

2021 VIRTUAL

SMA

CONFERENCE



Dear SMA Community,

During this week of our 2021 Virtual SMA Conference we are going to focus on the recent progress we have made in SMA, and our goals as we get back to in-person activities for our community in the year ahead.

We have plans for record funding of programs in the year ahead. This will include all areas from research for new targets and treatments, combination approaches, increased support for clinical research and trials, and for expanding our Cure SMA Care Center Network. Greater funding will also be put into our support and advocacy programs for independence and participation and at a local level.

Over the past year we were able to beat our goals for reaching the key milestones of having more than 50 percent of our community on a treatment and having over 50 percent of all newborns screened for SMA.

With expanding access to treatments, new research and improving care are more important than ever. We need to optimize the impact of these therapies, develop combination approaches, and find additional targets to treat all ages, stages, and types of SMA.

This conference was made possible by the support of our generous sponsors. Thank you to the National Presenting Sponsors for the 2021 Virtual SMA Conference, Biogen, Genentech, and Novartis Gene Therapies. We would also like to recognize Platinum Sponsors, Cytokinetics and Scholar Rock, Gold Sponsor, Accredo, and Silver Sponsor, Orsini Specialty Pharmacy, for their support.

During the 2021 Virtual SMA Research & Clinical Care Meeting, researchers from industry and academia will meet to create open communication and collaboration, accelerating the pace of research for the SMA community. Multidisciplinary clinicians will share knowledge, ask questions, and network to optimize care and grow our SMA network of healthcare providers.

We hope that our new virtual platform for this year will provide additional opportunities to network and connect with others in the community. We thank you for joining, and for supporting these programs that help achieve our SMA community's goals. We also hope that you share in our excitement for the year ahead and what will come next for our community.

Sincerely,

Kenneth Hobby
President

Colleen McCarthy O'Toole
Vice President, Family Support

Jill Jarecki, PhD
Chief Scientific Officer

Mary Schroth, MD
Chief Medical Officer

Jessica Clark
Director, Family Support

Dear Cure SMA Community,

I am Nick Farrell, and as new Board Chair I wanted to introduce myself. I have been involved with Cure SMA for many years now. My family and I have been fundraising for nearly a decade, I have spent three years on the Board of Directors, and last year took on the role as Chair.

I live in Cincinnati, Ohio, with my wife Kacey, and our three daughters, Finley, Blake, and Holland. Our middle daughter, Blake (age 10), was diagnosed with Type 2 SMA in 2012 at the age of 14 months.

Blake showed signs of SMA in her first year, showing a delay in common infant milestones. She never walked and had trouble even standing. It was not until we were finally referred to a neuromuscular doctor that Blake was diagnosed, almost on the spot. The diagnosis was heartbreaking—especially in 2012 when there were no treatments available for SMA. However, my family stayed positive through it all and around Blake's 4th birthday we were fortunate to get her into a clinical trial. Now, with the arrival of multiple therapies for SMA, we are paying close attention to the potential for combination and muscle-targeting therapies that could hopefully accelerate her development.

We were introduced to Cure SMA by Blake's neuromuscular doctor at Cincinnati Children's Hospital when she was first diagnosed. Within days, we received our Newly Diagnosed Care Package from Cure SMA. Words cannot describe how that felt and how impactful it was for Blake and my family. We were so confused and scared at the time, trying to navigate what this diagnosis was going to mean for Blake. This contact from Cure SMA really made us feel at ease. We knew there were others out there who were like us, and others out there who were working every day to help families like ours. I will never forget that day.

For my family, being part of the community means knowing we are not alone. SMA can be scary and can feel isolating but knowing others in our same situation and drawing upon their support, knowledge, and experience is truly life changing. The SMA community is so very active, positive, and selfless; and Cure SMA plays a huge role in that!

