

DIRECTIONS

COMMUNITY NEWSLETTER | SPRING 2021



«IAM»
Mcintosh Family
REDEFINING RARE



«IAM»
Brady
REDEFINING RARE



«IAM»
Emeily
REDEFINING RARE



«IAM»
Ben
REDEFINING RARE

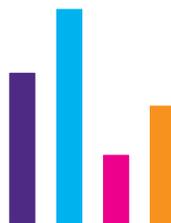


«IAM»
Ayça
REDEFINING RARE



«IAM»
Zoey
REDEFINING RARE

Thank You



RICHARD RUBENSTEIN

We would like to extend our gratitude and congratulations to Richard Rubenstein for his time and efforts dedicated to Cure SMA since 2009. He became Chairman of the Board in 2013 and has now completed his term in this role.

During that time, he advanced the organization's key strategic initiatives—pushing forward with new support programs for the adult community and local patient care for all. His dedication has helped bring SMA to where it is today, with widespread newborn screening and three FDA-approved treatments that have forever changed the diagnosis, treatment, and care for SMA.

We are pleased that he will be staying with our Board as a member emeritus in the years ahead.

Thank you, Richard, for your years of dedication, support, and leadership to the mission of Cure SMA!

Kenneth Hobby
President, Cure SMA

Nick Farrell
Chairman of the Board



Richard M. Rubenstein, Esq., speaking at the Annual SMA Conference.



Richard's son Max, who had SMA, passed away in 2009.



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While 2020 turned out to be a most unusual year, Cure SMA has remained committed to our mission and is extremely optimistic about the future for our community in 2021 and beyond. This year will likely still look different than years past as we continue to engage and educate through virtual programming. This includes taking the Annual SMA Conference and SMA Research & Clinical Care Meeting online once again.

That said, we hope to see positive changes as we open activities back up to be in-person in the coming months. We expect to host a series of in-person Summit of Strength events across the country starting in late spring and are looking at new ways to get our Walk-n-Roll teams back on the streets. These are vital programs for our community, and we know that these moments of engagement have been missed this past year.

As we do every year, Cure SMA is setting ourselves up to reach big goals—and 2021 is no different. We are advancing new research that focuses on non-SMN targeted therapies, symptom and muscle-targeting treatments, and the development of combination therapies that increase strength and enable independence for all people with SMA. In 2021, Cure SMA will continue working to implement newborn screening for SMA to ensure babies in every state have the best chance at a healthy, full life.

Finally, an important goal in 2021 will be the continued growth and integration of the Cure SMA Care Center Network to build our comprehensive SMA Clinical Data Registry and improve local patient care. We will also look to accelerate our work on policy issues most important to adults and young adults with SMA.

We appreciate all your efforts and ongoing support, knowing we could not have made the progress we have without you.



Kenneth Hobby
President, Cure SMA

ADVERTISEMENT



GET TO KNOW EVRYSDI

An SMA medication for infants, children, and adults

In people 2 months and older

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

What is Evrysdi?

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

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

Important Safety Information

- Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:
 - have liver problems
 - are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. Evrysdi may harm your unborn baby.
 - are a woman who can become pregnant:
 - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy. Because Evrysdi may harm your unborn baby, your healthcare provider will decide if taking Evrysdi is right for you during this time
 - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
 - are an adult male planning to have children: Evrysdi may affect a man's ability to have children (fertility). If this is of concern to you, make sure to ask a healthcare provider for advice
 - are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby. If you plan to breastfeed, discuss with your healthcare provider about the best way to feed your baby while on treatment with Evrysdi
 - **Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine
 - You should receive Evrysdi from the pharmacy as a liquid that can be given by mouth or through a feeding tube. The liquid solution is prepared by your pharmacist. If the medicine in the bottle is a powder, **do not use it**. Contact your pharmacist for a replacement
 - Avoid getting Evrysdi on your skin or in your eyes. If Evrysdi gets on your skin, wash the area with soap and water. If Evrysdi gets in your eyes, rinse your eyes with water
 - The most common side effects of Evrysdi include:
 - For later-onset SMA: fever, diarrhea, rash
 - For infantile-onset SMA: fever, diarrhea, rash, runny nose, sneezing, sore throat, and cough (upper respiratory infection), lung infection, constipation, vomiting
- These are not all of the possible side effects of Evrysdi. For more information on the risk and benefits profile of Evrysdi, ask your healthcare provider or pharmacist.
- You may report side effects to the FDA at **1-800-FDA-1088** or www.fda.gov/medwatch. You may also report side effects to Genentech at **1-888-835-2555**.

Please see accompanying brief summary for additional Important Safety Information.

Genentech
A Member of the Roche Group

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

What is EVRYSDI?

- EVRYSDI is a prescription medicine used to treat spinal muscular atrophy (SMA) in adults and children 2 months of age and older.
- It is not known if EVRYSDI is safe and effective in children under 2 months of age.

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

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

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

How should I take EVRYSDI?

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

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

Taking EVRYSDI

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

Reusable Oral Syringes

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

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

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

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

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

How should I store EVRYSDI?

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

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

General information about the safe and effective use of EVRYSDI.

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

What are the ingredients in EVRYSDI?

Active ingredient: risdiplam

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

Genentech

A Member of the Roche Group

EVRYSDI™ (risdiplam)

Distributed by:

Genentech, Inc.

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

EVRYSDI is a trademark of
Genentech, Inc.
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©2020 Genentech, Inc.

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

Letters from the Community



We received the package and my son, Joshua, was thrilled. He turned 13 years old this year and he was excited. When he received the package, it was like birthday gifts that he wanted. He loved the Alexa, back scratcher, wool blanket, mold pillow, and everything.

~ Josh's Mom

Thank you so much for our care package. Christine loved it!

~ Rachel Upshur



We wanted to send an email thanking you and Cure SMA for the information and care packages. Our son absolutely loves it! Thank you for everything you're doing. Amid this difficulty, you guys give us hope, encouragement, and happiness.

~ Elizondo family

We are so appreciative of the care package you sent to Isabelle! She has loved everything in it! She brought a couple of her things with her when she was at the doctor. Thank you so much!

~ Emily Furnival



My family and I thank you from the bottom of our hearts! Damian was so excited to see all the toys, and his Nana and I could not keep from tearing up! I had no idea this is what he would be receiving when we requested a care package!

The support we have received from Cure SMA has been amazing!

~ Stephanie Braxton



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. Also, since receiving the package, we also got a wagon from you and he loves it! I'm glad we have you as a resource as we go through this journey and look forward to meeting in person in the future.

~ Elizabeth Parker



We are so thankful for this organization and all it's done for us, all the people it's connected us with, and the generosity to further research. Our kids wouldn't be where they are without you!

~ Victoria Colone



Isaiah wanted to say a giant thank you for the awesome COVID care package we just received! It is a huge blessing to our family. You are amazing, as always. And he was thrilled to put his Cure SMA ornaments on the tree last night. It's always his favorite.

Thank you always!

~ The Kobernik Family

Yesterday, I received the Cure SMA COVID-19 PPE Package. Thank you so very much! My family has not left our home except for medical appointments, and many of the online retailers will not list PPE supplies for online orders, making it extremely difficult to obtain necessary supplies. These supplies will help us more than you can imagine! Please thank all who are responsible for providing, arranging for, and distributing these precious supplies!

~ Donna Joerger

GRATITUDE FOR TEEN & ADULT CARE PACKAGE

I just received the care package and wanted to thank you for this gift. It has so many essential items that people cannot afford or possibly did not know about, and this will make a huge difference in my day-to-day.

~ Lacey Tompkins

Thank you so much for the support package. I was surprised by how many truly useful things were included. I feel seen and understood, which is often a rare combination. The contents from the remote outlets to the Amazon Echo and shoulder wrap will all help me greatly.

~ LaToya Moseley

Thank you for the wonderful gifts. They are all thoughtful, creative, and very useful. I especially love the Echo Plus, sheepskin blanket, and neck cushion. While research is important, helping us live comfortably, safely, and independently today is also so very important.

~ Sue Hannibal

Thank you so much for the support package. I LOVED IT and everything you guys sent. I am very grateful for all of it and for all who are a part of Cure SMA. I think what you guys do for all of us with SMA is extraordinary.

~ Victor Guerra

I received the Cure SMA care package a few weeks ago, and just wanted to send a huge THANK YOU to everyone there that made it possible! Every item in the box was so thoughtful and useful. It's clear your team put a lot of thought and care into each one. I've incorporated almost every part of it into my daily life... Thank you so much for your kindness and giving spirit. Very grateful for all that you guys and gals do. Have a great week!

~ Aaron Solomon

Donations

I received the support package this week and wanted to thank you so much. Several of the items, including the sheepskin, neck heating pad, and pillow, are very helpful to me. Thank you for improving my daily quality of life, and all you do for those with SMA.

~ Danny Paul



Special thank you to Jaysa Armstrong for collecting these wonderful toys to donate for our Newly Diagnosed Care Packages in honor of Knox and Fynlee's 4th birthday!



Thank you to the Andrew Family for collecting an assortment of fantastic toys to donate to our Newly Diagnosed Care Packages in honor of their son, Tristan.

Thank you so much for the considerate care package you sent us! We wanted to take the time to mention how helpful the items included were, since they were clearly carefully chosen. In particular: The Amazon Echo is an incredibly helpful item for someone like my husband who can occasionally have difficulty reaching his phone, and the voice activation is a huge help for safety purposes. If he were to fall from his chair while I was out, or were unable to move, being able to make a hands-free phone call would be a lifesaver... We look forward to trying out the other kit contents as well, such as the extendable grabber and neck wrap, the next time we need them. Thank you again for this wonderful kit, and for keeping us in your thoughts.

