

DIRECTIONS

COMMUNITY NEWSLETTER | FALL 2020



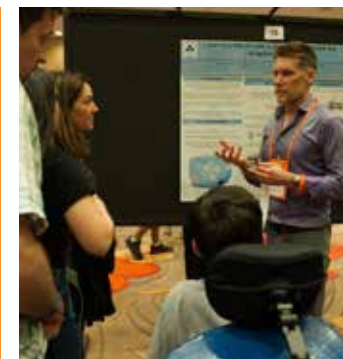
THE SMA COMMUNITY
IS ALWAYS CONNECTED

2021

Annual SMA Conference

THURSDAY, JUNE 10 – SUNDAY, JUNE 13, 2021

JOIN US IN AUSTIN, TEXAS!



If the unexpected circumstances of 2020 have shown us anything, it is how impactful this one week of the year is for families, individuals, researchers, and medical professionals in the SMA community. We cannot wait to welcome you back as we gather next June in Texas. The JW Marriott Austin will host the 2021 Annual SMA Conference and SMA Research & Clinical Care Meeting. The hotel is centrally located in Austin's lively downtown neighborhood with a variety of restaurants, live music, and entertainment venues.

Cure SMA is excited to reunite the SMA community for the 2021 Annual SMA Conference in Austin, Texas from Thursday, June 10 – Sunday, June 13, 2021.



STAY TUNED

WE LOOK FORWARD TO SEEING YOU IN AUSTIN NEXT JUNE!

to the Cure SMA website for more details on the 2021 Annual SMA Conference as they become available.



If you have any questions, please contact conference@curesma.org.

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

CORRECTIONS CORNER:

We have a correction from the Spring 2020 issue of Directions. On the cover, we did not mention Hannah Aylward in the photo with Shane Burcaw. We would also like to specify that the photo of the Scurria Family only included Anna Rose and Krista Scurria.

As the SMA community remains focused on keeping safe and healthy throughout the ongoing global pandemic, Cure SMA has moved quickly to launch a series of virtual programs that are bringing us together for education and social engagement.

Our first-ever Virtual Annual SMA Conference and SMA Research & Clinical Care Meeting was a great success, uniting more than 6,600 participants from 77 countries around the globe. This engagement has continued with our Virtual Summit of Strength Webinar Series and the recently launched virtual community social events. Along with other financial and physical support packages, we hope that these programs are helping to keep the community connected during these times of isolation.

We also continue to make progress in the SMA community, despite all that is happening around us. August marked another important milestone for SMA, with the broad FDA approval of Evrysdi. Along with Spinraza and Zolgensma, we now have three disease modifying genetic treatments available. Cure SMA will continue our work with our partners and healthcare professionals to ensure rapid and broad access to these treatments.

Our next key goal in research is to advance a symptomatic and muscle-targeting treatment that will work in combination with a disease modifying genetic therapy. Additionally, the progress in implementing newborn screening for SMA across the U.S. has been accelerating. We are now on track to have over 70 percent of all births screened by the end of the year, and hope to reach over 90 percent by the time of the conference next year. Finally, looking to the future, we are continuing to build our networks that will provide more local support and care programs to make the most of our drug treatments.

Thank you for all your efforts and ongoing support, which has made this progress in SMA possible.



Kenneth Hobby
President

ADVERTISEMENT

THIS
MOMENT
COMES
FROM WITHIN

ASHLEY // AGE 7

LATER-ONSET SMA, TREATED WITH SPINRAZA



STEPHEN // AGE 38

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.

Victories are personal for the **11,000+** who have been treated with SPINRAZA worldwide.*

For US individuals taking SPINRAZA:

40%

>40% of patients taking SPINRAZA are adults*

3-80
DAYS YEARS

Has treated SMA in patients 3 days[†] to 80 years old^{‡§}

90%

>90% of patients who started SPINRAZA remain on treatment[§]

*Based on commercial patients, early access patients, and clinical trial participants through June 2020.

[†]Includes clinical trial patients.

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

[§]Based on commercial patients in the US (including Puerto Rico) through June 2020.

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.

**CAMERON // AGE 4**

EARLY-ONSET SMA, TREATED WITH SPINRAZA

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

[Learn more at SPINRAZA.com](http://SPINRAZA.com)

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 or 1-844-4SPINRAZA (1-844-477-4672).

MANUFACTURED FOR

Biogen, Cambridge, MA 02142



Letters from the Community



Thank you, very much for the gifts. We are very happy with everything that came to us.
~Angélica Martínez

Thank you for the care packages! My daughter was so excited! We are going to bring a goodie to each of her treatments, which helps a ton. Thank you again!
~The Black Family



My husband and I have felt like a dark cloud was hanging over our heads all week...but let me tell you, our son has NOT stopped smiling since he opened this box! It has filled our home with so much joy and it was completely unexpected! Thank you so so much for the surprise box and all of the info you sent! We needed a really positive moment. And honestly, from our new neurologist to the Spinraza drug rep to CureSMA.org to the rest of his SMA squad, everyone has been so incredibly helpful and warm. It's made a huge difference. We are blessed to be taking this journey with such genuine, caring people.

Thank you again!
~ The Gonzales Family



Thank you, so much, for the huge box of toys. I played with them all morning and I loved the kinetic sand and the bubble maker. I appreciate that you gave this to me and a lot of other people. I hope everyone loves the toys.

~ Mickey Longo



When I responded to your offer to send a support package, I honestly thought it would consist of a few brochures and small items like grippers to open jar lids, etc. Perhaps you can imagine my surprise at the amazing bounty of high-grade items contained in the package! Thank you so very much for such wonderful aids to improve quality of life. Your care and kindness is much appreciated.

~ Susan Burke

Thank you so much for the very generous gift box! Both my kids were on cloud nine. It was like Christmas morning. They played with the toys all night. The sink and food were by far their favorite! Henry was so excited to go through it all. This box made our week. We are so very grateful!! He will have a nice blanket and pillow to bring with him to his second dose of treatment later this week. Thank you again!
~Jenny Yuede



I just wanted to write to say thank you for the COVID-19 support package you sent me. These are strange times for everyone. Going back to school is bringing with it a lot of nerves and some of the items you sent will be very helpful to combat those nerves. Thanks for always being so supportive to those of us living with SMA.

~ William Johnson



Eliana is doing great. Cure SMA helped greatly with the COVID-19 package, Amazon card, and equipment pool that sent a bath chair. You all are so appreciated!

~Tiffany Allemand



Our family received this SMA care package today. There are no words! Thank you does not seem like enough. Our little guy, Jaxon, loves everything and we are beyond appreciative. We vow to pay this blessing forward!

~ Jennifer, Michael, and Jaxon Adams



Grayson has received his care package and loves all the goodies inside; he especially loved the flags. We will be able to use what came in this care package for quite a few years. Thank you! We are blessed to have found such an amazing support system.

~ Steven Hoskins

My name is Seth Marlowe and I am 21 years old. I have SMA Type 1. I wanted to thank you for the support package. Everything in it is so nice and thoughtful. My family and I are so appreciative. I especially love the support pillow and fleece. I am also able to put my iPad on the table arm. Thank you so much for all my gifts!

~ Seth Marlowe





Thank you all so much for the amazing package you sent my son, Jace! He loves playing with the kitchen sink and food the most out of all his toys! Also, since receiving the package, we also got a wagon from you and he loves it! I'm glad we have you all as a resource as we go through this journey and look forward to meeting in person in the future.

~ Elizabeth Parker



Thank you, Cure SMA! I really appreciate all the gifts. It was quite a surprise. I really like the EZ shampoo basin, Sisu positioning pillow, remote control electrical plugs, medical fleece sheepskin, and Echo plus. My speech therapist programed my communication device with Alexa commands so I can do many things I could not before. I'm having a lot of fun with it.

~ Matthew Freitas

Thanks so much for the amazing care package you sent us. My wife, Michele, is very anxious to try everything contained therein. She is excited about using the Echo Plus. We are also looking forward to using the Medical Fleece Ease Sheepskin Bed Pad. Same with the recovery pillow. We are looking forward to exploring the remote outlet switches and the neck/shoulder wrap. Oh yes, she has already started using the extendable back-scratcher. Thanks again! Cure SMA is certainly a wonderful organization!

~ Tom and Michele Cappels



First of all, thank you for your email and supporting us. When I heard the first time about SMA, it was painful for me to accept. Second, we started searching about SMA immediately. Saying a million thanks is not enough to Cure SMA. We learned everything from you guys. We are so happy now. We have never lost our hopes. Hopefully everything will be great for our Luna and other children who are affected with SMA. We received the wagon and a big box of goodies. They are lovely and very useful, especially the mirror toy, which Luna gives a lot of attention to.

~ Filiz Garcia

I wanted to personally thank you guys for the original care package I received late last year that included the Echo, Sheepskin, Snake Grabber, etc., and the package I received for COVID-19. I have found everything extremely useful, especially in the current time. Once again, thank you for helping make my life a little bit easier.

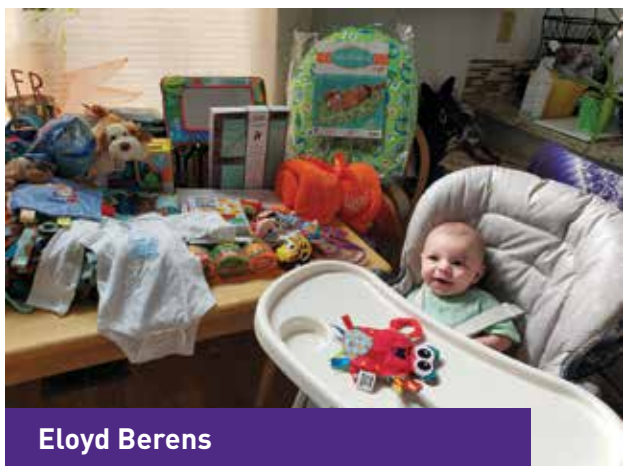
~ Chris Wolf



Brylee and Henry Yuede



Micky Longo



Eloyd Berens



Sofia Wylie



Maximus Scheratski



Juany Martinez



Rhys Devine



The Lasko Family



Rose Guarino



The Soriano Family



Daniela and Santiago Noboa



Terri-Lynn and Jewel-Ann Glucksman

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



E-Learning During a Pandemic: We Got This!

By Angela Wrigglesworth, SMA Community Member and Teacher

I have been an educator for more than 20 years, helping to shape the good kids of Houston. I am a bit of a “student whisperer” (if I do say so myself)—at least for the third graders I teach.

Throughout my years in the classroom, I have learned that SMA helps me establish bonds with my students, especially some of the more challenging ones. They all want to help me, and I use it to my advantage. I have an abundance of teacher assistants! Because SMA affects me physically, I have had to use technology to help me navigate the classroom, and I am often supported by a teacher’s aide to help me complete the bigger, more physical tasks. And, like most teachers in America, I work from home at least a couple hours on weeknights or weekends, grading assignments and completing other administrative tasks.