My time on the Cure SMA Board has been focused on evolving it from a "working board" into a governance body in support of the staff. This also includes setting the strategy for capitalizing on the rapid changes the community has experienced in the past few years and facilitating the future growth we envision to better serve all members of our community. We have big plans, and these first three approved treatments are only the beginning.

I look forward to connecting and networking with all of you. Please feel free to reach out at any time.

Sincerely,



Nick Farrell
Chairman of the Board

2022 ANNUAL SMA CONFERENCE



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

After two years of hosting our conference virtually, we know more than ever how impactful this one week of the year is for individuals with SMA, their families, researchers, and medical professionals in the SMA community. We cannot wait to (finally!) welcome you back as we gather next June in California. The Disneyland Hotel will host the 2022 Annual SMA Conference and SMA Research & Clinical Care Meeting.

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

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

**WE LOOK FORWARD TO
SEEING YOU AT DISNEYLAND
NEXT JUNE!**

STAY TUNED

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

Thank you to our generous sponsors for their support of the 2021 Virtual SMA Conference. These partnerships offer a unique opportunity to enhance groundbreaking research and provide individuals with SMA and their families the support they need today.

PRESENTING SPONSORS



PLATINUM SPONSORS



GOLD SPONSOR

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SILVER SPONSOR



Thank you to the support of our exhibitors who helped make the 2021 Virtual SMA Conference a great success!

PLATINUM EXHIBITORS



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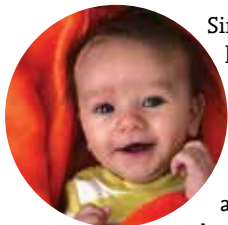
TRIUMPH MOBILITY

Make today a breakthrough.



Spinal muscular atrophy (SMA) is a disease that robs people of physical strength by affecting the motor nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe. **Historically, it has been the number one genetic cause of death for infants.** SMA affects approximately 1 in 11,000 babies, and about 1 in every 50 Americans is a genetic carrier. SMA can affect any race or gender. But there's great reason for hope. We now have multiple approved treatments that target the underlying genetics of SMA, with more treatments on the horizon. We know what we need to do to develop and deliver effective therapies. And we're on the verge of further breakthroughs that will continue to change the course of SMA for everyone—from infants to adults to families.

RESEARCH



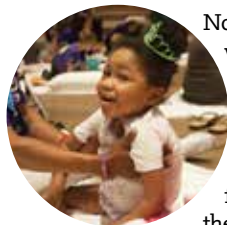
Since 1984, Cure SMA has led and invested in the research that has made today's breakthroughs possible. With deep connections and expertise in both the patient and research

communities, we're uniquely positioned to direct funds to where they can make the greatest difference as quickly as possible.

We have invested more than \$82 million in research and have funded half of the new drug programs for SMA, including all FDA-approved treatments for the disease.

We now have multiple FDA-approved therapies for SMA, and many more research programs ongoing.

COMMUNITY SUPPORT AND CARE

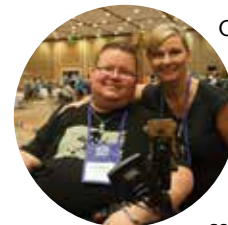


No matter your connection, we're committed to being an irreplaceable resource for anyone facing an SMA diagnosis and we seek to improve the quality of life for everyone with SMA so they can live active, engaged, and hopeful lives.

Each year, we send out approx. 4,000 information packets, newly diagnosed and teen/adult care packages, and much-needed equipment. We also support medical professionals through CME and our Care Series Booklets.

Because of FDA-approved treatments and successful initiatives, such as the Cure SMA Care Center Network and SMA Clinical Data Registry, we have made significant progress enhancing the lives of people with SMA.

CONFERENCE AND COMMUNITY



Our Annual SMA Conference brings together researchers and healthcare professionals, as well as individuals with SMA and their families, to network, learn, and collaborate.

The conference is the largest in the world focused specifically on SMA, and it attracts the top scientists and companies in the field.