~ Rob and Jo Rusch



Aina Farhanah



Madison Wolff



Anna Miemois Stromblad



Kelsey Roberts



Payton Lavoie



Maxwell Peppers



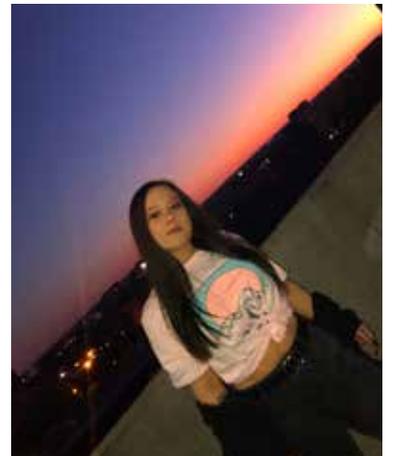
Toth Timea



The Carpenter Family



Max Lasko



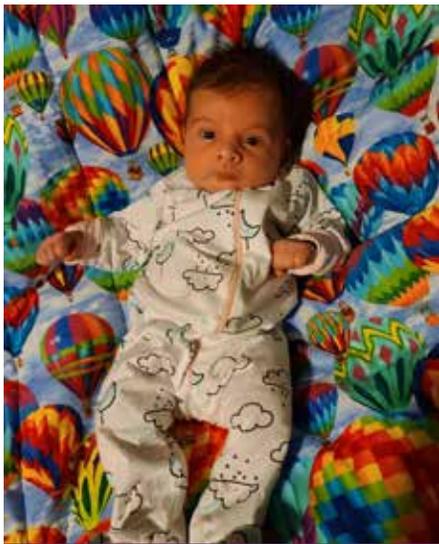
Madelyn Moss



Grayson Hoskins



Olivia Garcia



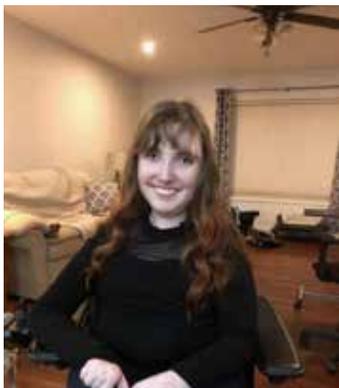
Aria Lopez



By Addison & Kelly Ferguson



Casey O'Neill



Lindsay Russell



Enguun Munkhbold



LyRick Cannady



Everly Tuck



Brian Chiorello



Christy Coenen

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



Paying it Forward to the Rare Disease Community

By Nikki McIntosh

When my son Miles was first diagnosed with SMA at 18 months old, I was fortunate enough to connect with other SMA moms within the first few months after the diagnosis. Those connections were a lifeline to me at one of the most challenging times—when it was all new and I did not know where our journey would take us.

My fellow SMA moms gave me practical advice and tips that only someone with a child facing a rare disease would know. Not only did it help me learn more about my son's SMA, but it helped me to see examples of other parents and families who were walking this road. They gave me a sense of community, and they helped me feel less alone. All of this was crucial in setting my family off on the right trajectory for approaching life with SMA.

After being in the SMA and rare disease communities for some years now, I've learned that the most common feelings parents face when receiving a rare disease diagnosis for their child are feeling afraid, alone, and powerless. One way to battle these feelings is to plug into a community of like-minded people, all championing the same cause. Doing something proactive can not only help parents contribute to positive change, but it can help us feel less powerless over our child's condition. Whether we are making contributions in advocacy, awareness building, fundraising, or sharing our stories, we can find purpose while strengthening our community.

There is a quote I love by George Eliot that says, "What do we live for if it is not to make life less difficult for each other." I love the idea of passing on learnings to make things less difficult for the next person or to better prepare them for what's ahead. Because I felt gratitude for the insight my fellow SMA mothers provided to me, I knew that one day I wanted to pay it forward and do the same. With that in mind, in September 2020, I launched RareMamas.com, an online resource for mothers whose children are diagnosed with a rare disease.

Rare Mamas offers practical tips, tactical tools, honest stories, and all the things parents want, and need, to know when facing a rare disease. Sharing my experience is my attempt to make things less difficult for other mothers whose children have been diagnosed with SMA or other rare diseases. Helping other mothers has, in a sense, become my purpose from the pain I experienced. I feel passionate about using my time and experiences this way, and I feel honored to help other mothers and families.



Nikki McIntosh of [RareMamas.com](https://www.raremamas.com)

As my son grows and we journey through new stages and phases, I continue to tap into my fellow SMA parents for guidance. As well, I've met many parents in the rare disease community who've been eager to help. I feel grateful to have others who are further in their journey to learn from and seek advice.

What I've found is that the rare disease community is made up of some of the most courageous, resilient, and resourceful individuals who are blazing trails and making changes for rare diseases. I see these brave patients and parents using their various talents and skills not only for their personal rare disease quest, but also to impact the full community. To me, this is what it's all about—helping the next child, the next parent, the next family along. In this way, we can all play a part in moving the needle in our collective groups, and we can impact future generations to come.

Think about how you can pay it forward in the SMA community and share with Cure SMA on Instagram, tagging @curesmaorg.



JOIN



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

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

BENEFITS INCLUDE:

\$10,000+

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

\$1,000 - \$9,999

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

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



Make a donation today at: www.curesma.org/donate.

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



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

Check out the updated Cure SMA Care Series Booklets.



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

**Some books are available in other languages*



Financial Planning with a Disability

By Mary Anne Ehlert

When you first learned that you or your child had SMA, you probably began analyzing your home life, your or your child's education, and other immediate concerns. In time, you realized that the needs would continue to evolve. How would you pay for medical expenses that insurance did not cover? What types of programs would you or your child qualify for as an adult? Where would you or your child live if something happened to the primary caretaker of the person with SMA?

There are so many things to take into consideration that you may feel overwhelmed. You need a solid plan to help you make sense of a complex situation. Here are some reasons why having a plan is not only a good idea but is also necessary.

Brings objectivity to a very emotional topic: A plan helps you look at a situation from a "big picture" standpoint. A traditional plan will look at the hard data, but planning with a disability will, more importantly, reflect the story beyond the numbers and how all those pieces fit together to make a whole. The first step of every plan is to create a vision of what you want for you or your child and write down what keeps you awake at night. Knowing those two things can make the difference between a plan consisting of only numbers and graphs and a plan that creates a map to a successful future.

Identifies options with one step at a time: A great plan will include more than one option and provide sound direction. It should be broken down into doable steps. We know that life can change in a moment and we should consider all the options to be considered. As you learn more, adjust the plan.

Focuses on legal options: It is essential to have the appropriate legal documents written by a legal source. You may need a special needs trust or even a payback trust. For parents of a child with a disability, you also need to make sure your estate plan is solid. If you are not prepared, then your child's care may suffer as a consequence. Special needs attorneys not only give legal advice, but they excel at providing a practical view of how an appropriately written trust will factor into your plan. A poorly written trust can jeopardize the individual with SMA's eligibility for benefits. First step is to find the right attorney.

Maximizes benefits and programs: A special needs plan has a benefits section that focuses on the myriad government and local benefits programs you or your child may be qualified to receive. This specialized area plays an essential role in your plan. Understand the limits on your or your child's assets and income when the person with SMA turns 18 to obtain SSI and Medicaid. For parents, understand how your own social security may impact your child's benefits into the future and possibly for their lifetime. There is a component of your benefits that may allow your child to obtain Disabled Adult Child (DAC) benefits that would also provide Medicare in their future. Obtain your own social security record to assist you in this portion of the plan.

Understands that transition is the key: Once a person with SMA reaches adulthood, benefits can, and will, change. A plan will help guide you through this process utilizing not only financial options, but program options to make the transition as easy as possible for all parties involved. Participate in groups that will help you find the future educational and residential options available to the individual with SMA.

Remains fluid and flexible with your own financial plan: Unlike a traditional financial plan, where graphs and numbers are followed, your own financial plan needs to address the many changes you or your child will go through, as well as your own planning needs. When you retire, your own plan needs to include the future costs of your child. Work with a financial professional who understands these concepts. Your investment solutions and insurance solutions need a long-term horizon. You cannot take chances that nothing will be left to fund the special needs trust.

Utilizes the ABLE Account as a savings plan: The newest planning tool in our world of disability planning is the ABLE account. This expands the ability to save for your child tax free, and the funds will not count against your child for future benefits.

Gets involved with the community: Become an advocate for yourself or your child. A benefit of being an advocate or activist in the SMA community is your newly gained knowledge of non-financial programs, support groups, recreational opportunities, and many other resources your family might be interested in.

A financial plan for a person with SMA is an essential part of providing the future care for this individual. Every parent wants to know that their children will be safe and happy when they are not able to be there, leading a life as independently as they are able to, and having lives of their own. These plans that focus on special needs can provide objectivity, guidance, knowledge, and, most importantly, empathy to your family. The plan will help make your dreams, or your dreams for your child, a reality.

Mary Anne Ehlert is the founder and president of Protected Tomorrows, Inc., the leader in enhancing the lives of families with members who have special needs. By guiding families through its comprehensive, proprietary planning process, Protected Tomorrows helps ensure the well-being of a loved one by creating a Future Care Plan™. Through their work with clients and the family's advisors, and alongside of other advocates and legislators, Protected Tomorrows addresses many concerns of families with special needs such as: future care funding, government benefits, legal considerations, residential options, employment opportunities, recreational choices, education options and family communication.

ADVERTISEMENT

THIS MOMENT

COMES
FROM WITHIN

CAMDEN // AGE 4

PRESYMPTOMATIC SMA, TREATED WITH SPINRAZA



NICOLE // AGE 45

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.*

3700+
ADULTS

Thousands of adults have been
treated with SPINRAZA worldwide*

3-80
DAYS YEARS

There's someone from almost
every age group who has
taken SPINRAZA^{†‡§}

7+
YEARS

Safety and efficacy evaluated
in the longest clinical trial in
SMA to date[§]

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


SPINRAZA[®]
 (nusinersen) injection
 12 mg/5 mL
BYRON // AGE 15

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.