But, wow, did 2020 throw me for a loop—me and the rest of the world. Within days, the entire education system pivoted, and we integrated to new virtual platforms to continue our curricula as best we could. My students’ technology skills blew me away. It made me wish I had been implementing even more technology in my face-to-face classroom all these years. And after finishing the spring semester, we set our sights on how to make the next school year even more fruitful.

This year, I will have a stronger focus on SEL (social-emotional learning) in my virtual classrooms. My team has set aside a good chunk of time in the mornings to conduct activities that build relationships and foster a closeness that is usually gained with proximity. But to be successful—or if I am being real, to survive—we are going to need to be partners. As an educator, here are my thoughts on how we can achieve this.

- **Set Up for Success:** First, at a very basic level, students need a quiet place to learn, with as few distractions as possible. Much like an unfocused student in the classroom, a child who is playing with their cat on camera can completely disrupt a virtual lesson. Second, the entire school year will be about constant recalibration. Every veteran teacher will tell you that we often spend the first few weeks of every school year focused on procedural expectations—it sets the tone for the entire year. Virtual learning is no different, but the procedures may evolve and for that we need your patience.

- **Encourage Accountability:** No matter how old your child, make sure to check in regularly with the work your student is submitting. Kids need accountability partners and it should be a responsible adult, especially in the beginning, but this cannot be the teacher. Asking your student if they have completed everything is not going to cut it. As micro-managing as it sounds, keeping tabs on students’ work is an essential piece to the partnership between school and home.
- **Be Kind:** Approach the school year as positively as possible. What is the saying, “You catch more flies with honey?” That is not to say you should become a pushover, but a diplomatically worded email goes a long way when voicing concerns to a teacher or administration. We are all building this plane while flying, right? Put everything you can in email, that way you will never find yourself in a he said/she said situation.
- **Listen Up...This is for You, Students:** My advice for students is the same as it always—do not be afraid to ask for help! If you are struggling, speak up. There are a lot of creative people out there who can help solve problems. Also, make sure to move around throughout the day. It is important for us to keep our stamina strong and sitting in front of a computer all day can quickly diminish endurance. Put on some music and dance around. Put on a mask and roll down the street. Whatever you choose, just keep moving!
- **Advice for the SMA Community:** Everyone is recognizing the power of technology and productivity. This is going to open a lot of doors for people with SMA! Walk through those doors and show them what you are made of. There is also a growing feeling of empathy for vulnerable populations. In this case, we are all susceptible to this virus and comparisons will be made to this time in our history from here on out.

People always talk about looking back on a moment of hardship and laughing. When my Zoom call is breaking up while trying to teach multiplication or the proper use of verbs and nouns, it is hard to imagine that day. But I do believe that the use of technology will grow exponentially, even when we get back to the classroom. But for years to come, we will have a new appreciation for being together in-person and for being able to hug, hold hands, and high-five (well, low-five, in my case). Hang in there, world...we got this!

2020
CURE SMA
EVENING OF

H O P E

A VIRTUAL MASQUERADE



Tuesday, October 27, 2020 | 7:30 p.m. CT

Cure SMA is hosting an evening to celebrate innovation and hope, bringing together the SMA community and its supporters from the comfort and safety of their homes. This mission-filled program features recognition, silent auction, plus more!

*To register, visit events.curesma.org/virtualgala
or email specialevents@curesma.org.*

*Missed the event? Don't worry...you can still watch the fun-filled festivities and make a donation today!
Check it out on events.curesma.org/virtualgala.*

**cure
SMA**



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

Check out the updated Cure SMA Care Series Booklets.



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

**Some books available in Spanish*



Perspectives on Working with a Disability

By Spencer Cook, Registered Accessibility Specialist

Living with a disability in the United States has never been easier. Through the ADA and the Architectural Barriers Act, many of the obstacles that made working and participating in society impossible for disabled people have been removed. Medicaid waiver programs provide many with self-directed attendant care, enabling people with all kinds of disabilities to live independently rather than in institutions. I am a grateful beneficiary of these policies and programs. Nowhere in history have people with disabilities been afforded so much freedom and support.

However, as a working person with SMA, I must admit that National Disability Employment Awareness Month inspires a deep ambivalence in me. While I applaud the intention behind the campaign, it fails to address the primary obstacle facing many disabled people who attempt to earn a living: the fact that eligibility for essential services is determined by not only the individual's physical capability but also by their financial need.

Though I expect most people reading this will be familiar with the issue, I'll summarize it here. Unless they can pay for it themselves, many people with SMA or another physical disability will at some point rely on state-funded attendant care. Because attendant care is provided through Medicaid waiver programs, eligibility is based in part on financial need. Once part of a program, they are subject to income limits and liquid asset caps. In Texas, I can make \$2,349 per month. The asset cap, established in 1989 and unchanged since, is \$2,000. But here's the real sticking point: the care I receive by artificially restricting my savings and earnings costs about \$90,000 per year.

As you can see, this puts people who rely on attendant care into an impossible situation. To maintain the benefits that are absolutely essential for our survival, we are forced to accept indefinite poverty. If I were to pay for my own care and maintain my modest standard of living, my income would need to jump from the allowed maximum of \$28,881 to about \$120,000 in a single year; quite a feat for anyone. This gap between what you can make while receiving essential services and what you would need to earn to pay for those services yourself sends a very clear message: do not risk trying.

Quite simply, the eligibility requirements work in direct opposition to the goals of the ADA. Instead of promoting independence and

inclusion, they force dependence. Instead of helping disabled people pursue meaningful, sustaining careers, we are incentivized to remain in positions we have outgrown, if we work at all. Without exaggeration, the struggle and uncertainty around maintaining my eligibility while working has been the single most difficult aspect of my disability I have dealt with as an adult. I have been averse to risks that I probably should have taken. I have succumbed to a lack of motivation, feeling trapped in a hopeless situation. This is a clear instance of policy actually amplifying the effects of disability rather than mitigating them. To live in fear of losing your means of survival is much more difficult to bear than simply being unable to walk.

I am sure someone reading this will suggest that Medicaid Buy-In or ABLE accounts might help. For some people, these are workable solutions and that is wonderful. For many others, however, these options offer only marginal improvements to an unjust situation. Though they do slightly raise the income limits and asset caps, the results are much the same. Quite frankly, you will still always be poor. Saving enough money for a significant emergency will still be impossible.

Though I do work and earn money, I have not even come close to solving this problem for myself. The solutions I have found are precarious at best; I have no guarantee that some administrator won't find an error while reviewing my case and revoke the services on which my independence is based with the stroke of a pen. For now, the best I can do is to work and plan a future within the limitations of the current system. Though I am working towards someday paying for my own care, it is still a way off.

True accessibility entails more than physical access and a lack of discrimination; it is about self-determination. An accessible society is one where people can pursue their lives and careers fully and freely without fear of losing the services on which their lives depend. Until essential services are guaranteed to all regardless of their financial situation, the potential of people with disabilities will always be stifled and work will never be truly accessible.

Are you interested in supporting advocacy efforts to improve access to essential services for people living with SMA? Contact Cure SMA's Advocacy Team at advocacy@curesma.org.



SPOTLIGHT *on* Nicole Stickane Advocating for Newborn Screening in Texas

In late July, the Texas Newborn Screening Advisory Committee held a meeting—virtually, of course—to hear an update on the status of statewide newborn screening for spinal muscular atrophy (SMA). State officials shared updates and members of the SMA community shared public comments. Nicole Stickane shared her family's experience in support of adding SMA to Texas' newborn screening panel, especially given that Texas represents approximately 10 percent of all babies born in the U.S.

Nicole's journey with SMA started 4 years ago. Born and raised in Texas, she and her husband, Daniel, live in Southlake with their three children—Sloan, Luke, and Jordan. Her middle son, Luke, was born in July 2016. At around 3 months of age, Luke started to fuss after feedings and was behind in early developmental milestones.

"We raised both concerns with our pediatrician at Luke's 4-month wellness check," recalls Nicole. "At the pediatrician's recommendation, we thickened his feed to address his feeding issues. But on his milestones, we were told that 'all children develop differently.'"

When Luke was 5½ months old, Nicole took him to a hospital in Dallas to check on his labored, belly breathing and a high fever. She will never forget that moment.

"I was at the side of Luke's hospital bed, where he was still hooked up to oxygen and machines when the head neurologist told us Luke's symptoms presented as a classic case of SMA Type 1, which can be fatal," Nicole remembers. "He did not pause, however, in noting that this was an exciting time given the recent U.S. Food and Drug Administration approval of the first treatment for SMA."

But, truthfully, all Nicole heard at the time was that SMA could be fatal.

In February 2017, when Luke was 7 months old, he started treatment. Today, Luke is 4 years old. He is vibrant, charismatic, funny, and sweet—just like he was when he was a baby. He completed his first year of preschool, where he took the bus to the same school his older sister attends. He loves to be outside and on the move at all times through either his manual or power wheelchair. He especially loves to dance, with "Baby Shark" being his current favorite song.

Nicole believes that SMA has changed her life. She and Daniel started the Luke 18:1 Foundation to help raise awareness, provide funding for research and equipment resources, and to find a cure for SMA, which they do in partnership with Cure SMA.

"Our connection to SMA has taught our family to be more kind, compassionate, and aware of others facing challenges. I am very proud of our family and what it is becoming," says Nicole.

Since Luke started his SMA treatment, he has gotten progressively better with fewer illnesses, fewer hospital visits, and fewer hospital overnight stays. Because of treatment and access to equipment and therapies, such as occupational and physical therapies, the Stickane family feels that they are "climbing out of the darkness of 2016 and into the light of living a wonderful life." Not just for Luke, but for the entire family.

"Recently, I have spent some time helping to urge Texas leaders to speed up consideration and implementation of newborn screening of SMA," comments Nicole. "With early diagnosis through newborn screening and early access to treatment and care, children born today with SMA can live a healthy, independent life. These are all the things I wish for Luke, whose diagnosis, like many, did not come for several months after his birth."

Nicole and Daniel believe there is no doubt that Luke could be stronger physically had he received a diagnosis at birth and started immediate treatment and care. But now the family's goal is to make sure Texas screens every child born in their state for SMA as soon as possible to help save countless families the fear and uncertainty they experienced back in 2016.

"Please speak out and persuade your legislators to speed up the implementation of newborn screening of SMA," encourages Nicole. "Early diagnosis through newborn screening and early access to treatment and care can make a world of difference for these children and their families. It will change people's lives."

Recognize the symptoms and save a life!



EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

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



SMArtMoves.CureSMA.org



CURE SMA TEEN AND ADULT SUPPORT PACKAGE



The Cure SMA Support Package for teens and adults is filled with helpful items that have been recommended by adults living with SMA. Many of the items included have been found to make activities of daily living easier and allow those with SMA to gain independence with certain tasks.