Today, we have more than 170,000 members and supporters, with 36 volunteer chapters throughout the country. We host over 300 fundraising and awareness events annually.

2021 SUMMIT OF STRENGTH PROGRAM



Throughout the rest of 2021, Cure SMA will continue to offer virtual Summit of Strength webinars, while also carefully and safely hosting in-person Summit events 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.

Summit of Strength webinars will continue to be 1-hour presentations held on many Wednesdays at 12:00 p.m. CT. Meanwhile, local in-person Summits will further strengthen the bonds that make our local SMA communities so vibrant! These live events are free and include breakfast, lunch, and parking for all attendees. Cure SMA will execute the necessary COVID-19 precautions per government guidelines.

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.

2021 event dates and locations include:

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.

- 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.**

Show Your Cure SMA Pride



Save 25% on your entire Cure SMA merchandise order.

Use the discount code **2021conference** at checkout.

Offer good now through 6/11

CHECK OUT ALL items available on our online store at www.curesma.org/merchandise. Support our mission and raise awareness by purchasing some Cure SMA merchandise.

Share a photo on your social media of you sporting your Cure SMA gear during this week's Conference, using the hashtag #VirtualSMAConference. Or you can send your photo to stories@curesma.org and we can add to our community album.

VIRTUAL EXHIBIT HALL HOURS

Join us this week in our Virtual Exhibit Hall, where you will be able to learn more about each exhibitor of the 2021 Virtual SMA Conference. You are able to view each exhibitor page at any time throughout the week. You can message the company through the chat feature, leave your card for them to follow up with you, and for the following exhibitors, you can have a Live Zoom interaction during our Exhibit Hall Hours – Biogen, Genentech, Novartis Gene Therapies, Scholar Rock, Accredo, Orsini Specialty Pharmacy, Hayek Medical, and Permobil Foundation. Each time you visit an exhibitor page, you will be entered to win a piece of Cure SMA merchandise. Winners will be randomly chosen and be announced on Friday, June 11th, during the SMA Community Conference Closing Social.



TUESDAY, JUNE 8TH
FROM 4:00 PM – 6:00 PM CT



WEDNESDAY, JUNE 9TH
FROM 11:30 AM – 1:30 PM CT



THURSDAY, JUNE 10TH
FROM 3:00 PM – 5:00 PM CT



FRIDAY, JUNE 11TH
FROM 1:00 PM – 3:00 PM CT

MONDAY, JUNE 7TH (ALL TIMES ARE CT)

10:00 AM – 11:00 AM

WELCOME: OPENING SESSION

Kenneth Hobby, President, Cure SMA
Nick Farrell, Board Chair, Cure SMA

Cure SMA would like to welcome the SMA community together as we kick off the 2021 Virtual SMA Conference. This opening session webinar will be an overview of the recent progress we have made and our goals for the future.

12:00 PM – 1:30 PM

NEWLY DIAGNOSED VIRTUAL SESSION

(for newly diagnosed families only)

Kenneth Hobby, President, Cure SMA
Colleen McCarthy O'Toole, Vice President, Family Support, Cure SMA
Mary Schroth, MD, FAAP, FCCP, Chief Medical Officer, Cure SMA
Rob Graham, MD
Jessica Clark, Director, Family Support, Cure SMA
Danyelle Sun, SMA Mother, Type 2 and Type 3
Amanda DeVay, SMA Mother, Newborn Screening, Type 1
Al Freedman, SMA Father, Type 1

This webinar is specifically for newly diagnosed families and will focus on an overview of Cure SMA, treatment and trials, as well as a parent panel where parents share their journey.

2:00 PM – 3:00 PM

FAMILY SYMPOSIUM, SPONSORED BY GENENTECH: TOGETHER IN ACTION: A COMMUNITY DISCUSSION ABOUT EVERYDAY EXPERIENCES

Genentech
A Member of the Roche Group

EVENING SOCIAL EVENTS

5:00 PM – 6:00 PM

NEWLY DIAGNOSED MINGLE

Connect with other families and individuals diagnosed within the last couple of years and share experiences, provide support, and join as a community in this Zoom mingle!