Learn more at [SPINRAZA.com](https://www.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





SPOTLIGHT *on* Brady and Mimi Chan

Ten-year-old Brady Chan from North Carolina has big goals that stem from his passion for video games. He is part of an e-gaming team, where his favorite games include “Brawl Stars” and “Overwatch.” In the future, Brady hopes to become an engineer and create his own video games. He is already learning to code! Brady and his younger brother, Lucas, also love to travel, and Brady hopes to visit France and Japan. “We are always planning adventures and experiences for our kids,” shares Mimi, Brady’s mom.

Brady was diagnosed with SMA at 18 months old while in Beijing, China. “We actually spent the 8 months prior going through test after test with a local pediatric neurology office. The results came back normal, but he had completely stopped meeting milestones and started losing ability,” recalled Mimi. “My mom had connections at the Beijing Children’s Hospital, and getting tests and appointments seemed much quicker and easier, so she and I took Brady to Beijing.”

As Mimi reflects on their early years, she notes that those were the hardest moments. “We were coming to terms with SMA while at the same time learning about it and how to care for Brady. It was overwhelming.” Despite this, Mimi’s advice to other parents is to be your child’s biggest advocate, and to not be afraid to question doctors, ask for a second opinion, and make requests for the best interest of your child.

The good news is that life shifted for the Chan family once Brady began school and started treatment for his SMA. “He became a little stronger, got sick less, and became a little more independent,” says Mimi. She also notes that the school provided an aide, which was a huge help and gave the family a little bit of normalcy.

Throughout the COVID-19 pandemic and times of quarantine, Brady has attended school remotely. “Doing school online is much harder and stressful, but my favorite subjects are probably math and science,” shares Brady. He also enjoys reading books and watching YouTube, so much so that his family started their own YouTube channel to document their journey and family memories.

In 2015, Mimi left her decade-long marketing career to start her own online shop, Style Suite by Mimi Chan. Brady started slowly helping in 2019 and became more involved once schools shut down, helping to make custom bracelets. “We were able to spend a lot of time together, and also raise money for causes that we care about,” Brady notes. Not only was Brady working on a fun project, but this was also an opportunity to learn business skills and the importance of philanthropy, which was important to Mimi. “He’s learned about profit margins, the difference between gross and net profit, created an elevator pitch for events, and so much more.”

Recognize the symptoms and save a life!



EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

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

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



SMARtMoves.CureSMA.org



STILL AVAILABLE

CURE SMA TEEN AND ADULT SUPPORT PACKAGE

This package is filled with helpful items that have been recommended by other teens and adults with SMA. Many of the items included in the package have been found to make activities of daily living easier.

Items include:

- Medical Fleece Ease Sheepskin
- Amazon Echo Plus
- Telescopic Back Scratcher
- Long Grabber Reacher Tool
- Gooseneck Phone or Tablet Mount
- Etekcity Remote Control Outlet Switch Kit
- EZ-Shampoo Hair Washing Basin
- Neck and Shoulder Heating Pad
- Sisu Positioner Pillow

If you have not already received this package please visit <http://events.curesma.org/supportpackage> to request one at no charge.

This program is generously funded by a grant from Biogen.



SPOTLIGHT *on* Doug McCullough

Doug McCullough was raised on a dairy farm in Pennsylvania and now resides in Central New Jersey. He is a self-described “fun-loving guy.”

“I take my work seriously but don’t take myself too seriously,” shared Doug. Doug has been working at Johnson & Johnson for 20 years. He also has SMA Type 3 and is a member of the Cure SMA Adults with SMA Advisory Council.

Doug is an avid sports fan and participates in adaptive sports, such as cycling, kayaking, snow and water skiing, surfing, canoeing, and camping. “Sports are a great opportunity to meet and learn from others with disabilities, and the camaraderie of doing activities with both the other athletes and the able-bodied volunteers,” he shared.

Doug spends a lot of his free time reading, listening to music, and traveling. He has visited 45 U.S. states and 25 countries! “I played tuba growing up and in high school was able to travel through Europe one summer with a band,” Doug explained.

Before connecting with Cure SMA many years ago, Doug had never met anyone else who had SMA. “I have really enjoyed becoming part of the community, both from a social perspective in meeting people like me and from an educational perspective in learning how others manage challenges in their care,” Doug continued.

Doug became an “accidental activist” after he learned to accept his disability. “I’m somewhat unique in that most of the able-bodied world sees me as disabled and most of the disabled community sees me as pretty able bodied. That means I often don’t fit in any particular group, but can relate to just about anyone.”

Doug works hard to promote inclusion of people with disabilities, or just about anyone who feels marginalized in today’s world. He is a leader in Johnson & Johnson’s Diversity and Inclusion community, serving on the global leadership team for the company’s Alliance for Diverse Abilities employee group. This group encompasses those with autism, disabilities, and mental health conditions. Doug also presented at a Johnson & Johnson TEDx event in June 2014, titled “A Billion People in the Shadows,” where he spoke about disability misconceptions. This talk is available to view on YouTube.

I kept waiting for people around me to tell me it was okay to be disabled, but eventually figured out that people mirrored my behavior. If I accepted my disability and was comfortable with it, then people around me would be comfortable with it.

~ Doug McCullough

Doug believes that “identity” is an interesting term in the disability community. Those in the SMA community often feel a roller coaster of emotions throughout their life from diagnosis to the challenges they face and the achievements they meet. “Despite the historical stigma of disabilities, I never thought of myself as disabled growing up and worked to hide my ‘shortcomings’ as much as possible. I learned SMA doesn’t define me, but it is part of who I am,” said Doug.

As Doug reflects, he shares this: “Every person is my ‘superior’ in some way and in that I learn from them. I am constantly learning from everyone around me and that certainly is true for everyone I meet in the Cure SMA community. My advice to fellow people with SMA, though it sounds simple, is to just do your best and have a positive attitude.”



Understanding Clinical Trials

Clinical trials for SMA are helping researchers answer important questions about the disease and offering new perspectives in the pursuit of future treatment options. For individuals with SMA and their families who may be considering participation in a clinical trial, below is information that can help in making an informed decision.

To determine if investigational treatments (e.g., new drugs, drug combinations, procedures, or medical devices) are safe and effective, clinical trials are conducted. This process can be long and complicated, with many steps in place to protect the safety of trial participants.

Benefits and Risks

Clinical trials are investigational in nature. There is no guarantee that participating in one will provide a medical benefit and, in fact, there may be risks involved – some known, some unknown. This is particularly true for Phase 1 trials when the investigational drug is tested in humans for the first time.

Although efforts are made to control potential risks, some may not be avoided because they may not be known. When potential risks are known, they must be fully explained by the study team to potential participants (or family members, depending on the age of the participant). However, there may be other potential benefits to consider that include:

- Contributing to SMA medical research.
- Monitoring of SMA and clinical trial-related care.
- Potential access to an investigational drug.

DID YOU KNOW?

In FY20, a portion of Cure SMA's clinical and regulatory funding went to increasing clinical trial site capacity and optimizing readiness by providing tools that would enhance sites' ability to conduct effective clinical trials in SMA. Additionally, Cure SMA has developed, and is continuing to develop, adult outcome measures for clinical trial use.

As part of the decision-making process, you will be given a chance to discuss the potential risks and benefits with the principal investigator. You should consider them as part of your decision. Moreover, you should decide only after having a full understanding of the risks that may be involved and what will be required of you or your family member as a participant.

Of note, receiving an investigational drug as a clinical trial participant does not guarantee improvement of symptoms. In fact, there is no guarantee that the investigational drug will have any effect, even making symptoms worse or leading to unwanted side effects. In some clinical trials, participants will not receive the investigational drug at all; rather, they will receive a placebo or sham, which are designed not to have any effect on the disease.

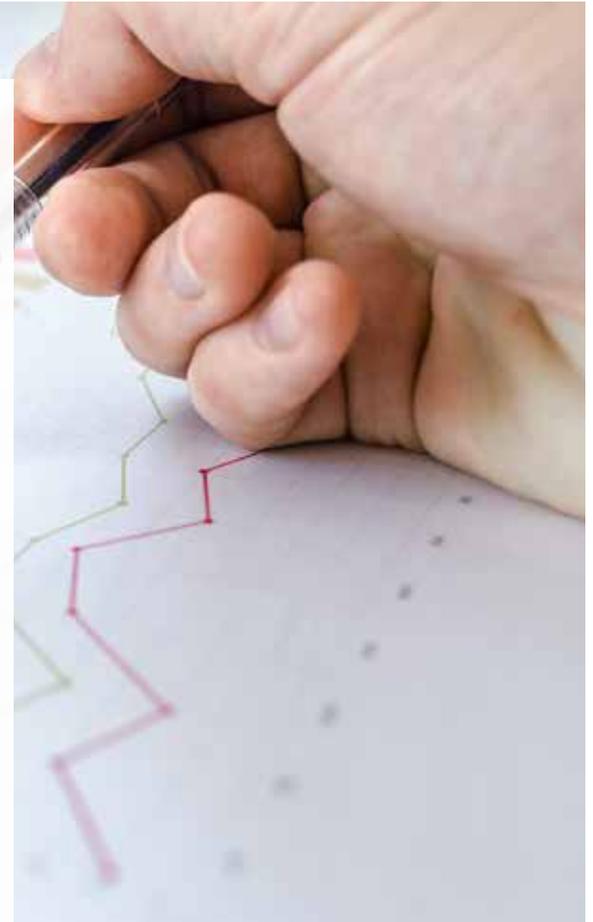
Responsibilities of Participants

Participation is always voluntary and you or your family member can withdraw at any time and for any reason (or no reason at all). Doing so will not affect the care that is being provided or that may be provided in the future.

There are certain responsibilities that clinical trial participants (or family members, depending on the age of the participant) are asked to follow, including:

- Following all instructions given by the study team.
- Attending all scheduled visits.
- Completing questionnaires about the status of the participant between visits.
- Telling the principal investigator of any new health-related problems. Even if you do not consider them to be caused by the clinical trial or the investigational drug, any small change is very important to report.
- Telling the principal investigator about any new medications or changes in doses or frequency of medications.
- Being mindful about discussing the clinical trial with other participants, including whether you think you or your family member may be receiving a placebo or sham.