If you are a teen or adult with SMA who hasn't already received this package, request one at no charge by visiting: events.curesma.org/supportpackage.

Arya Singh

Looking to Make a Difference



Resilience and perseverance are human traits that can be hard to teach a young child. For 20-year-old Arya Singh—a junior at Yale University studying the History of Science, Medicine, and Public Health, as well as Education—she realizes that these qualities were on display all her life.

“My grandfather worked in drug discovery, and to me was the true embodiment of resilience and perseverance,” Arya says. “Similarly, my parents started The SMA Foundation when I was diagnosed with SMA Type 3 at 18 months old. They have served as my greatest source of inspiration and hope.”

Born in Hong Kong, Arya was raised in New York City along with her younger brother and younger sister, both of whom she is incredibly close with. “They have been my biggest supporters throughout my entire life, and my brother is even joining me at Yale.”

When Arya was younger, she often felt confused, hopeless, and frustrated. “What started as complete confusion and denial quickly transformed into me asking my parents, ‘Why me?’ almost every day,” Arya remembers. “I couldn’t understand or appreciate that having SMA was not my fault, and I was certain it would lead to a less fulfilling life.”

Now, while there are still times Arya ponders that same question on a particularly hard day, she knows that SMA has given her a sense of perspective that has made her into the person she is today.

“My father used to tell me a Ray Charles’ quote all the time when I was younger,” continues Arya. “He said, ‘You might not be able to do things like a person who can see. But there are always two ways to do everything. You’ve just got to find the other way.’”

Arya believes that by needing to always “find those other ways” she has become more determined and empathetic. SMA or not, she is determined to make a difference in the world and excel at things she cares about.

Arya also realizes that while SMA has made her life undoubtedly different, it certainly has made for just as—if not more—a fulfilling life. She has been involved in the SMA community her entire life, first learning about Cure SMA as a young child in conjunction with her SMA Clinic and The SMA Foundation. More recently, she began receiving care packages and subscribed to the adult newsletter.

But Arya is also looking to take her experiences with SMA and help others, which she has done through the development of her new children’s book, available on Amazon, titled **“Courageous Calla and the Clinical Trial”** (<https://www.courageouscalla.com/>).

“I have grown up in clinical trials for as long as I can remember. While clinical research can be ultimately lifesaving and provide patients with invaluable hope, they can also be confusing, scary, and isolating—especially for children,” says Arya on why she created the book. “I consistently felt alone, and my peers could not understand what I was going through. There was also an incredible lack of resources about clinical research, at least not in a way that was digestible for young people.”

Arya hopes this book—which uses analogies to make cornerstones of clinical research understandable for children—can be a source of empowerment, education, and comfort for young patients in a clinical trial, and even the public.

These days, with no way to travel—as she loves to do with her friends and family—Arya spends time baking, watching movies and reality TV with friends, and studying. After graduation, she hopes to pursue a Master’s in Public Health and law degree with the goal to work in drug development and health policy, specifically surrounding clinical research.





where
science meets **humanity**™

Biogen is proud to continue to support Cure SMA

At Biogen, we are pioneering new science that takes us deep into the body's nervous system, and stretches wide across digital networks and patient communities, to better understand, and preserve, the underlying qualities of our essential human nature.

biogen.com





GET INVOLVED WITH YOUR LOCAL CHAPTER

Cure SMA has 36 volunteer chapters throughout the United States.

Our chapters provide support for affected families through networking, fundraising events, and advocacy. As representatives of Cure SMA, chapter leaders spread SMA awareness in their local communities and generate support for our organization.



VOLUNTEER OPPORTUNITIES ARE AVAILABLE NATIONALLY

Please contact fundraising@curesma.org for more information.

Visit www.curesma.org/chapters

Feeling Strong with a Mind-Body Balance

Contributors: Stacey Tarrant, Becky Hurst-Davis, and Anne Buckley-Reen



As our community approaches winter with a new sense of normalcy, taking care of ourselves with proper nutrition and movement can help boost immunity and sustain us through cold-weather months. Balancing nutrition and movement can be complicated and is different for every person living with SMA. To support, we have compiled these helpful tips and information with help from Stacey Tarrant, RD, LDN; Becky Hurst-Davis, MS, CD, RD; and Anne Buckley-Reen, OTR, RYT.

Movement Through Yoga

Because many people living with SMA have restricted movement, yoga postures offer the opportunity to stretch, elongate, and maintain mobility in all joints. Generally, postures involving extension (arms overhead) are energizing, while postures towards flexion (knees to chest) are calming, and twists (twisting the upper body to look over one shoulder) are organizing.

The general benefits of yoga are extensive and include ease to the mind, body, and spirit. For people living with SMA, Buckley-Reen believes that practicing yoga postures with routine, helps maintain mobility, flexibility, joint integrity and most importantly, comfort with one's body. Yoga postures can also maintain strength and enhance physical and emotional resilience.



Benefits of Yoga

- Influences oxygenated blood flow to the brain and extremities.
- Helps to prevent contractures.
- Supports muscle tone and maintain strength.
- Reduces stiffness and tightness of the joints.
- Supports the elongation of the supportive breathing muscles.
- Strengthens the immune system.



“Think of yoga as a preparation for the day,” says Buckley-Reen. “This begins your day with a sense of calm, improved energy, comfort, and focus. Most yoga exercises or postures can be done in the comfort of your bed upon waking up or before winding down at night and can also be integrated throughout the day while in-chair.” Buckley-Reen also suggests a couple breathing breaks in the day to clear the mind, open the lungs, and energize or calm the body.

The Basic Daily Practice of Yoga

1. Establish quiet environment
2. Warm up exercises or gentle massage
3. Breathing exercises
4. Balanced series of physical postures
5. Guided deep relaxation
6. Circle of Song*
7. Closing postures (spinal twist and seated-forward bend)

**The circle of song is another breathing exercise that invites a circle of communication, builds respiratory capacity, and strengthen lungs. It can also be adapted to meet the person's age and respiratory/speech ability. Humming can be substituted for singing.*

Yoga practices also help the body to establish and maintain homeostasis. This is the state in which the body produces enzymes necessary for the digestion and metabolism of proteins, carbohydrates, and fats—the essentials of a balanced diet.

“Yoga prepares the body as well for digestion and elimination by stimulating the ascending and descending colon—the digestive pathway of elimination. Poses that bring the knees to the chest, as well as the seated forward bends, aide in the digestive process,” says Buckley-Reen.

If you are interested in yoga practices, Buckley-Reen suggests a guided practice by a teacher with experience in working with physical challenges. You can also check out Cure SMA's YouTube channel for Summit of Strength webinars featuring demonstrations of yoga practices for children and adults.

Nutrition

Every person living with SMA is different and has varied nutritional needs based on age, mobility, and activity levels. Considering an individual's circumstance, concerns, symptoms, food preferences, and cultural food practices means each person will have their own unique starting point when it comes to nutrition. But what is the best diet for someone living with SMA?

“There is no single best diet for a person living with SMA because, now more than ever, every person with SMA is truly unique,” said Tarrant. “With the new therapies available to people with SMA and the wide range of abilities and strength that people are achieving, every person's needs are different.” She went on to point out that many in the SMA community may not be receiving treatment or may have started their treatment later in life, and their nutrition needs will also be different.”



Proteins: eggs, milk, chicken, fish, beans, nuts, grains, vegetables
Fats: salmon, hemp milk, plant oils such as flaxseed or canola, avocados
Carbohydrates: whole grain breads, cereals, pastas; beans/legumes; fruits, vegetables

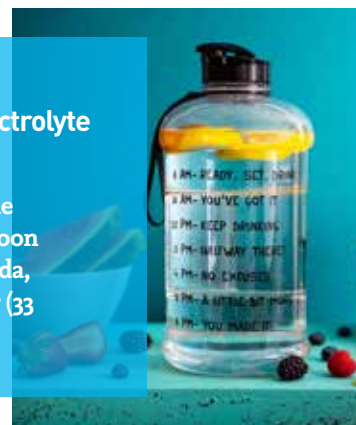
The key, experts say, is balance. Eating a wide variety of foods that are balanced between protein, fat, and carbohydrates help ensure the body is receiving the vitamins and minerals required for optimal function. Common gastrointestinal symptoms like constipation and reflux that people living with SMA experience can also be alleviated with proper nutrition.

“What most people don't realize is that eating healthful foods—fewer processed and sugary carbs and more whole grains, fruits, vegetables and lean proteins—will generally make them feel and move better,” says Hurst-Davis. “The only place that we get energy from is food. We need that energy and proper nutrients to move our bodies and help them function properly.”

Another common question Tarrant and Hurst-Davis receive from people living with SMA is about how to boost immunity to stay healthy during the pandemic. “One path to a strong immune system is to eat as healthfully as possible, including a variety of colorful fruits and vegetables. This will help you maintain a healthy weight and avoid nutrition-related health problems like high blood pressure, heart disease, diabetes, and obesity that have resulted in poorer outcomes with COVID-19 infection,” says Hurst-Davis. “Also, some newer research shows that foods rich with Vitamin D, such as salmon, yogurt, orange juice, or milk can aid in bone health and immunity.”

Maintain Hydration and Electrolyte Balance If Sick This Winter

Drink Pedialyte or a homemade electrolyte solution of ½ teaspoon lite salt, ½ teaspoon baking soda, 2 tablespoons sugar, and 1 liter (33 ounces) of water.





Immunizations Recommendations During Flu Season and COVID-19

Contributors: Dr. Mary Schroth and Dr. Robert Graham

Influenza (or flu) is a serious respiratory infection, and people of any age can get it. Since 2010, the Centers for Disease Control and Prevention (CDC) estimates that influenza has resulted in between 140,000 and 810,000 hospitalizations each year and 12,000 to 61,000 deaths. There is always uncertainty surrounding how severe the influenza season will be from year-to-year. The severity of the season affects the amount of available healthcare resources, and this year is further complicated by COVID-19. The CDC highlights that, because of the ongoing COVID-19 pandemic, reducing the spread of viral respiratory illnesses like the flu this fall and winter is more important than ever.



Symptoms of influenza and COVID-19 can be similar. It is important to identify the symptoms and contact your healthcare provider at the first sign of symptoms. The influenza season occurs primarily in the fall and winter, typically peaking between December and February; however, flu activity is seen as late as May. Getting vaccinated for the seasonal influenza is the best way to prevent it, and the CDC recommends everyone 6 months of age and older receive a flu vaccine every year. Although the flu vaccine will be available throughout the influenza season, to best protect yourself and your family, Cure SMA recommends getting your flu shot by the end of October.

Having SMA can result in more severe influenza infection and complications, due to underlying muscle weakness including respiratory muscle weakness and fatigue, or chronic lung issues related to aspiration. The best way to reduce health complications is to ensure individuals with SMA, their caregivers, and close contacts are up to date on their immunizations. General health maintenance is essential for everyone. In the context of the COVID-19 pandemic, all preventative measures are advantageous.