6:00 PM – 7:00 PM

GRANDPARENTS NETWORKING SOCIAL

Join other grandparents who are affected by SMA as you “mingle” together at home. Connect, network, and chat through Zoom.

7:00 PM – 8:30 PM

ADULTS WITH SMA VIRTUAL TRIVIA NIGHT

Looking to kick-off this year's Virtual SMA Conference with a fun social event. Join us for this 90-minute Trivia Night for adults with SMA, where you can gather online in a friendly setting. Through Zoom breakout rooms, attendees will be able to network, catch up with old friends, and meet new adults with SMA in the community! Socials are only for adults with SMA over the age of 18 years and is hosted by Trivia Hub!

TUESDAY, JUNE 8TH (ALL TIMES ARE CT)

10:00 AM – 10:45 AM

THE PRACTICAL SIDE OF THE POOL

Jennifer Martyn, PT, Wave Therapies, PLLC

Since we cannot be together in the pool this year, come join us for some video time in the water. See demonstrations of our favorite exercises, equipment, and tips for maximizing your time in your hot tub, pool, or out on the lake.

10:30 AM – 11:15 AM

SPECIAL PLANS FOR A SPECIAL LIFE

Richard Rubenstein, Esq.

Legal and financial planning that parents must undertake to protect their family can be difficult and confusing. It is vital for all parents to have an estate plan in place, more so for parents of children with disabilities. Find out how to assure that the money you set aside for your child with special needs does not jeopardize government benefits your child is entitled to receive. Richard will give a general overview of the basics of estate planning and the differences between the various types of Special Needs Trusts. He will also discuss various techniques to advocate on behalf of your child in order maximize resources from insurance companies and government agencies. Parents must go through these difficult steps to ensure a well-planned future for their special child. Join this interactive session where Richard will connect with attendees answering questions live.

11:00 AM – 11:45 AM

REPRODUCTIVE OPTIONS FOR SMA FAMILIES

Harvey Stern, MD PhD, FACMG, FAAP

This workshop will briefly describe the genetics of SMA, concentrating on the reproductive options available to families, including prenatal diagnosis of an ongoing pregnancy by chorionic villus sampling or amniocentesis. Preimplantation Genetic Diagnosis with in-vitro fertilization will be discussed in detail.

11:30 AM – 12:15 PM

ADDRESSING THE MISSING PIECE: MENTAL HEALTH AND SMA

Julia Feinberg, Adult with SMA

Life with SMA can be exhausting. We are constantly reminded to keep track of our physical health, stay in touch with our doctors, and maintain healthy habits. But how often do we consider our mental health? People with disabilities and their loved ones deserve thoughtful, comprehensive mental health care. Join this session to learn about why mental health is critical for people with SMA, understand what causes mental health issues, and what you can do about it. This session will be informative and include helpful tools for you to take with you after the session.

12:00 PM – 12:45 PM

TAKING CHARGE OF YOUR HEALTH: THE TRANSITION FROM PEDIATRIC TO ADULT CARE

Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN

Bakri Elsheikh, MBBS, FRCP

Diane Murrell, LCSW

Sarah Stoney, MSW, LSW

This workshop will focus on the following aspects of transition from pediatric to adult healthcare: Understand the importance of “why” transition; identify some of the transition skills needed before transferring to adult care; a single clinic study on Advance directives; and, an adult provider’s perspective on adult clinics and differences you might anticipate. This session is ideal for parents, adolescents, and young adults who are preparing transitioning from pediatric to adult care.