If you decide to participate, you must also provide your consent. This is called the “informed consent process” and it is standard for participation in a clinical trial.



Learning About SMA Clinical Trials

There are many resources available if you are interested in learning about SMA clinical trials. In addition to your doctor, these include the Cure SMA website at <https://www.curesma.org/clinical-trials/> and the U.S. registry of trials, available at <https://clinicaltrials.gov/>.

Clinical Trial Phases

A drug must pass each individual phase of a clinical trial before advancing.

PHASE 1

Tests safety and dosage levels, usually on 10-20 individuals. Volunteers may be healthy individuals. However, for an orphan disease like SMA, some or all may be patients with the condition being studied.

PHASE 2

Tests a slightly larger group, usually 20-40 individuals, all of whom have the condition being studied.

PHASE 3

Increases the number of people tested (up to 100-200), including the control or placebo group. A drug that passes these three phases can be approved and marketed to the public.

PHASE 4

Primarily involves ongoing evaluation and monitoring, even after a drug is approved for the public.



Coping with Isolation by Bringing the Outside World In

By: Diane Murrell, LCSW and Danyelle Sun, MSW

The physical distancing we have had to comply with since the start of the COVID-19 pandemic continues to disrupt work and school routines, access to medical care, and the ability to connect socially with family and friends. When our emotional well-being has been stricken by isolation or stressors, what do we do to find a balance and restore ourselves? Do we sit under a favorite tree, on the beach, or in a crowded café? Where do you feel a moment of freedom that, no matter how short, brings respite and gets you ready to face the world again?

We can use sight, sound, smell, touch, and taste as sensory connections to bypass our negative thoughts and take hold of us in a comforting or exhilarating way. When you find yourself feeling isolated and disconnected from life as we used to know it, try some of these strategies below to help you feel grounded and reconnected.

Visual Connections

Go outside...amidst the simple beauty of nature...and know that as long as places like this exist, there will be comfort for every sorrow, whatever the circumstances may be.

~ Anne Frank, *Diary of Anne Frank*.

Being outside, where our senses are so alive, can bring hope, healing, and comfort. Nature has its own way of helping us through a season that requires much less in-person interaction. Check out apps like TrailLink, WildLab Bird, Yonder, and even Pokémon Go that can help you find new ways to connect with the outdoors.



Find something you're passionate about and keep tremendously interested in it.

~ Julia Child



Learning Connections

When your day-to-day becomes monotonous, consider taking up a new activity to expand on your thinking and experiences. From live painting and cooking classes you can easily find online to bird watching in your local park, there are many ways to inject some positive energy and activity into your days.

Auditory Connections

In this more isolated time we are living in, some people miss the buzz of large groups or the chatter of people being around. Background sounds can be comforting while you work, sleep, or mill around. You can bring the outside into your house by listening to crowd sounds to stimulate your brain's sense of being around others. You can use a home device, like an Amazon Alexa, which has playlists available to help you capture these sounds.

Tactile Connections

Physically distancing means that we are not hugging, hand holding, or engaging in other ways that involve touch, which we all need. Caring for plants and pets can help fulfill our need to talk to, touch, and care for other living things, even if not human. Caring for something releases our nurturing side that thinks beyond ourselves and gives us purpose. Both are scientifically reported to help reduce anxiety and depression.

Olfactory Connections

Smells can bring us immediately back to a positive, comforting memory. What smells recapture happy memories for you? Some people use aromatherapy with candles or oils to create feelings of well-being or relaxation. There are other types of smells, such as fresh baked cookies or a particular meal, that can arouse positive feelings of comfort and a time of feeling safe. Think of a smell you connect with positively and how to recreate it.



People Connection

As we experience “virtual fatigue,” it is important to find ways to connect with those we love. Get creative with virtual trivia nights and board games with friends. Games like Battleship, Guess Who, or Heads Up all work well when played through a screen. TeleParty is a great platform to watch movies virtually with others, while Roblox and Minecraft can be a fun way for kids to connect with their peers (while supervised, of course). The key is to achieve consistent connection. Research has shown that a daily phone call with the same person helps to combat loneliness and depression.

The simplest of routines can provide an anchor in uncertain times. When our brains and our souls are yearning for the comfort of those we love and for the experiences that make us feel alive, we can tap into our senses to recreate some of the comfort and connection we all need until the pandemic and physical isolation recedes.

“

We are hardwired to connect with others, it's what gives purpose and meaning to our lives, and without it there is suffering.

~ Brenè Brown

”

We hope you found this information useful. Do you have your own tips on how to manage isolation during these difficult times? Share them with Cure SMA and the rest of the community on Instagram, tagging @curesmaorg.

2021 National Partners

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

National Premier Partners



National Platinum Partner



National Gold Partners



Resources and Communications Partners



NEW!

Cure SMA Merchandise



\$2

WE. HAVE. STICKERS!

\$6

Each of the 6 stickers in the square sticker pack can be purchased separately in the online store!



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.

**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.**



In Memory of Piper Grace

This was written by Jessica Hoefler whose daughter, Piper, passed away in May 2020, shortly after being diagnosed with SMA Type o—the rarest form of SMA.

Piper Grace came into this world on Thursday, April 23, 2020, at 8:29 p.m. What appeared to be a standard pregnancy turned out, in retrospect, to be quite different. The birth itself was uneventful, quick, and natural. But when she came out gasping and struggling to breathe, they whisked her away to try and clear her lungs of fluid. It was at this point they became aware of her neuromuscular problems. She was immediately intubated and transferred, with my husband Ryan with her, to a children's hospital NICU.

By this point, I had only seen her for about 15 minutes, as I still had to check out of labor and delivery, but she had to get to the appropriate NICU right away. My easy recovery allowed for a morning discharge, so I could finally see my baby. After days of testing and consults with a team of doctors—ranging from geneticists, neuromuscular specialists, and neonatologists—we received the crushing diagnosis of SMA Type o, a very rare form of an already rare genetic condition.

Now we were faced with what choices, if any, there were for a baby diagnosed with a disorder few were familiar with. We were told that nothing would reverse her condition and any hope of her breathing, eating, or even moving (fine or gross movements) was impossible. In the end, she did not qualify for treatment, so our choices took on a different focus.

On Wednesday, May 20, 2020, at 4:32 p.m., the NICU staff orchestrated our final goodbye. I was able to carry her outside with a ventilator rolling behind me and our incredible NICU and Palliative Care family next to us. Outside in a beautiful courtyard surrounded by blooming hydrangea bushes, we sat with Piper as little raindrops fell on our cheeks. We felt the moment to remove her ventilator was upon us and just as the clouds parted, her tube was removed and Piper looked calmly into my eyes, free from medical equipment for the first time in her life. As the sun came out and beamed down on her, our sweet Angel took her last breath.



Piper's life began with a hectic gasp but ended with a peaceful, soft breath. Though she left the physical world, we know she is running free in Heaven with our late dog, Rudy. Piper Grace will forever be missed but we take comfort in knowing she is unlocked from pain and freed by love.



Clinical Care Update

Understanding COVID-19 Vaccines

The breakthrough in managing the COVID-19 pandemic are COVID-19 vaccines that have been, or are under review, with the U.S. Food and Drug Administration (FDA). COVID-19 vaccination is the best way to protect yourself and others from the virus. These vaccines have been demonstrated to be safe and effective at reducing COVID-19 disease and severity. Of note, data is not available regarding whether the vaccines prevent transmitting the COVID-19 infection.

In December 2020, two COVID-19 vaccines received Emergency Use Authorization (EUA) in the U.S. In late February 2021, a third vaccine also received EUA designation in the U.S. These COVID-19 vaccines are given as a shot into the arm muscle, or tissues if there is limited muscle, and stimulate the immune system to make antibodies against the SARS-CoV-2 virus spike proteins, which are used to enter and infect human cells. All are approved for adults.

Emergency Use Authorization (EUA) means that the vaccines were developed, studied, and approved during a time of a public health emergency. Authorization is vigorous and rapid, but under the unique circumstances of the COVID-19 pandemic. Evidence regarding each vaccine's effectiveness will continue to be reviewed. The EUA can be revised or revoked by the FDA at any time.

The Pfizer-BioNTech and Moderna vaccines are made from small amounts of genetic material, called single stranded messenger RNA (mRNA), wrapped in oily lipid particle bubbles to protect the mRNA. The material is injected, and the mRNA is taken up by the local cells. Human cells read this mRNA and make the virus spike proteins. The Johnson & Johnson vaccine is made from double stranded DNA of the virus spike proteins and added to modified Adenovirus 26 for transport into cells.

After the vaccine is injected, the DNA is taken up by the body's cells and moves to the nucleus where the DNA is read and copied into mRNA. mRNA moves out of the nucleus and spike proteins are made.

The spike proteins produced by vaccinated cells may protrude on the surface of the cells or may be released by the cells. These spike proteins are exposed to, and taken up by, the immune system cells. The immune system cells turn on antibody production and these antibodies recognize the SARS-CoV-2 surface protein. The immune system contains special cells called memory B cells and memory T cells, which recognize the coronavirus particles months after vaccination. At this time, it is not known how long this memory will last.

The most common reported COVID-19 vaccine side effects were short-term, including mild-to-moderate pain at the injection site and arm, swelling of the lymph nodes in the same arm of the injection, fatigue, headache, muscle pain, joint pain, chills, nausea or vomiting, and fever. More symptoms were reported after the second dose. Severe allergic reactions have been reported in people with a history of allergies. If you have a history of significant allergies, or a known allergy to any of the components of the vaccine, such as polyethylene glycol (PEG), please discuss the COVID-19 vaccine with your physician.

After you receive the vaccination it is important to continue to use double masks, good handwashing, and physical distancing until advised by the U.S. Centers for Disease Control and Prevention (CDC) that the isolation protocols in the U.S. can be relaxed.