Individuals with SMA should ensure all their immunizations are current. Pneumococcal vaccines, including Prevnar 13 (PCV13) and Pneumovax (PPSV23), should be up to date, especially for older people with SMA. Pneumococcus is a common bacterium that causes infection in the respiratory system, including pneumonia and ear infections. After receiving the first tetanus, pertussis, and diphtheria vaccine, a tetanus booster vaccine should be obtained every 10 years. Additionally, Synagis (palivizumab) is a treatment used to help prevent serious lung disease caused by respiratory syncytial virus (RSV) in high-risk children under the age of 2 years during the viral season.

Generally, a well-balanced diet will provide the vitamins and minerals your body needs to keep you healthy. Some families and individuals may choose to supplement their diets with naturopathic options, like Vitamin C, echinacea, or elderberry. There is little published evidence of efficacy for these supplements. Be sure to communicate any supplements or medications you are taking with your healthcare team and be wary of anything that seems too good to be true.

If you have any questions regarding this article or recommendation for flu/other vaccination, please reach out to us at patientcare@curesma.org.



Common Flu Signs and Symptoms

Flu signs and symptoms usually come on suddenly. People who are sick with flu often feel some or all these symptoms:

- Fever* or feeling feverish/chills
- Cough
- Sore throat
- Runny or stuffy nose
- Muscle or body aches
- Headaches
- Fatigue (tiredness)
- Some people may experience vomiting and diarrhea, though this is more common in children than adults

**Not everyone with flu will have a fever*

Source: Centers for Disease Control and Prevention



COVID-19 Vaccine Precautions

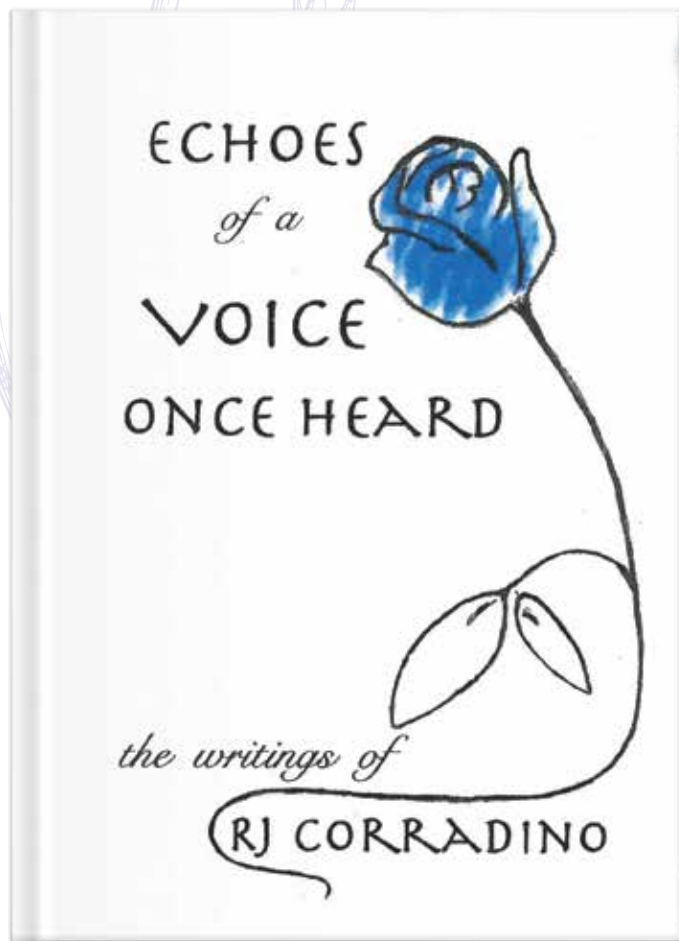
With the potential for a COVID-19 vaccine in the future, all individuals with SMA should take caution before volunteering for vaccine clinical trials. We encourage you to talk with your neurologist about trials and potential interactions with your current treatment protocols.

This consideration is especially important for those considering gene replacement therapy, such as Zolgensma. Some COVID-19 vaccines are using Adeno-Associated Virus (AAV), and Zolgensma uses the AAV9 vector to deliver the SMA gene. Antibodies to AAV can interfere with the effectiveness of AAV-based gene replacement therapies. Additionally, steroids, like those used with Zolgensma treatment, may impact the timing of vaccinations. Vaccines may be delayed while on prednisone, depending on dose and duration. Any new vaccine that contains AAV should be discussed with your healthcare team.

**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.**

The Words of RJ Corradino



It has been said that, “A person is not dead while their name is still spoken. That we are only truly gone when we disappear from the memories of those who loved us.” For Randy and Holly Corradino of Manasquan, NJ, they have found a way to keep the spirit of their son, RJ, alive.

“Echoes of a Voice Once Heard” are the observations and dream life of a poet, RJ Corradino, who died at the age of 20 years from spinal muscular atrophy (SMA). RJ lived his life in a wheelchair. His poems strike at themes of normalcy and the simple things that people not challenged with disabilities take for granted.

In this collection, written between 1996 and 2000, we follow a young man who is finding himself as a writer and who is determined to find success by being published. RJ’s poems were published, sadly just after his passing.

RJ wrote, “I don’t think writing is about power. I don’t feel a desire to create or destroy. My desire is for connections. I write to form connections with people. That’s all.” Throughout the lines of his poetry, RJ weaves the struggles we endure throughout our life, along with life’s celebrations. We do indeed connect with the timeless messages portrayed in RJ’s poetry.

As a student at Richard Stockton College, RJ studied Literature and Creative Writing under Pulitzer Prize winning poet, Stephen Dunn, until his untimely death. Despite his disability and wheelchair dependence, RJ maintained a loving spirit, a sense of humor and a quiet dignity. His poetry lives on as a tribute to his memory.

Among the many reasons why people loved him was that in his wheelchair, with his physical difficulties, RJ never attempted to elicit pity. He asked merely to be taken as he was—an interesting, young man who shared your interests. I admired his intelligence, good humor, and most of all his dignity. At the time of his death, he was writing indeed some very moving poetry.

~ Stephen Dunn, 2002

“By publishing a book of his poetry and essays, and some very heartwarming tributes from family and friends, the book is a celebration of the spirit, of a young man’s desire to live his life to the fullest,” said Holly. “The stories of his intellect, his kindness, his sense of humor are inspiring. It is as if RJ knew his time was limited and he was determined to be the best person he could be. No one ever heard him complain, but they always heard him laughing. He was wise beyond his years and had insights usually reserved for people later in their lives.”

This book will make you sad for one reason only, that you never got to meet RJ.

“Echos of a Voice One Heard” is available on Amazon. All proceeds from the book will go to Cure SMA.

2020 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 Partners



National Gold Partner



Resources and Communications Partners



Cure SMA Merchandise



NEW ACCESSORIES:

- \$25 Embroidered Plush Blanket
- \$15 Hand Blown Glass Ornament
- \$15 Reusable Black Face Mask
- \$15 Reusable Purple Face Mask
- \$15 Reusable White Face Mask
- \$20 Tuffy the Plush Puppy
- \$12 Cure SMA Infant Bib
- \$12 Cure SMA Luggage Tag

NEW APPAREL:

- \$18 Baseball Tee Onesie (6months-24months)
- \$18 We Can Cure SMA Onesie (6months-24months)
- \$49 Unisex Microfleece Full-Zip Jacket (XSmall-3XLarge)
- \$20 We Can Cure SMA Doggie Tank (XSmall-2XLarge)



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

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



Clinical Care Update

Why Clinical Care is Important

The SMA Community now has three drugs approved by the U.S. Food and Drug Administration (FDA) for the treatment of people with spinal muscular atrophy (SMA). This includes Spinraza (nusinersen), a treatment that works by modifying splicing of the SMN2 gene; Zolgensma (onasemnogene abaparvovec), a gene replacement therapy for SMN1; and Evrysdi (risdiplam), a small molecule that targets the SMN2 gene. All treatments result in increased survival motor neuron (SMN) protein, and none are a cure for SMA.

With these three FDA-approved treatments for SMA, continued and close follow up with your healthcare team is essential, no matter your SMA type, age, or abilities. Think of clinical care as how your health is managed every day to stay healthy. If you cut your finger, for instance, you will act quickly by washing the wound, applying pressure to stop the bleeding, and covering the wound with a bandage to help it heal. In the same way, if a child is not gaining weight as expected, has signs of reduced or poor leg movement, and/or has belly breathing, they should be evaluated by a neurologist for SMA. Similarly, if an older child or adult has difficulty climbing stairs or walking, they should be evaluated by a neurologist and SMA should be considered. After a confirmed diagnosis, treatment should be offered and initiated, per family choice.

Concurrent to receiving any SMA treatment, a person living with SMA should be evaluated by the entire neuromuscular team and interventions should be implemented. Do not wait to see possible improvement. Improvement can take weeks to months. Nutrition, breathing, motor function, and emotional health should all be evaluated by respective healthcare team members—nutritionist, pulmonologist, physical therapist and physiatrist, and social worker and/or psychologist. For example, if a person living with SMA is losing weight, they should see a nutritionist to evaluate diet intake and/or feeding concerns, followed by recommendations for dietary changes.

Similarly, a pulmonologist should evaluate a symptomatic child recently diagnosed with SMA and provide respiratory equipment for support and management, per the SMA standard of care recommendations.^{1,2} For those on treatment who experience a change in well-being (e.g., increased fatigue, more difficulty sleeping, difficulty maintaining weight), these concerns should be discussed with the neuromuscular team and evaluated. Keep in mind that SMA treatments may possibly contribute to new symptoms.

Health concerns must be addressed when they arise to maintain optimal health. Again, do not wait for a response to an SMA treatment, as it can take time and health will not wait. There are many symptoms and situations that may arise that healthcare providers will not have the answers for. The best way to get to an answer is to have conversations about concerns and work together with your healthcare team to address the medical needs.

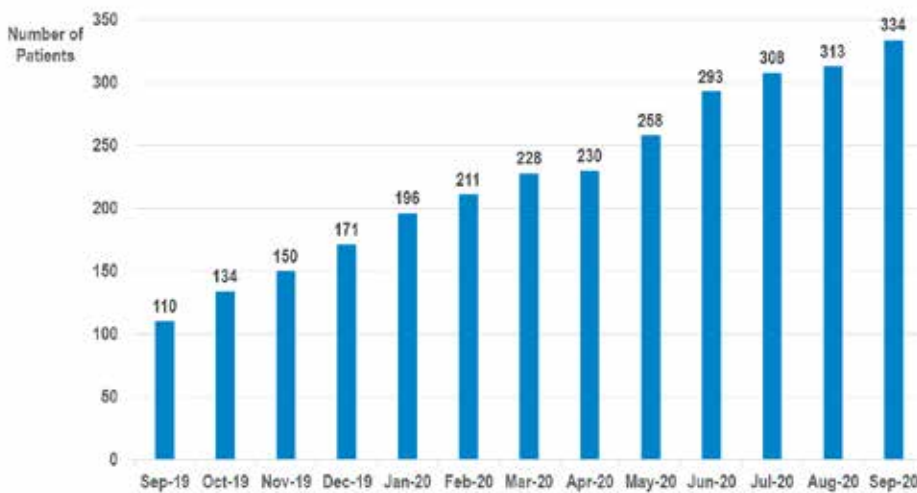
Regardless of whether you have been living with SMA for less than one year or more than 10 years, continued and regular follow up with your healthcare team is the best way to monitor progress and response to SMA treatment; to identify problems early and intervene in a timely way; and to stay informed about SMA treatment options and innovations. Partnership with your healthcare team is essential to staying healthy. Please consult with your healthcare professional regarding all treatment considerations. Cure SMA is also here to provide information and support your decisions.