TUESDAY, JUNE 8TH (ALL TIMES ARE CT)

12:30 PM – 1:15 PM

SLEEP PROBLEMS SEEN IN SMA

Richard Kravitz, MD

Individuals with SMA are at risk for various sleep disorders. Whether it is caused by their underlying muscle weakness or due to normal childhood development, families need to be aware of these disorders so that they can be brought to the attention of their primary care providers and SMA care team. There are various diagnostic tools that can be utilized in establishing the etiology of these sleep problems. Both medical and behavioral interventions are available that are useful in the management of sleep disorders. With the arrival on SMN modulators, the standard of care is changing, and families need to be aware of the options now available to help provide for a good night's sleep. In this workshop, we will discuss how SMA impacts sleep and what options are available to optimize both the individual's and their families' sleep so that all can be well rested.

2:00 PM – 3:00 PM

FAMILY SYMPOSIUM, SPONSORED BY BIOGEN: MEETING THE CHALLENGES OF SMA: PIVOTAL DATA IN LATER-ONSET SMA AND INDEPENDENT, OBSERVATIONAL DATA IN ADULTS



4:00 PM – 6:00 PM

VIRTUAL EXHIBIT HALL

Explore informational booths during our Virtual Exhibit Booth Hours. Chat with the companies, leave your card for them to follow up with you, and explore the resources, products and features each booth has to offer. As you do this, you will be entered to win a piece of Cure SMA merchandise for each day you attend and visit a booth. Winners will be randomly chosen and announced on Friday, June 11th, during the SMA Community Conference Closing Social.

EVENING SOCIAL EVENT

7:00 PM – 8:30 PM

MOMS' NIGHT IN

Calling all Moms! Join each other for this evening's social to catch up and wind down after a full day of virtual conference sessions. Pour yourself a favorite beverage and join in on this fun mingle!

WEDNESDAY, JUNE 9TH (ALL TIMES ARE CT)

10:00 AM – 10:45 AM

NAVIGATING THE SPECIAL EDUCATION PROCESS: SETTING OUR CHILDREN UP FOR SUCCESS IN PRESCHOOL AND ELEMENTARY SCHOOL

Kimberly Cook, Inclusion Specialist, SMA Parent
Tina Lewis, Reading Support Teacher, SMA Parent

As you embark on the journey to send your preschool or elementary child to school, this session will set you and your child up for success as we share our own experiences and expertise in navigating the special education process for children with SMA. In this session, we will discuss special education timelines, distinguish between a 504 and an Individualized Educational Plan (IEP), as well as share considerations for services and accommodations that will make each day a successful one for you and your child. A brief question and answer session will follow.

10:30 AM – 11:15 AM

HEALTHY NUTRITION STRATEGIES FOR ADULTS

Stacey Tarrant, BS, RD, LDN
Rebecca Hurst Davis, MS, RD, CSP, CD, CNSC
Laura Watne, MS, RD, CSP

This adult-only session will address optimal health through good nutrition for adults with SMA. We will talk about stocking your pantry for quick healthy meals and snacks on the go, tips for maintaining a healthy weight, healthy eating when swallowing is difficult, nutrition lab numbers that adults should know, and more! Come with your questions for the live Q&A at the end.

11:30 AM – 1:30 PM

VIRTUAL EXHIBIT HALL

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2:00 PM – 3:00 PM

FAMILY SYMPOSIUM, SPONSORED BY NOVARTIS GENE THERAPIES: VIRTUAL CAREGIVER ROUNDTABLE - RAISING OUR VOICES FOR SMA



3:30 PM – 4:15 PM

USING YOUR BREATH FOR BIG EMOTIONS

Tara Davenport, SMA Mother

What is light as a feather but even the strongest person on earth cannot hold it for very long? Your breath!! Hey kids and caregivers! Join Tara Davenport and her nine-year-old daughter, Claire Miles, as they share ideas on how to use your breathing to help you when things get tough. Tara and Claire will lead you through hands-on activities to try and ideas on how to use them.