Currently, there is no "best vaccine." The goal throughout the world is to have as many people as possible vaccinated as soon as possible. These vaccines do not interfere or cross react with any of the available prescribed SMA treatments. Please also consult with your healthcare provider.

COVID-19 Vaccines (cont.)

Rollout of COVID-19 vaccine has been slow, and vaccination is only available by appointment. State and local public health guidelines determine when each group may receive vaccine. Location options for securing an appointment for first dose include the following:

- Local public health vaccine facilities.
- Healthcare provider or healthcare system.
- Employer.
- Local pharmacies.

To see how states are prioritizing the disability community in COVID-19 vaccine distribution, see the COVID-19 Vaccine Prioritization Dashboard at <https://disabilityhealth.jhu.edu/vaccine/>. You can also find more resources at www.curesma.org/covid19/.

Real-World Evidence Collaboration Rollout

The Cure SMA Real-World Evidence Collaboration (RWEC) is established in 2021 to leverage the experience, expertise, and resources of pharmaceutical and biotechnology companies and nonprofit organizations involved in SMA to guide the future direction of real-world evidence collection and use in SMA. Current members include Biogen, Novartis Gene Therapies, Genentech, and SMA Europe.

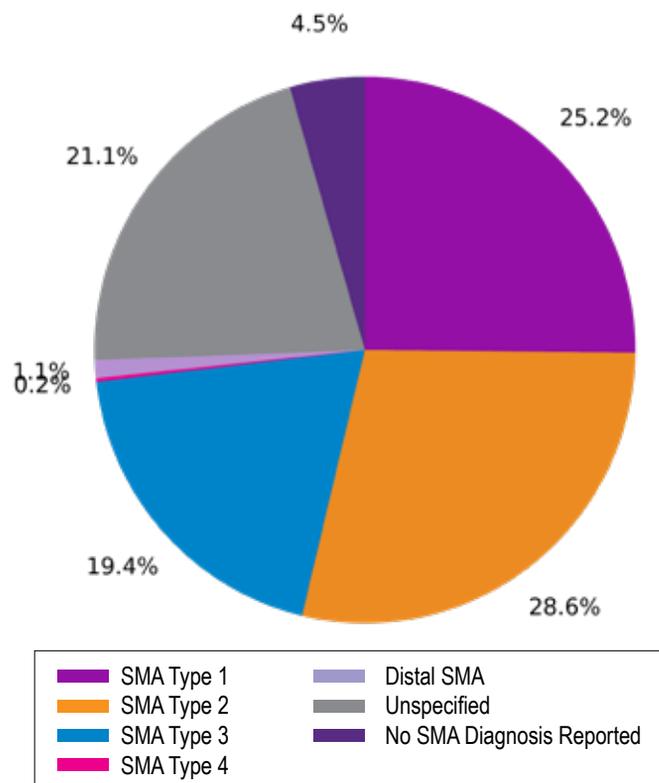
The primary goal of the RWEC is to lead efforts improved quality, and increased volume, of real-world evidence data collection and its use to improve the standard of care for SMA. Activities for 2021 include the following:

- Supporting the infrastructure for ensuring and validating data quality, accuracy, and completeness of the SMA Clinical Data Registry.
- Expanding the Cure SMA Care Center Network to achieve reliable representation of the SMA community experience.
- Reviewing the evidence to guide development of updated care and treatment guidelines and subsequent dissemination of SMA care information.

This collaboration will deliver an expanded clinical dataset to be used in the development of the updated standards of care, an additional 10 centers to join the Cure SMA Care Center Network, and standard of care updates on diagnosis, treatment considerations, and mental and emotional health.

SMA Clinical Data Registry Update

The SMA Clinical Data Registry continues to grow in partnership with the Cure SMA Care Center Network. As of January 31, 2021, the Registry includes 469 patients, while the Care Center Network has integrated 19 centers across the U.S.



SMA Newborn Screening Registry Invitation

As of January 31, 2021, 36 families have shared information about their child identified by SMA newborn screening. If SMA newborn screening helped to identify your child, we invite you to participate in our annual surveys to ensure we have the most up-to-date information. This will help us better understand the impact of SMA newborn screening.

You can register for the Cure SMA Newborn Screening Registry at <https://curesma-enrollment.rexdb.net/caregiver>.



Advocacy Update

Cure SMA Educates 117th Congress on Issues Important to SMA Community

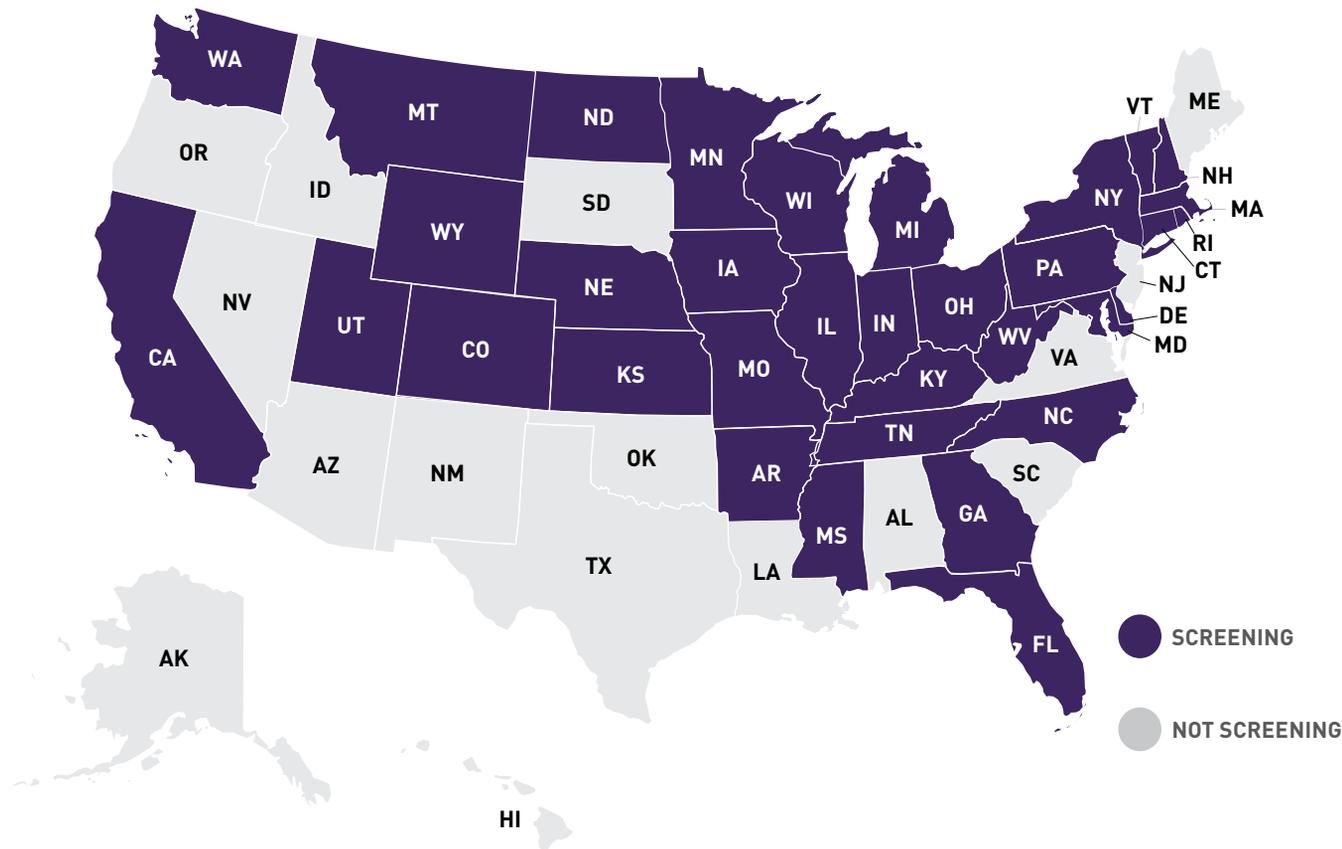
In the opening days of the new 117th Congress, Cure SMA wrote a letter to every Member of Congress to educate them on SMA and to highlight the issues important to the SMA community. These letters, signed by Cure SMA President, Kenneth Hobby, and Cure SMA Board Chair, Nicholas Farrell, described SMA, its prevalence, and highlighted the recent advancements in treatment, care, and newborn screening. They also outlined the community's priorities for the new Congress.

Cure SMA's advocacy agenda and outreach were developed in response to direct feedback from the SMA community. First, in November, as part of the Cure SMA Chapter & Leadership Meeting, we collected ideas from active members of the Cure SMA community. Then, in December, adults and young adults with SMA shared their top priorities with Cure SMA through an advocacy-related survey. As a result, we have engaged with several disability, health, and rare disease coalitions in Washington, D.C., in support of Cure SMA's advocacy agenda. In addition, Cure SMA will actively track and engage on activities during the 117th Congress and with the new Biden Administration.

Top Advocacy Issues: Adults and Young Adults with SMA

Thank you to the adult SMA community members for ranking their top priorities in a recent advocacy survey:

1. Healthcare
2. Employment & Financial Security
3. Disability Rights
4. Housing
5. Transportation
6. Technology
7. Education
8. Emergency Management
9. SMA Awareness



New Advocacy Resource Available for SMA Advocates

Advocacy Information Center

Below please find some advocacy resources for your use and advocacy actions that Cure SMA has taken on behalf of the SMA community.

Cure SMA's Advocacy Principles and Priorities

- Transportation
- Housing
- Healthcare
- Employment and Financial Security
- Disability Rights

Advocacy Resources

- Newborn Screening Fact Sheets by State
- Cure SMA Statement in Support of Rare Disease Council Legislation
- Newborn Screening Report
- COVID-19 Information Center
- Cure SMA Advocacy Action Center



Cure SMA Advocacy recently enhanced our online resources to help individuals with SMA and their families to stay informed and engaged on advocacy issues important to the SMA community. The Cure SMA Advocacy Information Center is a new one-stop center for Cure SMA advocates to access key advocacy resources—from state fact sheets and priority statements to quick links to the COVID-19 Information Center and other online resources. In addition, the Advocacy Information Center showcases Cure SMA's most recent advocacy-related letters and other coalition activities that Cure SMA has engaged in on behalf of the individuals with SMA and their families.