1. Mercuri, E, Finkel, RS, Muntoni, F, et al. Diagnosis and management of spinal muscular atrophy: Part 1: Recommendations for diagnosis, rehabilitation, orthopedic and nutritional care. *Neuromuscul Disord.* 2018;28(2):103-115.
2. Finkel, RS, Mercuri, E, Meyer, OH, et al. Diagnosis and management of spinal muscular atrophy: Part 2: Pulmonary and acute care; medications, supplements, and immunizations; other organ systems; and ethics. *Neuromuscul Disord.* 2018;28(3):197-207.

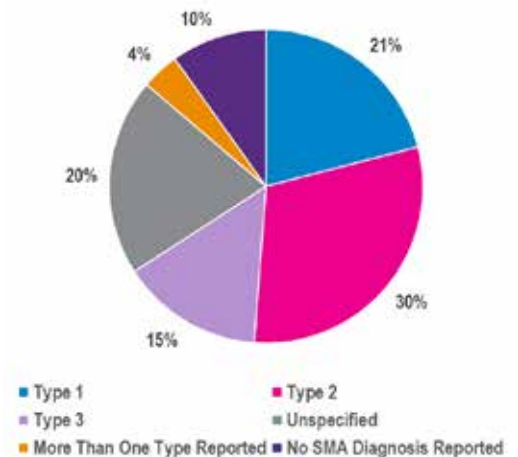
Update on the SMA Clinical Data Registry

The SMA Clinical Data Registry continues to grow as new patients are consented at Cure SMA Care Center Network sites across the country. As of September 9, 2020, we now have 334 patients in the Registry, representing a diverse cross-section of individuals with SMA in the U.S.

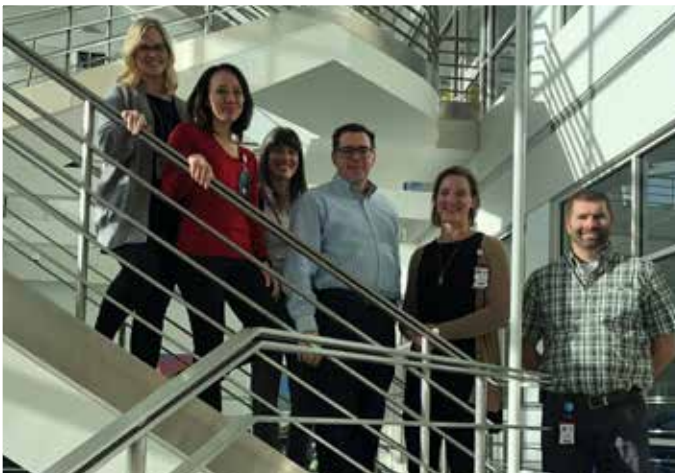
SMA Clinical Data Registry Patient Accrual (last 12 months)



SMA Clinical Data Registry Patients, By SMA Type



Cure SMA Care Center Network *Spotlight:*



University of Utah

The University of Utah in Salt Lake City, Utah, joined the Cure SMA Care Center Network in 2018 as one of the first sites. The center director, Dr. Russell Butterfield, serves on Cure SMA's Clinical Data Registry Committee. Furthermore, Utah was the first state in the U.S. to implement permanent newborn screening for SMA. The team at the University of Utah receives all referrals for newborn screening in Utah and advocates for the best care possible for their patients.

The University of Utah and Primary Children's Hospital SMA Care Center Network Team. From left: Meagan Bernatz, APRN, CPNP, Sarah Moldt, CRC, Melissa McIntyre, PT, DPT, Russell Butterfield, MD, Amelia Wilson, PT, DPT, and James Rogers, RN.



Advocacy Update

Above: Iam Zurawski, from Michigan, who has SMA Type 2, successfully got his requests approved by the Governor for an SMA Awareness Proclamation for August!

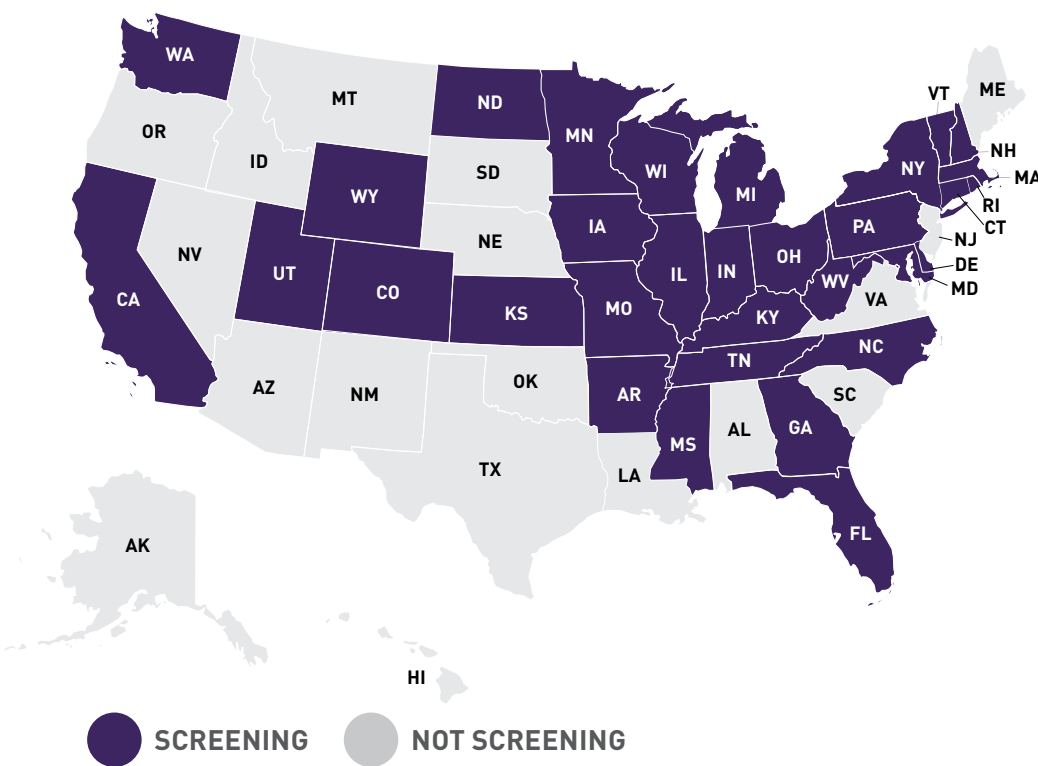
Advocates Bring Attention to SMA during SMA Awareness Month

Thank you to all the Cure SMA advocates who helped raise awareness about SMA during SMA Awareness Month in August. Cure SMA supporters from across the country were able to work with their state and local officials to declare August 2020 as SMA Awareness Month, enacted through proclamations. These proclamations can be an effective grassroots outreach tool for educating and connecting with key decision-makers and their staff. To assist in this annual effort, Cure SMA has developed a proclamation template that can be used in outreach with cities and states. For more information, please contact Cure SMA Advocacy at advocacy@curesma.org.

Cure SMA Works to Ensure Access to Treatments

With the recent U.S. Food and Drug Administration (FDA) approval of Evrysdi, Cure SMA contacted public and private insurers to continue educating them on the ongoing needs of the SMA community. This included highlighting the clinical evidence of treatments, and asking for full, unrestricted access to individuals with SMA over 2 months of age, according to the FDA label. In addition, Cure SMA has presented testimony and provided public comment in support of full and unrestricted access to treatments, therapies, equipment, and services.





STATE SCREENING FOR SMA

32 States Currently Screen for SMA
68% of Newborn Babies in the U.S. are Screened

RUSP 2 Years Later: 2 in 3 Babies in the U.S. Are Screened For SMA!

Approximately 2.6 million babies born in the United States are screened for SMA, according to a nationwide newborn screening status report released by Cure SMA earlier this summer.

In its newborn screening status report, Cure SMA reported that 32 states now screen newborns for SMA through permanent and pilot programs. Together, these SMA screening states represent more than two-thirds of all U.S. births. The percentage of newborns screened increased considerably with the 2020 implementation action by large states, such as California, Florida, Illinois, and Washington. Cure SMA expects the percentage of newborns screened through a state program will increase to 75 percent by the end of 2020.

“Our goal is to ensure newborns in every state have the best chance at a healthy and full life, and the best way to do that is to implement nationwide screening for SMA at birth. We’ve made tremendous progress, but we need the rest of the country to catch up to better prepare families for the decisions that come with an SMA diagnosis,” remarked Kenneth Hobby, President, Cure SMA.


Texas, which will host Cure SMA’s 2021 Annual SMA Conference, is the largest of the non-screening states, representing more than 10 percent of all U.S. births. Cure SMA is working with SMA families and partners in those states to help expedite implementation of newborn screening for SMA.

“Early diagnosis leading to early treatment offers a family facing SMA the best possible outcome and that was our goal when we set out to get SMA added to the federal Recommended Uniform Screening Panel,” said Jill Jarecki, Ph.D., Chief Scientific Officer, Cure SMA. “We now need states that have yet to implement newborn screening to act now so parents have the critical information they need to make the best decisions for their child and so that all families across the U.S. are treated equally.”

The SMA newborn screening progress report was shared with Governors and key stakeholders on the 2-year anniversary of SMA being added to the Federal Recommended Uniform Screening Panel (RUSP). Do you live in a non-screening state? Visit the Cure SMA Advocacy Action Center at <https://www.curesma.org/ActionCenter> for information on how you can help support newborn screening action in your state.

INTERESTED IN ADVOCACY? WANT TO SHARE YOUR SMA EXPERIENCE?

Cure SMA Advocacy relies on individuals with SMA and their families acting, participating in meetings, and sharing their SMA experiences. If you are interested in advocating in support of newborn screening, access to treatments and care, or other SMA-specific issues, contact the Cure SMA Advocacy Team at (202) 871-8004 or at advocacy@curesma.org.



Each year, we are honored by thousands of passionate donors who support our Year End Campaign. The critical funding that we receive during this charitable time helps add new support measures for all those affected by SMA. These gifts are essential to expanding access to treatments, improving care, and funding our next research breakthroughs.

