got involved with Cure SMA, hosting an annual golf event while also participating in local Walk-n-Rolls and chapter activities.

As Nick reflects on his time as part of the SMA community, he highlights the strong bond Cure SMA offers individuals with SMA and their families. "For us, being part of the community

# Great Gift Ideas

## Cure SMA Merchandise

### CURE SMA TRAVEL MUG

Take our new Cure SMA mug to go inside this double-wall travel tumbler. The plastic liner and stainless-steel outer wall help keep your beverages from getting cold.



### CURE SMA PURPLE WATER BOTTLE

This 36 oz plastic sport water bottle is great for taking on the go! This transparent purple water bottle is BPA-free and features the Cure SMA logo in white as well as a screw-on, tethered black lid.



### CURE SMA AWARENESS KIT

Looking for ways to spread SMA awareness? Our new Cure SMA awareness kit is just for you!

#### Kit Includes:

- (25) Cure SMA Bracelets
- (25) Cure SMA Temporary Tattoos
- (25) Cure SMA Fact Cards
- (10) Cure SMA Pens
- (5) 2.25" Square Purple Cure SMA Logo Sticker
- (5) 2.25" Square Orange Cure SMA Logo Sticker
- (5) 2.25" Square Pink Cure SMA Logo Sticker
- (5) 2.25" Square Blue Cure SMA Logo Sticker
- (5) 2.25" Square Black Cure SMA Logo Sticker
- (5) 2.25" Square Light Purple Cure SMA Logo Sticker
- (1) Cure SMA Vinyl Decal
- (1) Canvas Cure SMA Zippered Pouch



### CURE SMA FACT CARDS (PACK OF 25)

These Cure SMA fact cards feature 6 facts about SMA and are perfect to throw in your wallet and pass out to people who aren't familiar with SMA!

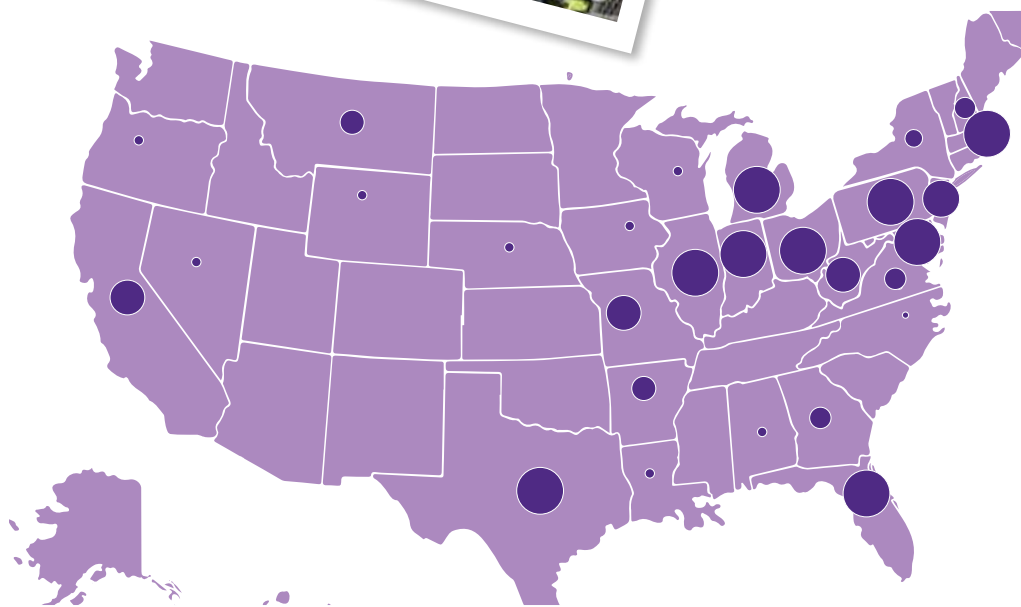
Check out even more items available on our online store at [www.curesma.org/merchandise](http://www.curesma.org/merchandise) to support our mission and raise awareness by purchasing some Cure SMA merchandise.

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

# 2021 WALK - N - ROLL



Although we were unable to come together in-person for our Spring Walk-n-Roll events, the SMA community still rallied together for our Spring 2021 Virtual Walk-n-Roll. Throughout the spring, friends and families took part in various challenges and activities to celebrate their efforts, have fun together, and fundraise for Cure SMA. All activities culminated on a Walk-n-Roll Virtual Celebration on June 5, 2021, after which participants and teams celebrated in their own unique ways.