Cure SMA Records Most Successful Year for Newborn Screening Implementation

Despite COVID-19 and other challenges during the past year, 2020 represented the most successful year ever for implementation of newborn screening for SMA. A record 17 states started screening for SMA in 2020, through either permanent or pilot state newborn screening programs, bringing the total number of screening states to 33. The states that started SMA screening in 2020 were: Arkansas, California, Colorado, Connecticut, Delaware, Florida, Georgia, Illinois, Iowa, Kansas, Michigan, Nebraska, North Dakota, Rhode Island, Tennessee, Washington State, and Wyoming. Together, these 33 states screen nearly 7 in 10 babies born in the United States, a 32% increase over 2019 levels. Cure SMA is working with advocates and partners in the remaining non-screening states to continue implementation progress in 2021.

MILLION DOLLAR MOVEMENT

Fundraising Update

Million Dollar Movement – One Team. One Mission. One Million Dollars.

Cure SMA has always understood that our strength comes from the people in our community. It is the individuals with SMA who refuse to go unheard, the parents who make daily sacrifices, the grandparents and family members who offer unconditional support, the volunteers who donate their time, and the professionals who dedicate their lives to our cause. Together, these are the people who give us an unmatched strength that is changing the future of SMA.

It is this collective power that our Million Dollar Movement campaign is built on, and with your help, we can continue to shape a new, brighter future for people with SMA and their families.

We can help usher in a new generation of combination treatments that will build strength, create an evidence-based standard of care for SMA that will elevate protocols and help train the next generation of health care professionals, as well as continue to provide first-class Cure SMA support programs meeting the diverse needs of our community. By rallying around a common goal—our Million Dollar Movement campaign—we can achieve unprecedented progress in 2021 together.

I fundraise for Cure SMA because of my grandson, Henry. It feels good knowing that I am helping fund critical research and support programs that help all individuals with SMA and their families.

~ Sue Davis – Bryn Mawr, PA – Sue's grandson, Henry, is 3 years old and lives with SMA.

Join us this spring by walking, rolling, running, and fundraising your way to help Cure SMA achieve our goal of raising \$1,000,000 by June 30, 2021. Remember, when you fundraise for Cure SMA, you are not just raising money; you are making an investment in the SMA community. Email us at fundraising@uresma.org for help getting started.

Interested in joining our Million Dollar Movement?
Visit www.curesma.org/fundraise.



Walk-n-Roll

Create/join a team and walk, roll, or move your way with others nationwide.



Endurance

Join Team Cure SMA for a virtual marathon, half marathon, 10K, or 5K race.



DIY

Fundraise YOUR way! Start your own creative fundraiser.



Donate

Make a one-time or monthly gift to support the movement.

Online: Visit www.curesma.org/donate

Call: Call us at (800) 886-1762

Mail: Send your gift to Cure SMA, 925 Busse Road, Elk Grove Village, IL 60007

IMPACT REPORT



Cure SMA is thankful for every gift we receive, and your support helps us grow the research, care, advocacy, and support programs that the SMA community depends on. Every dollar goes to...

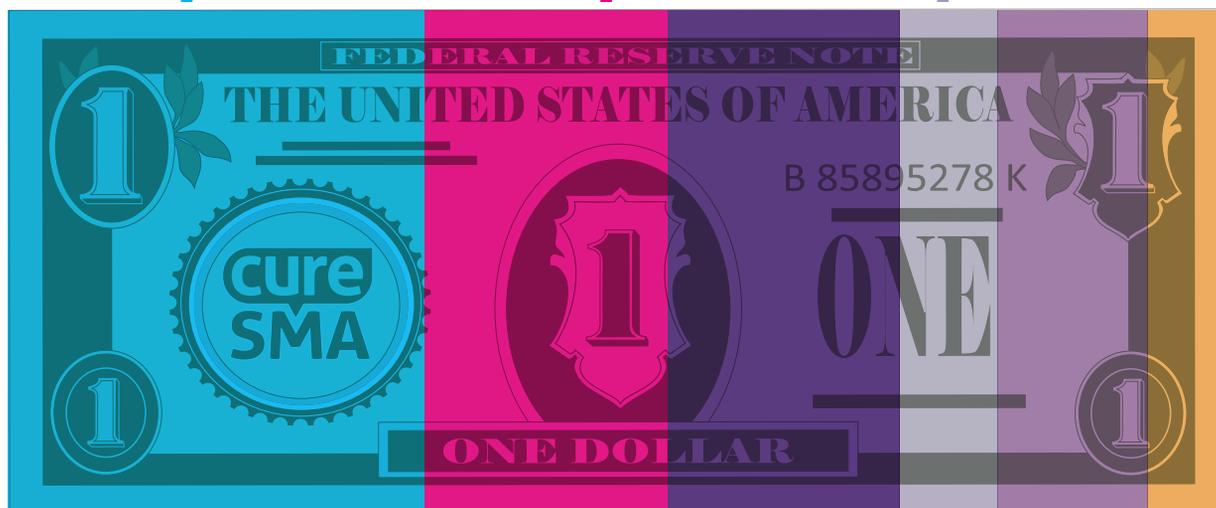
RESEARCH 34%

to advance the development of combination therapies that will help everyone with SMA increase voice strength, improve mobility, and build the stamina needed to effectively participate at work and in school.

CLINICAL CARE 20%

to continue gathering information from the Cure SMA Clinical Data Registry, track patient outcomes and treatment progression, and create an evidence-based standard of care for SMA.

MANAGEMENT & GENERAL 8%



COMMUNITY SUPPORT 19%

to grow our local presence and unite communities through support programs, ensure access to approved treatments, and create customized programs that meet the needs of everyone affected by SMA.

AWARENESS 7%

to advocate for access to care - including in-home care and personal care attendants, access to approved treatments, protections for COVID-19 vaccine allocation, and other needs for everyone with SMA.

FUNDRAISING 12%

Note: The figures above are taken from the 2020 fiscal year (07/01/2019-06/30/2020). Our complete financial information can be found at www.curesma.org/annual-reports-financials/.

Research Update

Update on the SMA Industry Collaboration



Cure SMA is pleased to announce the launch of an expanded Phase 6 of our SMA Industry Collaboration. The Collaboration is a multi-faceted partnership that brings together pharmaceutical and biotechnology companies, Cure SMA, and other nonprofit organizations involved in the development of SMA therapeutics to share information, ideas, and data to benefit the broader SMA community.

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

- Development and refinement of outcome measures.
- Distribution of a survey for adults and teens to evaluate unmet medical needs upon transition from pediatric to adult care.
- Distribution of a caregiver survey to assess quality of life within the caregiver community.
- Evaluation of opportunities to “bring trials to patients” via the adoption of telemedicine and remote monitoring.
- Development of a competency-based formal education program for SMA physical therapists.

The SMA Industry Collaboration is currently comprised of our partners at Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, Scholar Rock, and SMA Europe. The work of the Collaboration is supported by funds provided by Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, and Scholar Rock.

Manuscript Published on Quality of Life in Teens and Young Adults

Current knowledge regarding clinical meaningfulness and quality of life amongst teens and young adults with SMA is limited.

Much of the available qualitative data on this population has been obtained from the perspective of the parents/caregivers rather than the individuals themselves. Knowing this, Cure SMA is pleased to announce the publication of a manuscript, titled “I have SMA, SMA doesn’t have me: A qualitative snapshot into the challenges, successes, and quality of life of adolescents and young adults with SMA.” This piece, published in the February issue of *Orphanet Journal of Rare Diseases*, describes the clinical meaningfulness, quality of life, and the experiences of teens and young adults with SMA.

The manuscript presents the findings of two initiatives launched by Cure SMA in 2018. First, a clinical meaningfulness survey, completed by 85 individuals, captured information on the quality of life of this population. Cure SMA then hosted a video contest to raise awareness about the impact of SMA on the daily lives of teens and young adults, and to learn about their strategies to help navigate experienced challenges. An analysis of the survey and video content highlighted the impact of fatigue and lack of independence on school participation and the ability of respondents to complete activities of daily living. Also, while individuals noted the importance of relationships with family and friends on emotional well-being, many reported challenges with socialization due to accessibility concerns and activity limitations. However, despite adversity, many adolescents and young adults with SMA are leading productive lives. Participants highlighted numerous academic achievements and interests in a variety of activities, including video games, swimming, and wheelchair sports. To encourage resilience, many also suggested the creation of peer support groups, as well as college and independent living resources.

These findings help Cure SMA support and address the unique mind-sets, challenges, and motivations of adolescents and young adults with SMA. The support programming for teens and young adults remains a key priority at Cure SMA. Current programs include support packages, virtual social events, and useful tech products.

This study was financially supported by the SMA Industry Collaboration. At the time financial support was provided, members of the Collaboration included Novartis Gene Therapies, Biogen, Genentech/Roche Pharmaceuticals, Novartis Pharmaceuticals, Astellas, Cytokinetics, and Scholar Rock.

Recruiting and Ongoing Clinical Trials for SMA

The process of conducting clinical trials can be long, complicated, and difficult. Current research in SMA ensures that treatments are addressing the unmet needs of the SMA community, and that the community's priorities and goals are incorporated into the development, review, and approval of therapies. The following clinical trials are either recruiting or currently ongoing:

RESPOND, conducted by Biogen, is a two-year, open-label study to evaluate the efficacy and safety of Spinraza in SMA patients previously treated with Zolgensma to further optimize treatment decisions. The study will enroll 60 children up to 3 years old who are determined by the investigator to have the potential for additional clinical improvement after receiving Zolgensma. The first child was treated on January 8, 2021. This study is currently recruiting. More information can be found at www.clinicaltrials.gov using study code NCT04488133.