~ Kenneth Hobby - President, Cure SMA

Fundraising Update

It's the Most Generous Time of the Year

Since 1984, Cure SMA has been a steward of the unparalleled passion and support of the SMA community. As the leader in SMA, we understand that there are many reasons our supporters choose to donate to our cause during the giving season, and that there is a special level of dedication behind each and every gift donated to Cure SMA.

In the last few months of any year, it is typical for people to reflect on the passing year, while also beginning to feel excitement about the new year to come. As we close out 2020, Cure SMA is reflecting on the groundbreaking advancements in SMA treatment, our continued response to the ongoing COVID-19 pandemic, innovative ways we have engaged and created interactions with the SMA community, and on the progress in implementing nationwide newborn screening for SMA. We are also focused on the future of our mission to fund research, care, and support services—all of which are invaluable to the community we serve.

Moving into 2021, we will continue to advocate for broad access to all approved SMA treatments for the entire SMA community. At the same time, we will turn our focus towards what's next on the research horizon. Our funding for basic research and partnership with the Pediatric Neuromuscular Clinical Research Network (PNCRN) will accelerate progress to produce new non-SMN and combination treatments for all ages and stages of SMA. We are also looking ahead to important milestones that we seek to achieve before our next Annual SMA Conference in Austin, TX.

By June 2021, 90 percent of all babies born in the U.S will be screened for SMA—an achievement that would not have been possible without the grassroots advocacy of our community. Finally, working with Cure SMA's Adults with SMA Advisory Council, we will continue to advocate for full access to care for people living with SMA, and will launch new services for adults and teens.

Since 2015, our Year End Campaign has raised nearly \$4 million in funding for the vital programs that people living with SMA depend on. The support that we receive this coming November and December is critical to fueling our ongoing mission and, without it, we would not be able to continue the unprecedented progress we are making with our research, advocacy, care, and support programs.

The end of the calendar year is a crucial time to connect with donors. According to Nonprofits Source, approximately 30 percent of all annual giving occurs in December, and 10 percent of all annual giving occurs in the last 3 days of the year, as people take the opportunity to make their last tax-deductible gifts. But the value of our Year End Campaign is measured by the individual impact we have in the lives of everyone living with SMA. This year is no different. We are asking everyone to participate in our Year End Campaign and help bring new people to our cause. By embracing the essence of the giving season and making a donation, you can help Cure SMA achieve our goals in 2021 and beyond.

ARE YOU INTERESTED IN GETTING INVOLVED IN OUR YEAR END CAMPAIGN?

Visit us at www.curesma.org/donate | Call us at 800.886.1762 | Send your gift to Cure SMA at 925 Busse Road, Elk Grove Village, IL 60007

You can also reach out to Brett Kinley, Director of Development, at brett.kinley@curesma.org or Anna Parker, Manager of Development, at anna.parker@curesma.org with any questions.

IMPACT REPORT



July 1, 2019 – June 30, 2020

- ✓ **194** NEWLY DIAGNOSED CARE PACKAGES SENT
- ✓ **374** TEEN AND ADULT CARE PACKAGES SENT
- ✓ **311** PIECES OF EQUIPMENT SENT FROM OUR EQUIPMENT POOL
- ✓ **7** GRANTS PROVIDED **\$1M** IN BASIC RESEARCH FUNDING
- ✓ **2,262** FAMILIES AND INDIVIDUALS RECEIVED SUPPORT FROM COVID-19 PROGRAMS
- ✓ **6,650+** ATTENDEES FROM ALL ANNUAL SMA CONFERENCE AND SMA RESEARCH & CLINICAL CARE MEETING ACTIVITIES
- ✓ **1,000+** PARTICIPANTS FROM 35 STATES REGISTERED FOR MAY 31ST VIRTUAL WALK-N-ROLL EVENT

Cure SMA wants to thank our donors, event organizers, and volunteers who support our mission every day. We continue to invest in comprehensive research to create new breakthroughs in treatment and care, while providing the local and national programs that the SMA community depends on today. In fact, 83 cents of every dollar raised is funding research, patient services, family support, and awareness programs.



Don Reynolds with his granddaughter, Ellie Stitzer. Ellie is 23 years old and lives with SMA Type 2.

Donor Spotlight Don Reynolds

Don Reynolds is a proud grandfather. His granddaughter, Ellie Stitzer, was first diagnosed as an infant and today lives with SMA Type 2. A summa cum laude graduate from the University of Missouri, Ellie is now 23 years old and in her second year of law school at Washington University in St. Louis, MO. When she graduates, she plans on working to advocate for people living with disabilities. Over the years, Don and his family have been heavily involved with Cure SMA, attending many local events and the Annual SMA Conferences. Today, Don's support continues as one of Cure SMA's most devoted donors. Each year, Don contributes to our annual year end campaign because he believes that his support will help lead to a cure for SMA. He recently shared, "I can assure you that donating to Cure SMA will make an incredible difference for those living with SMA because donations go directly to SMA research and family support programs." Don is one of many passionate donors who annually supports our mission. Without passionate people like Don, we would not be continuing to make unprecedented progress with our research, advocacy, support, and care initiatives. If you would like to join Don as an annual donor to Cure SMA, please visit: www.curesma.org/donate.

A woman with long brown hair, wearing a blue top, is speaking into a microphone. She is looking slightly to her left. In the background, other people are seated, some looking towards the speaker. A large purple circle is overlaid on the left side of the image, containing text.

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!

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 living with SMA and helping to build a hopeful future.

A woman with long dark hair, wearing a plaid shirt, is kneeling and smiling next to a young boy with glasses sitting in a motorized wheelchair. They are in a large indoor space with a "cure SMA" sign 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

Make a donation today! Visit us at 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



Research Update

Cure SMA Funding Network of Prominent SMA Clinical Research and Treatment Centers

Cure SMA announced this spring its new funding for the Pediatric Neuromuscular Clinical Research Network (PNCNRN). In 2020, Cure SMA will provide \$1.2 million to this Network of highly skilled clinical trial investigators, clinical evaluators, clinical coordinators, statisticians, and data management personnel.

“In these unprecedented times, we see the investment in the PNCNRN as critical in our mission to advance treatment and care,” says Kenneth Hobby, President of Cure SMA. “We are eager to build on the past accomplishments of the Network, most notably the SMA Natural History Study that was clearly defined by the Network and has been used as a key benchmark to assess safety and effectiveness of potential and approved therapies.”

Originally funded by the SMA Foundation in 2004, the PNCNRN established a team of SMA clinical trial experts that have integrated clinical research, education, and care to achieve the best possible clinical trial outcomes. Cure SMA has collaborated with the SMA Foundation as co-sponsor of the PNCNRN since 2018.

In recent years, the PNCNRN has been involved in the seminal clinical trials that have led to the U.S. Food and Drug Administration (FDA) approvals of breakthrough SMA treatment options that have forever changed the natural history of the disease.

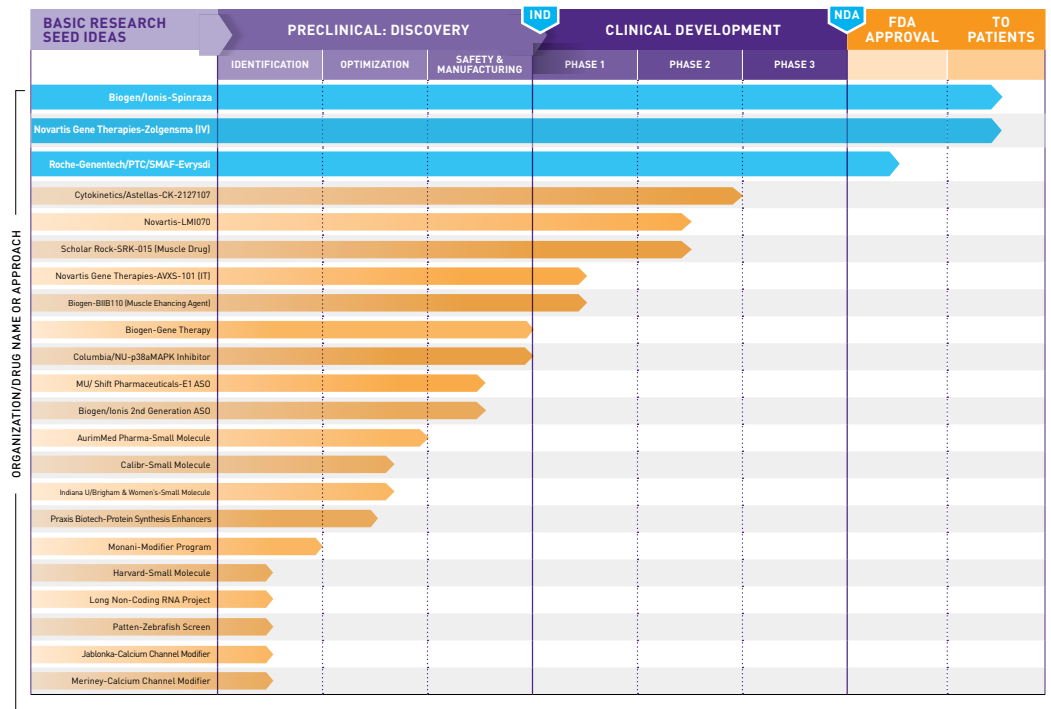
The Network continues to develop and refine more sensitive outcome measures, conduct ongoing clinical trials, accelerate newborn screening programs, and identify promising new treatment approaches. Cure SMA sees its support of the PNCNRN leading to more ground-breaking collaboration in SMA research and clinical care, especially as we expand the number of effective treatments for SMA and pursue the ultimate goal of a cure.

“Since the advent of effective SMA treatments, the recognition and description of new SMA phenotypes in treated patients and the durability of the approved treatments have emerged as critical issues, making the continuing work to understand the disease mechanisms and phenotype-genotype correlations that much more important,” says Darryl De Vivo, M.D., Director of the PNCNRN and Professor of Neurology and Pediatrics, Columbia University Irving Medical Center, New York City. “Cure SMA’s leadership in SMA research, and its new support of the PNCNRN, will allow us to sustain this ground-breaking collaboration in SMA research and clinical care, especially as we expand the number of effective treatments for SMA and pursue the ultimate vision of a cure.”

Six sites comprise the PNCNRN for SMA, including Boston Children’s Hospital, Boston, MA; Children’s Hospital of Philadelphia, Philadelphia, PA; Columbia University Irving Medical Center, New York, NY; Nemours Children’s Health System, Orlando FL; Stanford University, Palo Alto, CA; and the data coordinating center at the University of Rochester, Rochester, NY. These clinical research and treatment sites have also now been integrated into the established Cure SMA Care Center Network, which will lead to real-world evidence that increases access to approved treatments for SMA and improves care for individuals and families affected by SMA.