4:00 PM – 4:45 PM

DO IT YOURSELF FUNDRAISING: SUPPORT CURE SMA BY FUNDRAISING YOUR WAY

Erin Oganessian, Vice President of Development, Cure SMA

Join us as we walk through how to plan and execute a Do IT Yourself (DIY) fundraiser to benefit Cure SMA! Hosting a fundraiser is a terrific way to spread awareness and support our mission. We will provide resources, tips, and tricks so you can fundraise your way.

4:30 PM – 5:15 PM

ORTHOPEDIC MANAGEMENT IN SMA

Samuel Rosenfeld, MD
Brian Snyder, MD

This workshop will focus on life with SMA as it pertains to maintaining the best possible orthopedic management. It will also address the orthopedic complications of SMA. Children with SMA often have musculoskeletal impairments that interfere with mobility, function, and efficiency. This can contribute to restrictive pulmonary disease. Orthopedic intervention can improve or stabilize these impairments and help prevent deterioration in function. Orthopedic surgical procedures can correct hip instability and scoliosis, facilitate orthotic management, and accommodate the demands of continued growth. Musculoskeletal integrative medicine is important in normal development, especially in the child with SMA.

WEDNESDAY, JUNE 9TH (ALL TIMES ARE CT)

5:00 PM – 5:45 PM

THE ABLE ACT...ENABLE SAVINGS FOR LIFE

Mary Anne Elhert, CFP

This presentation will be educational and interactive. Mary Anne will address what you need to know regarding The ABLE Act in your state and the pros/cons of having an ABLE Account. Topics of discussion include: What is an ABLE Act, is your child eligible, the impact on other resources, how much money are you allowed, what type of accounts are allowed, and how to set up an ABLE Account.

EVENING SOCIAL EVENTS

5:30 PM – 6:30 PM

NEWBORN SCREENING SOCIAL

Join in on this friendly social for families with children who were diagnosed through newborn screening. Connect, network, and chat through Zoom with others in the community.

6:30 PM – 7:30 PM

TEENS WITH SMA TRIVIA NIGHT

Teens with SMA ages 13-17 years are invited to test their intellect and random knowledge in these entertaining quizzes. Teams will be created on Zoom to collaborate and compete for three rounds of trivia questions with a fun theme of Disney Movies!

7:00 PM – 8:30 PM

ADULTS WITH SMA EVENING SOCIAL

This virtual social will offer an opportunity for adults with SMA to gather online. Through Zoom breakout rooms, attendees will be able to network, catch up with friends, and meet new adults with SMA in the community! This social is only for adults with SMA ages 18 years and older.

THURSDAY, JUNE 10TH (ALL TIMES ARE CT)

10:00 AM – 10:45 AM

EARLY SCOLIOSIS MANAGEMENT IN SMA

Samuel Rosenfeld, MD
Brian Snyder, MD

This workshop will focus on care of early scoliosis with SMA, including monitoring and management considerations and strategies.

10:30 AM – 11:15 AM

PRE-SYMPTOMATIC PHYSICAL THERAPY CONSIDERATIONS

Leslie Nelson, PT, PhD, OCS
Anne Stratton, MD

This workshop will focus on physical therapy considerations for pre-symptomatic individuals with SMA including monitoring and management considerations and strategies.

11:00 AM – 11:45 AM

REHABILITATION OF SPEECH AND SWALLOWING DEFICITS IN CHILDREN AND ADULTS WITH SMA

Kristen Allison, MD
Katlyn McGrattan, MD

Deficits in speech, voice, chewing, and swallowing are frequently reported by older children and adults with SMA. In this lecture, Dr. Katlyn McGrattan and Dr. Kristen Allison will explain the evidence and theoretical models from other populations to guide treatment of these speech and swallowing deficits in older children and adults with SMA.

11:30 AM – 12:15 PM

FERTILITY, PREGNANCY AND SMA

Ahemd, Ahmed, MD
Snigdha Alur-Gupta, MD,
Debra Guntrum, MS, FNP, ANCC

This workshop will highlight the unique obstetrical issues confronted by patients with SMA and will highlight the importance of identifying specific risks encountered during pregnancy, in addition to providing recommendations to improve maternal and fetal outcomes. A vital component of successful pregnancy for a woman with SMA is a multidisciplinary approach in a tertiary facility with familiarity in managing neuromuscular disorders.