Together, we raised more than

**\$765,000**

for the SMA community

## Top 5 Teams & Top 5 Individual Fundraisers for Spring 2021 Virtual Walk-n-Roll

### TEAMS:

- Team Murphy
- Pete's Philadelphia Eagles
- Mac's Pack
- Team Emma and Nick
- Caitie's Heroes

### INDIVIDUALS:

- Allyson Henkel
- Eileen Brown
- William Aguilera
- Heidi Johnson
- Silvia Murphy

- Nearly 550 participants registered for 17 different Walk-n-Roll events
- Hosted 92 total Walk-n-Roll teams
- Celebrated 30 participating sponsors





## Champions Club

We would also like to thank and congratulate the following Spring Walk-n-Roll participants, who became the first members of the Cure SMA Walk-n-Roll Champions Club! This is a new program that recognizes and rewards individual participants who raise at least \$1,000 at their respective Walk-n-Roll event. These individuals are helping to lead the way for fundraising for Cure SMA.

The good news is that there is still time for you to become a member of the Walk-n-Roll Champions Club by participating in one of our Fall Walk-n-Roll events and fundraising through November 30, 2021. A full list of our Fall events can be found at [www.curesma.org/walk-n-roll-program/](http://www.curesma.org/walk-n-roll-program/).

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

William Aguilera: \$8,200	Gretchen Dorer: \$2,730	Beth Lockwood: \$2,130	Jahden Riner: \$1,745
Melissa Becker: \$3,135	Kathryn Farrell: \$1,045	Elizabeth Lockwood: \$1,601	Joseph Scully: \$6,500
Courtney Bennett: \$5,000	Blake Farrell: \$1,000	Barbara Jean Mirabile: \$3,615	Kate Sohl: \$2,375
Liza Brooks: \$2,000	Carrie Graves Capone: \$2,050	Katie Mirabile: \$1,000	Lauren Solimine: \$1,050
Eileen Brown: \$11,110	Allyson Henkel: \$15,230	Silvia Murphy: \$7,435	Pam Swenk: \$1,275
William Chu: \$1,235	Heidi Johnson: \$8,050	Brian Murphy: \$2,000	Lynne Vaudry: \$1,650
Susan Davis: \$1,750	Robert Kilo: \$3,000	Johanna Murphy: \$1,650	Leah Young: \$5,100
Devon Decker: \$2,475	Lisa Kruchan: \$1,125	Krista Nunemaker: \$5,255	

# 2021 National Partners

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

## National Premier Partners



## National Platinum Partner



## National Gold Partners



## Resources and Communications Partners





# DO IT YOUR WAY

FUNDRAISING

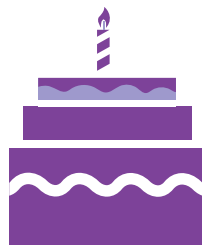
## Do It Yourself Fundraising Allows You to Make an Impact Your Way for Cure SMA!

There is no one way to help raise awareness of SMA and fundraise for Cure SMA. Whether you're thinking about fundraising in honor of your birthday or a special occasion, participating in an athletic event, or starting your own e-Sports tournament, we've got you covered! The ideas below are only a few ways you can fundraise your way for Cure SMA. We have seen the SMA community get so creative, and you can join in the fun.



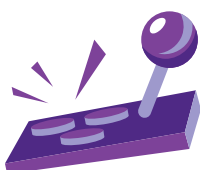
### Host a Community Event

Bring your community together for a bigger purpose. These events can be as big or as small as you would like. A bake sale, movie night, community walk, virtual wine tasting, and even youth fundraising events are simply a few of the unlimited ideas possible to raise money.



### Donate Your Birthday

Your birthday is a time where all eyes are on you! Take advantage of the attention and use this moment to raise awareness of SMA and funds for Cure SMA! Set up a Facebook Fundraiser or Personal Fundraising Page and ask your community of friends and loved ones to donate in honor of your special day.



### Take It Online with e-Sports

Are you interested in taking your event to the internet? The best part about e-Sports is that you can host participants and spectators from anywhere. The new virtual gaming world is full of possibilities and Cure SMA wants to be a part of your virtual impact. If you are hosting your event on Twitch, check us out on Tiltify at [tiltify.com/cure-sma](http://tiltify.com/cure-sma) to make fundraising easy as 1, 2, 3



### Celebrate a Special Occasion

Birthdays are not the only reason to celebrate. Set up a personal or Facebook fundraising page to ask for donations in lieu of gifts for any special event or achievement in your life (e.g., anniversaries, graduations, weddings, etc.). These pages make it easy to share why Cure SMA is important to you.