SMA DRUG PIPELINE

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



Cure SMA Met with U.S. FDA for Critical Path Innovation Meeting

In August, Cure SMA met with the U.S. Food and Drug Administration (FDA) in a Critical Path Innovation Meeting (CPIM), where key leaders at the FDA had the opportunity to communicate with clinical investigators, members of academia, industry, scientific groups, and patients about how to improve efficiency and success in the next stages of drug development for SMA. At the virtual CPIM, Cure SMA worked to enhance the understanding among these regulatory leaders of the most significant unmet medical needs of children and adults living with SMA.

The goals of this meeting added to those of the successfully held SMA Patient-Focused Drug Development Meeting (PFDD) with the FDA in 2017. At the PFDD meeting, more than 400 families and adults affected by SMA, and other SMA stakeholders, came together to share what mattered most to the SMA community and expectations for future treatment and care.

Cure SMA's most recent discussions with the FDA centered around the effects of treatment on the quality of life of individuals, parents, and caregivers living with SMA, as well as the tangible ways that having access to treatment has positively impacted the SMA trajectory for many of the 10,000+ Americans living with SMA. However, Cure SMA also discussed the continued impact of SMA in our community following the approval of treatments—three of which are now approved by the Agency. This includes the distinct trajectories of children and adults with SMA who are treated after significant symptom development, compared to those treated earlier in disease progression.

Opportunities to collaborate with the FDA to help address existing unmet medical needs were also discussed. As we continue to see advances in new lines of therapies for SMA, we must address new scientific and regulatory issues, including the following topics:

- The changing natural history of SMA and continued, unmet medical needs among older patients with chronically progressing disease (i.e., people with SMA Types 2 and 3).
- Development of novel outcomes in SMA, particularly for adults with SMA.
- Exploration of novel biomarkers—specifically, the already identified neurofilament (NF) as a marker of disease progression in clinical trial and real-world settings.
- Approaches to a combination of therapies as new therapeutic candidates in development, mainly those with complementary mechanisms of action to those already approved, will be tested in clinical trials, and used in an additive fashion in the real-world.

Cure SMA representatives, as well as key experts in SMA basic and clinical research, clinicians (doctors and physical therapists), and industry partners participated in this discussion. Two patient representatives, a mother to two boys with SMA Type 1 and an adult with SMA, also impactfully shared their journeys.

An action plan of agreed-upon priorities between Cure SMA and the FDA resulted from this meeting. This may affect the design of future clinical trials, expectations for future therapies, and, ultimately, help to better meet the changing needs of our community.

See the latest SMA Drug Pipeline on our website at <https://www.curesma.org/sma-drug-pipeline/>.



Community Support Update

Cure SMA Launches New Medical Alert Bracelet Support Program

Along with funding SMA research and care, Cure SMA provides thousands of individuals and families with vital support and resources that help people navigate life with SMA. In addition to our current support programs, Cure SMA is thrilled to launch a brand new program offered to all individuals affected by SMA to help identify their medical needs in the case of a medical emergency.

Through this program, a Responder PHR (Personal Health Record) package is provided so medical personnel will have immediate access to the patient's medical records stored online, whether the patient is able to communicate for themselves or not. This includes prior medical conditions, allergies, current medications, etc. Each Responder PHR comes with a waterproof, silicone Medical Alert adjustable bracelet and matching waterproof Medical ID keychain.

Also included is the option to customize two sets of engraved acrylic plates with the patient's most vital health information. This—and an engraved plate with instructions on how to retrieve personal medical information from the bracelet—is meant to be read at-a-glance by medical responders who need immediate access to the PHRs online. These can be easily accessed from any internet capable device, such as a smartphone, tablet, or any computer using a simple PHR-ACCESS login URL and secured with a unique Member ID number. The patient can also choose to give access to all records or select the health information they want to share stored within the online system.

The Responder PHR system also serves as an organizational tool and allows the user to input personal information and previous health records into categories, including:

- Medical conditions
- Medications
- Allergies
- Emergency and doctor contacts
- Insurance information
- Immunization records
- Medical procedures
- Advance medical directives
- Living will
- Durable power of attorney
- Organ donor status
- Other pertinent information



If you are an individual with SMA, or a legal guardian of a child with SMA, who is interested in receiving a Responder PHR with Medical Alert Bracelet and Keychain, you can request one at no charge by filling out the online form available at <http://events.curesma.org/responderPHR>. Applicants must reside within the United States.

This program is generously funded by a grant from Genentech, a member of the Roche Group. Cure SMA would like to extend a big thank you for their continued and ongoing contributions towards our support programs for individuals living with SMA.



Virtual Community Engagement Events

As we continue to adapt to COVID-19 and social distancing, “virtual” has become second nature to how we do our jobs, educate kids across the country, and even celebrate major personal milestones. The idea of isolation is not a new concept for the SMA community, though the unprecedented levels of isolation during the pandemic has led to a disconnection of the community. At the same time, maintaining that real-time connection with others has become all the more critical for our emotional health.

This all said, it is no surprise that at a time when in-person meetings and events are off the table, communities are looking for fresh ways to create engagement online. After a successful turnout for the virtual socials during the 2020 Virtual SMA Conference, Cure SMA has set out to introduce new virtual community engagement events for various groups within the SMA community. These live virtual get-togethers are meant to provide that much-needed social boost that we have been missing from one another.

To learn more about existing and new virtual social programs as they are made available, visit
www.curesma.org/virtual-community-engagement-events/.

Virtual Adults with SMA Socials

Join other adults with SMA each month for a 90-minute virtual social! Sponsored by Biogen, these video calls offer an opportunity for adults with SMA to gather online in a friendly setting. Through Zoom breakout rooms, attendees will be able to network, catch up with old friends, and meet new adults with SMA in the community! These socials are only for adults with SMA over the age of 18 years.



Virtual SMA Family Socials

These fun-filled Virtual SMA Family Socials, sponsored by Novartis Gene Therapies, are 1-hour events offering individuals and families the chance to connect, interact, and learn from one another on Zoom! SMA affected individuals and immediate family members—including parents, siblings, grandparents, spouses, or children—are all welcome to join.



A special thank you to the Devine Family, who recently donated Otteroo's for the newly diagnosed care packages in honor of their sweet baby, Rhys. We are grateful for these wonderful additions and that we can continue to enhance these packages sent to families across the U.S.



Virtual SMA Conference

As we entered 2020 and prepared to meet up in Orlando in June, no one could have imagined what the world was about to face and how our lives would be changed. And, when it was clear that it was safest for everyone to stay home, Cure SMA quickly pivoted its plans for the 2020 Annual SMA Conference and SMA Research & Clinical Care Meeting, moving everything online. Despite these unexpected changes, the SMA Community once again demonstrated its strength to overcome.

The 2020 Virtual SMA Conference took place June 8-12, 2020, and although we were unable to come together in-person, it was such an honor to have individuals and families affected by SMA, clinicians, and SMA researchers virtually attend the conference from 77 countries around the globe. It was an impactful week of online workshops, networking, and community-building, with more than 6,600 participants taking part in the 28 offered live events.

The Virtual SMA Conference kicked off on Monday, June 8, with the Opening Session, Clinical Care & Research Update Session, and a Newly Diagnosed Virtual Session. This set the stage for a week of education and engagement. That first evening, we also hosted a “virtual mingle” where SMA grandparents were invited to network and connect online.

Family workshops kicked off on Tuesday, June 9, and ran through Friday, June 12, covering topics such as Aquatic Therapy, Women’s Health in SMA, Education, Medical Management of Adults, Breathing Basics, and much more. We also hosted a few Adults with SMA Socials at Home and an Adults with SMA Lounge Night.

These evening events were an opportunity for adults with SMA to gather online in a friendly setting, and connect together via Zoom. These events were generously sponsored by Biogen.

The conference concluded with the special celebration, where all members of the SMA community were invited to share messages of unity on their social channels. In an enthusiastic display of our community’s strength, more than 5,000 people liked and shared Cure SMA’s content or posted their own messages to show how we were all “United, At Home.” Additionally, a special message from actors James Wolk and Elizabeth Lail—who are filming a new NBC pilot where they portray parents of a child with SMA—received nearly 25,000 views across our channels.

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

If you weren’t able to tune in for the live events, we’ve got you! Recordings of all online sessions from the Annual SMA Conference are still available to view on the Cure SMA YouTube channel.

We look forward to coming back together for the 2021 Annual SMA Conference in Austin, TX, June 10-13, 2021, at the JW Marriott Austin.

A Special Thanks to Our Sponsors of the 2021 Virtual Annual SMA Conference and Virtual SMA Research & Clinical Care Meeting

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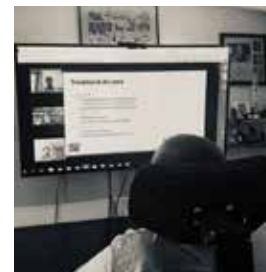
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Virtual SMA Research & Clinical Care Meeting

On Wednesday, June 10, Cure SMA hosted its Clinical Care CME Session, kicking off the 2020 Virtual SMA Research & Clinical Care Meeting. The Clinical Care CME Session provided a valuable opportunity to share knowledge and earn continuing education credits for 423 clinical attendees. The goals of the session were to share updates on innovations in clinical care and the impact of new therapies. This session also emphasizes the value and importance of multidisciplinary care teams in SMA care and creates meaningful dialogue between care providers across the globe.

The research portion of the meeting continued on Thursday, June 11, with a session on Basic and Clinical Research, and on Friday, June 12, with a session on Clinical Drug Development.

The goal was to create open communication of scientific data, accelerating the pace of research. The meeting also sought to build productive collaborations—including cross-disciplinary dialogue, partnerships, integration of new researchers and drug companies, and educational opportunities for junior researchers.

Several industry partners also released new data from their programs, covering research representing the entire SMA population—from infants to adults—and including different therapeutic approaches, including those that target the underlying genetic cause of SMA and those that work on the muscles affected by SMA.



Notes from the SMA Community

This is the first time for me attending this conference. Being in NYC, it's too far to make it to these conferences in-person. I'm hoping a majority of these sessions will be available for replay. I'm working full-time while trying to attend these, so was in and out for most of them. They're so insightful and inspiring. Thanks!

~ Wendy Lam

We are really enjoying the 2020 virtual conference. It's very informative and applicable to us.

Thanks for all you do for the SMA community!!

~ Dinie Goldberger

It's the first day of the virtual conference and I just wanted to extend a huge thank you for making this happen. Dan and his brother are watching the SMA My Way presentation and I've already watched the clinical trials update. It is striking me just how important this shot in the arm is for us. I didn't expect it to mean this much, frankly, as a virtual experience, yet the impact on our hearts and mindset on managing and thriving with SMA is still happening...even virtually. Thank you!

~ Mary, Dan, Joe, and Robby McHale

I just read the agenda for the virtual conference and it is fantastic. It feels so much less hectic and more relaxing than the in-person event. I know how much the families need to engage with each other in-person, but with the continuing stress from COVID, this is a really well put together program. It is thoughtfully spaced out so families have time to participate around their children's needs. It is impressive. I am so proud of you guys.