DEVOTE, conducted by Biogen, will examine the potential for greater efficacy, as well as the safety and tolerability of Spinraza, when administered at a higher dose. The trial is a Phase 2/3 randomized, controlled dose-escalating study that will be conducted at 50 sites around the world with a projected enrollment of 126 individuals with SMA of all ages, including adults. This study is currently recruiting. More information can be found at www.clinicaltrials.gov using study code NCT04089566.

TOPAZ, conducted by Scholar Rock, is a Phase 2 trial examining treatment with SRK-015, a selective inhibitor of the activation of myostatin, which works with other proteins and hormones to help regulate muscle mass. In August 2020, the U.S. Food and Drug Administration (FDA) granted Rare Pediatric Disease designation for SRK-015, and in October 2020, Scholar Rock announced positive results from an interim analysis of this trial. Top-line data for the 12-month treatment period are expected in the first half of 2021. This study is ongoing, but no longer recruiting. More information can be found at www.clinicaltrials.gov using study code NCT03921528.

RAINBOWFISH, conducted by Genentech/Roche, is an open-label, single-arm, multicenter study investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of Evrysdi in 25 infants from birth to 6 weeks old who have been genetically tested and diagnosed with SMA, but have not yet shown disease symptoms. Evrysdi is not FDA approved for people with SMA that are younger than 2 months of age. This study is currently recruiting. More information can be found at www.clinicaltrials.gov using study code NCT03779334.

JEWELFISH and FIREFISH, conducted by Genentech/Roche, are trials to further understand the efficacy and safety of Evrysdi, as well as evaluate safety and efficacy in additional populations. JEWELFISH is investigating the safety and tolerability of Evrysdi in people with all types of SMA aged 6 months to 60 years who have been previously treated with other SMA therapies. SUNFISH is evaluating the efficacy and safety of Evrysdi in people with SMA Types 2 or 3 aged 2 to 25 years. These studies are ongoing, but no longer recruiting. More information can be found at www.clinicaltrials.gov using study codes NCT03032172 and NCT02908685, respectively.

10-Year-Old Raises \$50,000 to Support a Cure SMA Research Grant



Dana Perella of COOKIES4CURES

Dana Perella is a 10-year-old on a mission to raise \$50,000 for SMA. She created Cookies4Cures to raise money to fund research for rare pediatric diseases. After becoming friends with Ben Lou, a 17-year-old with SMA, she set out to support our community by funding a Cure SMA research grant that could benefit Ben and thousands of others with SMA. In February 2020, she started baking cookies to raise money for Cure SMA and her plan was to inspire others to host their

own local bake sales and increase her campaign's impact. But as the COVID-19 pandemic hit, she quickly changed strategies and started sharing her Go Fund Me page to reach her goal. Dana worked with national companies to secure donations, held multiple virtual events, and received donations from family and friends from across the country. She even donated her prize money from winning the Gloria Barron Prize—which honors young heroes who are changing the world—as a match campaign leading up to #GivingTuesday. Dana shows the true spirit of philanthropy: the desire to promote the welfare of others. Cure SMA appreciates her work and dedication to our mission, and we look forward to seeing Dana's future work to help the SMA community! Visit www.cookies4cures.com for more information.



Community Support Update

Cure SMA Continues Virtual Community Events

Over the past year, Cure SMA has begun to offer a wide variety of virtual events to foster community engagement while we cannot gather in-person. Offering an entirely new way to engage and connect, some programs are for the full community while others help create peer-to-peer connection. You can learn more about our virtual programs at www.curesma.org/virtual-community-engagement-events/ and can email familysupport@curesma.org with any questions.

SOCIAL EVENTS



Virtual Kids & Teens Talk It Outs

Kids and teens in the community, aged 7-17 years, connected with old friends and met new friends through Zoom. These socials were facilitated by Dr. Al Freedman and Angela Wigglesworth, who run our in-person kids and teens sessions at the Annual SMA Conference. In these sessions, kids and teens were able to share, learn, and laugh together. Stay tuned for more dates in 2021!

Sponsors: Biogen, Genentech, Novartis Gene Therapies, and Scholar Rock



Virtual SMA Family Socials

Cure SMA connected SMA families and individuals from across the U.S. on Zoom, giving them an opportunity to chat and interact with old and new friends. Socials were either regional or national to give attendees from across the country the chance to virtually meet! The Cure SMA Illinois Chapter was even able to keep up the tradition of hosting a Holiday Party with a virtual guest appearance from Santa Claus!

Sponsor: Novartis Gene Therapies



Adults with SMA Virtual Evening & Morning Socials

These social events have offered adults with SMA a space to connect and come together for some social time. Evening socials are longer, 90-minute video calls providing a space to network, catch up with old friends, and meet new adults with SMA in the community. Meanwhile, morning socials allowed for quick, 30-minute interactions to check in with friends, have a quick mental break before a busy workday.

Sponsors: Biogen (evening socials) and Genentech (morning socials)

WEBINAR SERIES



Career Panel Webinar Series

Interested in learning about career paths, experiences, accommodations, and more from adults with SMA in the community? Check out our 3-part webinar series where you can hear from panelists about their career journeys, ask questions, and engage with the panelists. If you missed one, no worries! You can see archived career panel webinars on Cure SMA's YouTube Channel.

Sponsor: Biogen



Wellness Webinar Series

We want the SMA community to take time and focus on your own mental health and wellness. These 1-hour webinars included topics on mindfulness, laughter yoga, and family art-based therapy, and provide valuable tools and strategies for self-care and care for others. We invite you to view past webinars on Cure SMA's YouTube Channel.

Sponsor: Genentech



Physical Therapy Webinar Series

This series offers two, 1-hour webinars led by physical therapists who are experienced in providing care for both adults and children with SMA. These webinars provide information for exercises at home and attendees will receive a free therapy item upon registration. There will be one session focused on adults and another session focused on children, but if you missed your session, you can check it out on Cure SMA's YouTube Channel.

Sponsor: Biogen and The Luke 18:1 Foundation

JUST FOR ADULTS



Adults with SMA Virtual Book Club

Cure SMA's first book club covered the novel, *Disability Visibility: First-Person Stories from the Twenty-First Century*, edited by Alice Wong. Adults received the book as either a hard copy, e-book, or audible book and then discussed during two live, virtual events. Small groups were formulated to allow for robust discussion and sharing. We look forward to introducing our next book club pick soon!

Sponsor: Biogen



Adults with SMA Virtual Trivia Night

This fun event has been a great evening social for adults in the community! Working via TriviaHub, Cure SMA has hosted three virtual trivia nights, where random facts, lots of laughs, a bit of friendly competition were seen throughout the 90-minute events. We hope to see more adults with SMA joining in the future!

Sponsor: Biogen

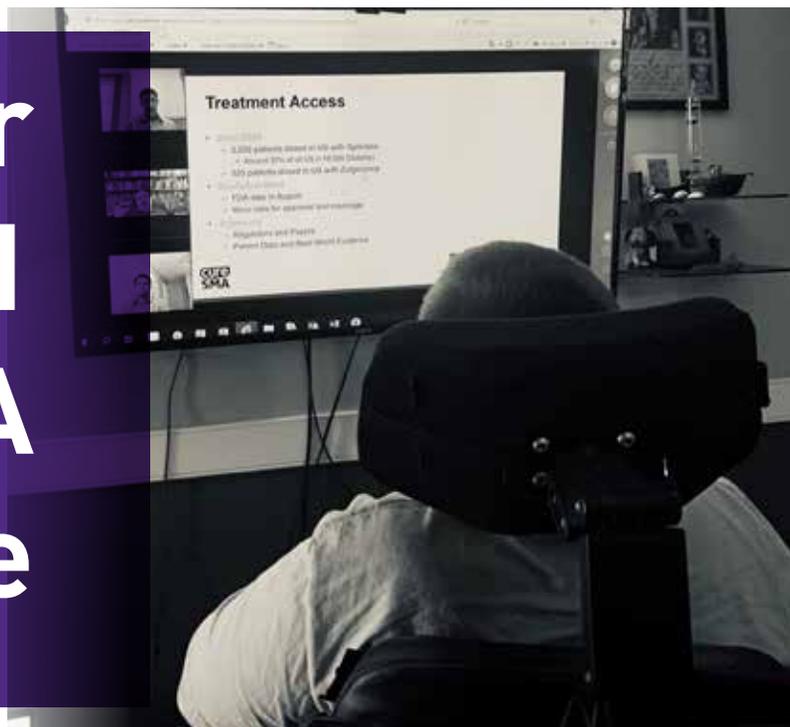


Adults with SMA Virtual Therapy Program

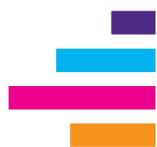
This program is available to adults with SMA, making virtual therapy more accessible during these times where we cannot get out. To learn more, visit our virtual engagement website to fill out our request form and be eligible to receive up to three (3) 30-minute live video sessions with a licensed therapist through the online therapy platform, TalkSpace.

Sponsor: Genentech and The Dhont Family Foundation

Join Us for the 2021 Virtual SMA Conference



Given the current state of the pandemic and COVID-19 vaccination roll-out, Cure SMA has decided that it will hold its 2021 Annual SMA Conference and SMA Research & Clinical Care Meeting virtually again this year. We look forward to hosting the SMA community for this important online event—Monday, June 7 through Friday, June 11, 2021.



**2021 Annual SMA Conference and SMA Research
& Clinical Care Meeting will be virtual!**



JUNE 7-11, 2021

We know how impactful this one week of the year is for individuals with SMA, families, researchers, and healthcare professionals alike, and for one more year, we welcome you back virtually as we come together to network, learn, and collaborate. We cannot wait to provide more opportunities to exchange ideas, engage in educational workshops, and interact with attendees from across the SMA community. Although, we will miss seeing everyone in-person, we are excited to still connect virtually!