### Host an Athletic Event

Feeling active? Raise funds by creating an athletic event! Gather family and friends for a socially distanced athletic event and donate the proceeds to Cure SMA. From a cornhole or golf scramble to biking or swimming, the sky is the limit!

Ready to get started?

Email [diy@curesma.org](mailto:diy@curesma.org) and we'll set you up for success!



# Making Moves with Team Cure SMA

Have you checked out Cure SMA's endurance race fundraising program? You can make moves with Team Cure SMA by joining us this season. Team Cure SMA is an endurance program that connects runners and cyclists with Cure SMA. Over the past few years, Team Cure SMA athletes from across the country have trained for endurance events while also raising money and awareness of Cure SMA.

Sign up as participant in a marathon, half marathon, 10K, or 5K race. Team Cure SMA race participants—veterans and newbies alike—can expect brand new endurance gear, comprehensive training programs, and fundraising guidance every step of the way.

- **GEAR:** New Team Cure SMA apparel will have a refreshed look for 2021, and include new, dry-fit racing shirts, fanny packs, fandanas, and more! These items will only be available to Team Cure SMA 2021 race participants.
- **TRAINING:** Whether you are walking, running, or rolling your way through a new endurance challenge, Cure SMA will provide the training schedule and support needed to help you reach your goal. You are not alone if this is a new endeavor.
- **FUNDRAISING:** Participants will receive tips to help maximize their fundraising efforts, including templated emails to share with connections, graphics to help promote your efforts on your social channels, and ideas to get your community involved.
- **COMMUNITY:** Participants will be a part of an online community of support to make sure they are reaching both their fundraising and fitness goals every step of the way!



## 2021 ASICS Falmouth Road Race

Congratulations to Rachael Brown, Sanjiv Lal, Matt Pembroke, Lin Lavallo, Patrick Lavallo, Kimberly Muoio, Michael Melia, Charlene Maison, Katherine Buerger, Tricia Pembroke, Joseph Bierwirth, Christopher Bierwirth, Annelie Benson, and Tricia Michalovicz for training, running, and fundraising for this annual event. Together, they raised more than \$30,000 for Cure SMA!

## Upcoming Events for Team Cure SMA

Atlantic City Marathon  
10/16/2021

Detroit Marathon  
10/16/2021

San Diego Rock N' Roll  
Marathon and Half Marathon  
10/23/2021

Flying Pig Marathon  
10/31/2021

CNO Financial Indianapolis  
Monumental Marathon  
11/6/2021

Allstate Hot Chocolate  
15k/5k Chicago Race  
11/7/2021

TCS New York City Marathon  
11/7/2021

Interested in running for Cure SMA? Please contact Alyssa Zavislak at [alyssa.zavislak@curesma.org](mailto:alyssa.zavislak@curesma.org) with questions or for more information.

# Thank You

# MILLION DOLLAR MOVEMENT

In 2021, the SMA community and its supporters set out to walk, roll, run, and fundraise its way to help Cure SMA achieve its goal of raising \$1,000,000 in the first six months of 2021. And thanks to the support of the SMA community, sponsors, donors, and volunteers – we did it to the tune of \$1,118,392!



Thank you to all who supported this incredible collective effort to continue funding research breakthroughs, offering life-changing support, and empowering the SMA community to meet the challenge! We could not do it without you and we're grateful for your continued support!









# Thank you!



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

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



**Make today a breakthrough.**

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



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



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



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



[youtube.com/user/FamiliesofSMA1](https://youtube.com/user/FamiliesofSMA1)



[www.linkedin.com/company/families-of-sma](https://www.linkedin.com/company/families-of-sma)



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

### Change of Address

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

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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.

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Cure SMA  
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# 2022 ANNUAL SMA CONFERENCE

JOIN US IN ANAHEIM, CA!  
THURSDAY, JUNE 16 – SUNDAY, JUNE 19, 2022

WE LOOK FORWARD TO SEEING YOU  
AT DISNEYLAND NEXT JUNE!

## STAY TUNED

to the Cure SMA website for more details on the  
2022 Annual SMA Conference as they become available.

Cure SMA is excited to reunite the SMA community for the 2022 Annual SMA Conference in Anaheim, Calif., from Thursday, June 16 – Sunday, June 19, 2022. Additional conference details will be announced in the upcoming months and registration will launch in the fall of 2021.

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