The clinical conference is amazing and it means several of my colleagues who would not have been able to attend are able to now and are excited about this. Take care, take time to breathe and take a moment to acknowledge you have done an amazing job.

~ Diane Murrell

Thank you for all your efforts in making a really tough situation a chance to learn, hear from others, and unify...despite being separate. I appreciate the time, creativity, learning curve, and collaboration it took to make this be as successful as it was.

~ Jennifer Martyn

I want to applaud you all for a great conference. The amount of work you have done to put it together is remarkable and it shows — BRAVO!

~ Gail Hartin

Congratulations on pulling off an essentially 'glitch-free' virtual Annual SMA Conference. While it was not quite the same regarding having the opportunity to interact with the other attendees, the content, as I've come to expect, was excellent. Thank you for all your efforts to make this happen!

~ Stacy Rudnicki

I hope you are doing well. I wanted to send you a note of congratulations on completing your first-ever virtual annual conference. The internal team and all patients and HCPs that we have spoken with have had very positive things to say and it was great to see so many people attending the events. Looking forward to tonight's Celebration of Unity...we are excited to be able to be with everyone next year in Austin!

~ Matt Winton



First-Ever Virtual Walk-n-Roll Was A Success!

In a typical year, the SMA community is hosting dozens of Walk-n-Roll events across the country. But we all know that this year has been anything but typical. All events since March have been cancelled and local individuals and families who lead these events have had to rethink how to engage their walk teams and communities. On May 30, 2020, Cure SMA held its first-ever national Virtual Walk-n-Roll to continue our support for teams and help keep community traditions alive.

Gathering on this Saturday morning, teams, participants, and sponsors representing 14 different Walk-n-Roll event locations from across the U.S. united for a successful online program celebrating the strength of the SMA community. Together, these walks raised \$265,820 thanks to the support of more than 1,700 generous donors.

In the 6 weeks leading up to the Walk-n-Roll, 133 teams made up of 1,000 participants from 35 states participated in challenges to fundraise for Cure SMA. People chalked sidewalks, decorated doors, created team logos, and more to help motivate their teams and raise SMA awareness.

All these activities culminated with a Virtual Walk-n-Roll day, when Cure SMA hosted the Celebration Ceremony. More than 1,000 viewers joined us on YouTube to revel in everything our community had achieved so far in 2020. Stories were shared by individuals and families living with SMA from across the country, each demonstrating in their own way how the SMA community is stronger together!

After the Celebration Ceremony ended, participants walked and rolled on their own to celebrate Cure SMA's mission. More than 125 community members submitted photos and videos of their families walking and rolling in cities from coast-to-coast.

**Thank you to everyone who participated in
our Virtual Walk-n-Roll this spring!**

A second Virtual Walk-n-Roll was just held a few weeks ago, on Saturday, September 12, 2020, and the final Virtual Walk-n-Roll of the year will be held on Saturday, November 14, 2020.

Check out how you can get involved by reaching out to fundraising@curesma.org.

Thank you to all the teams, participants, and volunteers that participated in the first-ever Virtual Walk-n-Roll in May 2020. We would especially like to thank the volunteer committees from the following events for their support!

- Arkansas Walk-n-Roll
- Cincinnati Walk-n-Roll
- Cleveland Walk-n-Roll
- Connecticut Walk-n-Roll
- Dallas Walk-n-Roll
- Houston Walk-n-Roll
- Los Angeles Walk-n-Roll
- Michigan Walk-n-Roll
- New England Walk-n-Roll
- North Jersey Walk-n-Roll
- Pennsylvania Walk-n-Roll
- St. Louis Walk-n-Roll
- Walk-n-Roll in Honor of Graham
- West Virginia Walk-n-Roll

A special shout out to our top teams and top participants from the Virtual Walk-n-Roll on May 30, 2020.

Top Team Fundraisers

Team Murphy	\$ 40,480.00
Pete's Philadelphia Eagles	\$ 12,930.00
Caitie's Heroes	\$ 12,285.00
Team RogoCup	\$ 9,685.00
Emma and Nick's Team	\$ 9,295.00

Top Individual Fundraisers

Allyson Henkel	\$ 7,545.00
Silvia Murphy	\$ 6,690.00
Joseph Scully	\$ 6,200.00
Beth Lockwood	\$ 4,305.00
DeAnna Dillon	\$ 4,000.00

Largest Teams

Emma and Nick's Team	32
Team Mila Rae	29
Pete's Philadelphia Eagles	27
Winnie's Warriors	27
Our Lovely Angels	23

Top First-Time Fundraising Teams

Quaranteam	\$ 6,460.00
Team Erin	\$ 2,710.00
Cure for Chloe	\$ 2,375.00
Love for Locklynd	\$ 1,290.00
Jake's Village	\$ 1,215.00

Virtual Walk-n-Roll

CELEBRATION CEREMONY

**SATURDAY,
NOVEMBER 14, 2020**

**11 a.m. ET/10 a.m. CT/
9 a.m. MT/8 a.m PT**



2020 VIRTUAL
WALK - N - ROLL

Learn more about Cure SMA's next Virtual Walk-n-Roll on November 14th
by visiting Events.curesma.org/NovemberWalk.

TEAM CURE SMA

VIRTUAL PROGRAM

Virtual Team Cure SMA is a new endurance program that offers runners and cyclists—both currently supporting Team Cure SMA or just thinking about it—a way to participate in races virtually while sharing their results and accomplishments with the SMA community.

Each month Team Cure SMA offers a new and exciting virtual endurance challenge. There is no fundraising requirement to participate, but athletes can earn awesome Team Cure SMA endurance gear if they choose to fundraise and reach designated milestones. We know this is a group dedicated to taking on fun fitness challenges already, and we are here for you every step of the way, even virtually!



Check Out Upcoming Virtual Endurance Events

October Challenge:

Complete 20 miles, 200 air squats
200 pushups in 7 days

November Challenge:

Complete 30 burpees a day for 14 days

December Challenge:

Run or bike 30 miles in 30 days



Regina Philipps (right) at the Vermont Marathon

TEAM CURE SMA

Runner Spotlight

Regina Philipps

I run for Team Cure SMA in honor of our 2-year-old son, Shane, who has SMA Type 2. I want to teach him that you can do anything you put your mind to if you work hard and give it your best effort—although, we have realized that maybe he is really the one teaching us! I run for Team Cure SMA to raise awareness and because I know all of our fundraising is put to the best use possible. Cure SMA has done so much for Shane—from funding the initial research for treatments that have literally given him life to providing him with his lightweight Panthera Micro wheelchair free of charge from the equipment pool, which helps him navigate the world. We are #stronglikeshane thanks to Cure SMA!

If you have questions about how to fundraise and train for an endurance event contact Alyssa Zavislak at alyssa.zavislak@curesma.org.

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



twitter.com/cureSMA



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youtube.com/user/FamiliesofSMA1



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



VISION AND MISSION STATEMENT

Cure SMA leads the way to a world without spinal muscular atrophy, 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 spinal muscular atrophy. 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 affected by SMA and communicate openly and honestly, giving them clear and accurate information.

Determination

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

On the cover:

Neeley Hahn, Virtual Walk-N-Roll Event, Huntington Beach, CA
Dan Phillips and Hanna Eide, Virtual Annual SMA Conference, San Francisco, CA
TJ's SMA Superheroes, Virtual Walk-n-Roll Event, Oakville, CT
Joseph Geueke, COVID Support Package Recipient, Bradenton, FL
Jadie Lynn Andersen, SMA Awareness Month, Davenport, IA
Bethannie Bernand, COVID Support Package Recipient, Burlington, IA
Kate and Amelia Brown, Endurance Event Falmouth Race, Falmouth, MA
Cure SMA Care Center Network Site, Duke University Children's Hospital, Durham, NC
Brooks and Allie Wolfe, Brooks Wolfe Pack at Virtual Walk-n-Roll Event, Troy, NY
Farrell Family, SMA Awareness Month, Cincinnati, OH
Nicole Stickane, SMA Newborn Screening Advocate, Dallas, TX
Ochoa Family, Virtual Walk-n-Roll Event, Wimberly, TX
University of Utah, Utah Program for Inherited Neuromuscular Disorders, Salt Lake City, UT



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Submissions

To submit articles or make other contributions to our newsletter, please contact us at: newsletter@curesma.org.

Digital images are encouraged! Send your digital pictures to: newsletter@curesma.org.

Change of address

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

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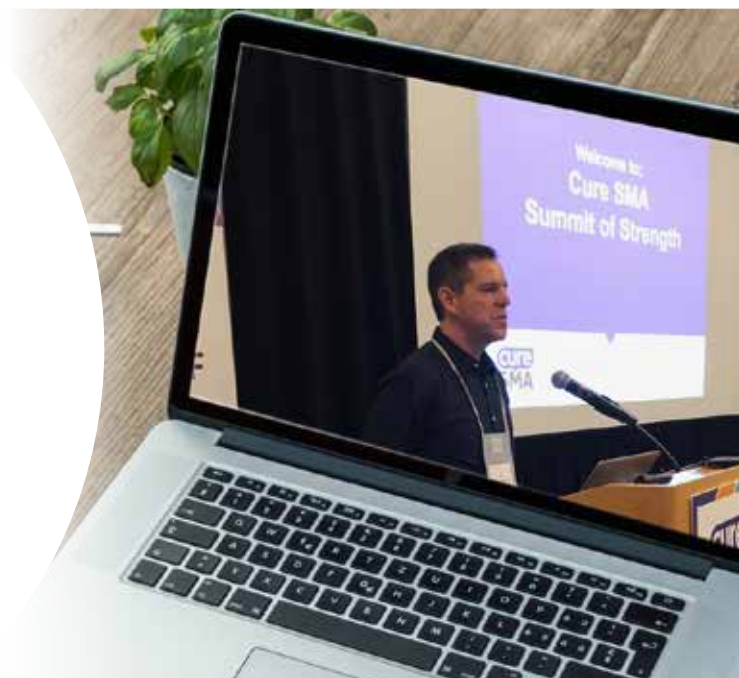
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2020 SUMMIT OF STRENGTH WEBINAR SERIES

Cure SMA has launched the Summit of Strength Webinar Series—focused on topics tailored to help individuals and families living with SMA during this life in quarantine.

Webinars are held most Wednesdays at 12 noon CT.

These webinars cover topics such as emotional health, emergency preparedness, mobility, and pain management, among others.



Visit www.curesma.org/summit-of-strength/ to learn more about the Cure SMA Summit of Strength Webinar Series and register for an upcoming virtual seminar.

Thank you to the National Presenting Sponsors for the 2020 Summit of Strength Programs: Biogen, Genentech, and Novartis Gene Therapies. We would also like to recognize Platinum Sponsor, Scholar Rock, for their support.

These webinars are intended for U.S. audiences only.