Please visit our Annual SMA Conference page at

www.curesma.org/annual-sma-conference/

for more details on registration and to find out more details around this virtual week of community and fun!

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

We look forward to seeing you virtually in June!

2021 SUMMIT OF STRENGTH PROGRAM



Alongside the many virtual programs Cure SMA has launched in recent months, we are continuing our Summit of Strength Program—offering multiple ways to engage in 2021. This program, which has historically been held in-person across the country, was transformed to virtual for much of 2020. As we look at the year to come, we will continue to offer virtual webinars, while also carefully and safely beginning in-person Summits across the U.S. We believe both options offer people with SMA and their caregivers the opportunity to network and learn about the latest advances in SMA treatment, care, advocacy, and support.

The Summit of Strength Webinar Series in 2021 will offer a new variety of educational content specifically tailored for individuals with SMA and their families. These 1-hour presentations, which cover an array of support and medical topics, are held on many Wednesdays at 12:00 p.m. CT.

The local in-person Summit of Strength events will also be starting up again in 2021. This local engagement highlights Cure SMA's core values and further strengthens the bonds that make our local communities so vibrant! This is a free program which includes breakfast, lunch, and parking for all attendees. Cure SMA will be executing necessary COVID-19 precautions per guidelines from the U.S Centers for Disease Control and Prevention (CDC).

To learn more and to register for these virtual and in-person events, please visit www.curesma.org/summit-of-strength/.

If you have any questions, please reach out to familysupport@curesma.org

Disclaimer: Medical or legal opinions expressed or shared should not be substituted or interpreted as personal medical or legal advice. Please consult your healthcare provider regarding any health-related concerns.

Thank you to the National Presenting Sponsors for the 2021 Summit of Strength Program, Biogen and Genentech. We would also like to recognize Platinum Sponsor, Accredo, and Supporting Sponsor, Scholar Rock, for their support of the 2021 event series.

This year's event dates and locations include:

May 1 | Dallas, TX

May 15 | Raleigh, NC

June 26 | Philadelphia, PA

July 10 | Indianapolis, IN

July 31 | Kansas City, MO

August 7 | Orlando, FL

August 21 | Cincinnati, OH

August 28 | Minneapolis, MN

September 11 | Boston, MA

September 25 | New York City, NY

October 2 | Anaheim, CA

October 16 | San Francisco, CA

November 13 | Louisville, KY

November 20 | Jacksonville, FL

December 4 | New Orleans, LA

The health, safety, and well-being of the entire SMA community is important to us. Cure SMA is continually monitoring and evaluating the impact of COVID-19 in the U.S. and in-person summit events are subject to change.

2020
CURE SMA
EVENING OF

H O P E

A VIRTUAL MASQUERADE



Brad Nunemaker, Member of the Cure SMA Board of Directors, toasts the community during Cure SMA Evening of Hope: A Virtual Masquerade

Cure SMA Raises Over \$200,000 at Virtual Gala

On October 27, 2020, Cure SMA hosted the Cure SMA Evening of Hope: A Virtual Masquerade—the organization's first-ever virtual gala. The event was streamed live online and was an evening of celebration and hope that brought together members of the SMA community from the comfort and safety of their homes.

The virtual program featured a mission-focused program, toasts from the SMA community, an online silent auction, and so much more. Hosted by Emcee Damon Casatico, the evening program was heartfelt and included a fund-a-need appeal that raised funds for Cure SMA's latest research partnerships and initiatives with the Pediatric Neuro-muscular Clinical Research Network—a group of highly skilled clinical trial investigators, clinical evaluators, clinical coordinators, statisticians, and data management personnel in the SMA space.

The event's online silent auction featured more than 20 exciting packages, including signed sports memorabilia, wine packages, jewelry, as well as an Explorer Mini, which was generously donated by the Permobil Foundation. Thanks to the generosity of sponsors, attendees, and in-kind donors, Cure SMA raised more than \$200,000 to fund vital research programs that continue to further the mission of the organization.

The Cure SMA Evening of Hope: A Virtual Masquerade was made possible by these generous sponsors: Aveson Family, Barsotti Family Juice Company, Biogen, CTIA, Cytokinetics, Faegre Drinker, Genentech, North Canyon Cider Company, Novartis Gene Therapies, PhRMA, and Scholar Rock.



Making Moves with Team Cure SMA

Team Cure SMA is an endurance program that connects avid runners and cyclists with Cure SMA. Over the last two years, Team Cure SMA athletes from across the country have trained for endurance events while also raising money and awareness of SMA and Cure SMA.

In 2021, with many endurance events still to be postponed or cancelled due to the ongoing pandemic, we are making moves with Team Cure SMA and want you to consider joining our enhanced endurance program.

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

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



**MARK YOUR CALENDARS FOR
VIRTUAL RACE DAY ON SUNDAY,
MAY 23, 2021.**

**Thank you to the National Premier Sponsor
for Team Cure SMA, Genentech.**

Genentech



WALK - N - ROLL



Walk-n-Rolls from Across the Country Unite

In the fall of 2020, Cure SMA hosted two virtual national Walk-n-Rolls in partnership with 26 event committees from coast-to-coast. Supporters from across the country came together to show that the SMA community is stronger than ever! Together, these communities recruited 141 teams and 1,214 participants, secured 38 sponsors, and raised a total of \$340,493.99.

Throughout the fall, friends and families took part in various challenges and activities to celebrate their efforts, have fun together, and fundraise for Cure SMA. All activities culminated on a Walk-n-Roll day, where everyone came together to view Cure SMA's Celebration Ceremonies—one held in September and the other in November! More than 3,700 viewers tuned in on YouTube and Facebook to celebrate the SMA community's successes. Together, we also recognized what we were able to accomplish in 2020 by sharing community stories, welcoming special guests, and honoring our top fundraisers.

After the ceremony, participants and teams celebrated in their own unique ways. Many of them walked through local parks and neighborhoods, hiked, planned drive by parades, decorated their houses, set off rockets, or baked delicious treats!

Thank you to everyone who supported our Walk-n-Rolls in 2020!

A special thank you to the committees that made our fall Walk-n-Rolls a success!

- | | |
|--------------------------------|--------------------------------------|
| Arizona Walk-n-Roll | Madison Walk-n-Roll |
| Atlanta Walk-n-Roll | Minnesota Walk-n-Roll |
| Capital Region Walk-n-Roll/5k | Nashville Walk-n-Roll |
| Charlotte Walk-n-Roll | Northern California Walk-n-Roll |
| Chesapeake Walk-n-Roll | Oklahoma Walk-n-Roll |
| Colorado Walk-n-Roll | Omaha Walk-n-Roll |
| Fayetteville Walk-n-Roll/5k | Raleigh Walk-n-Roll |
| Greater Florida Walk-n-Roll | San Diego Walk-n-Roll |
| Greater New York Walk-n-Roll | Seattle Walk-n-Roll |
| Jogging for Joshua Walk-n-Roll | Sioux Falls Walk-n-Roll |
| Kansas City Walk-n-Roll | Vermont Walk-n-Roll |
| Kennedy's Dream Walk-n-Roll | Virginia Walk-n-Roll |
| Kentucky Walk-n-Roll | Western Illinois Suburbs Walk-n-Roll |

Shout Outs to Top Teams and Participants

Top Team Fundraisers

Hammerin Hank the Tank	\$20,730
Annie's Dragons!	\$20,350
Loving Melia	\$16,511
Charlie's Crew	\$12,510
Team Levi	\$10,400

Teams with Most Members

Bryker The Fighter	81
Genentech Grateful Hearts	65
Hammerin Hank the Tank	38
Team AveXis	37
Team Kayley	37

Top Individual Fundraisers

Cathy Barsotti-Dooling	\$12,000
Kevin Schaefer	\$10,225
Kim Sykora	\$4,505
Erin Quinnell	\$4,110
Ashley Fussell	\$4,000

Top First Time Teams

Team Blake (Oklahoma)	\$1,505
Mighty Maxson	\$1,340
Strong Like Stella	\$1,080
Team Chelsey	\$1,070
Team Grayson	\$1,070



**MARK YOUR CALENDARS FOR OUR NEXT VIRTUAL WALK-N-ROLL
EVENT ON SATURDAY, JUNE 5, 2021.**

Thank you to the National Premier Sponsors for these Cure SMA's Walk-n-Roll programs,
Biogen and Genentech



Fundraising Event *Spotlight:* Links 4 Luke



The Stickane Family

Luke 18:1 Foundation Donates \$350,000 to Cure SMA

The 3rd Annual Links 4 Luke Golf Tournament took place on September 29, 2020, at Timarron Country Club in Southlake, Texas. This annual event benefits the Luke 18:1 Foundation, whose mission it is to work together as a community to find a cure for SMA through raising awareness of SMA and early detection, funding research through fundraising events, and supporting individuals and families by connecting those impacted by SMA with meaningful programs and mentors to aid in their journey.

Players enjoyed 18 holes of golf with exciting course games, refreshments provided by community sponsors, as well as a program with dinner and a silent auction. Thanks to the generosity of the sponsors, players, and community, the Luke 18:1 Foundation was able to donate \$350,000 to Cure SMA to help fund critical research for new therapies, invest in 77 additional pieces of equipment for Cure SMA's loan program, and purchase 500 new TheraBands to support the Cure SMA's at-home physical therapy webinar series.

Thank you to the Luke 18:1 Foundation and the Stickane Family for their continued support and partnership with Cure SMA.

VISION AND MISSION STATEMENT

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

OUR VALUES

Innovation

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

Balance

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

Collaboration

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

Respect

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

Compassion

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

Determination

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



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

Submissions

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

Change of Address

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

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

On the cover:

Top row from left to right; Mcintosh Family, Brady Chan, and Emeily Flyr.

Bottom row from left to right; Ben Lou, Ayça Şahin, and Zoey Carpenter.



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August is

SMA Awareness Month



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