12:00 PM – 12:45 PM

NAVIGATING THE SPECIAL EDUCATION PROCESS: TRANSITIONING TO MIDDLE SCHOOL, HIGH SCHOOL, AND COLLEGE

Kimberly Cook, Inclusion Specialist, SMA Parent
Tina Lewis, Reading Support Teacher, SMA Parent

Knowing what you can expect for your child and asking for appropriate services and accommodations can be difficult, especially when your child is transitioning into a new environment. We will be discussing transition planning, tools for independence, and disability services typically available for students with disabilities who plan to attend college. Question and answer session to follow.

12:30 PM – 1:15 PM

SMA NUTRITION - YOU'VE ASKED, WE'VE ANSWERED

Stacey Tarrant, BS, RD, LDN
Rebecca Hurst-Davis, MS, RD, CSP, CD, CNSC
Laura Watne, MS, RD, CSP

What is the best diet for someone with SMA? What supplements will help a child with SMA? What is the best tube feeding formula? These are just some of the questions that families frequently ask during a nutrition visit with an SMA dietitian. During this workshop, we will answer these questions and more, and if we have missed anything that you want to know, there will be a live Q&A at the end of the session.

3:00 PM – 5:00 PM

VIRTUAL EXHIBIT HALL

Explore informational booths during our Virtual Exhibit Booth Hours. Chat with the companies, leave your card for them to follow up with you, and explore the resources, products and features each booth has to offer. As you do this, you will be entered to win a piece of Cure SMA merchandise for each day you attend and visit a booth. Winners will be randomly chosen and announced on Friday, June 11th, during the SMA Community Conference Closing Social.

EVENING SOCIAL EVENT

7:00 PM – 8:30 PM

DADS' NIGHT IN

Attention Dads! Join each other for this evening's social to catch up and wind down after a full day of virtual conference sessions. Pour yourself a favorite beverage and join in on this fun mingle!

FRIDAY, JUNE 11TH (ALL TIMES ARE CT)

10:00 AM – 10:45 AM

DECISION MAKING IN SMA

Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN,
Rob Graham, MD
Gina Santucci, MSN FNP APRN-BC,
Julie Parsons, MD

Individuals and families of children with SMA are required to make many decisions regarding care. Improvements in healthcare, as well as advancements in technology, are providing individuals with SMA better quality of life. For some, determining what interventions make sense can be challenging, as some decisions may lead to unexpected outcomes. In this workshop, we will consider options presented to families and potential consequences that may affect quality of life. We will also identify signs of pain and explore various modalities to treat pain in individuals with SMA.

10:30 AM – 11:15 AM

RESEARCH AND CLINICAL CARE UPDATES

Jill Jarecki, PhD, Chief Scientific Officer, Cure SMA
Mary Schroth, MD, FAAP, FCCP, Chief Medical Officer, Cure SMA

As we close out our last day of our Virtual SMA Conference, Cure SMA will provide updates on from Cure SMA leaders on research and clinical care topics.

11:00 AM – 11:45 AM

RESPIRATORY CARE IN SMA

Richard Kravitz, MD
Oren Kupfer, MD
Peter Schochet, MD
Jane Taylor, MD

Individuals with SMA are at risk for difficulty breathing due to muscle weakness. This workshop will discuss why the muscle weakness causes difficulty breathing during sleep, illness, and after surgery, as well as how to know when breathing support machines (e.g., BIPAP) should be considered. We will talk about the modalities available to use at home and in the hospital, as well as strategies to help your care team support respiratory health at home. In this workshop, we will discuss how cough works and why it is important, why cough strength is diminished in SMA, how we can measure cough strength, and how we can help cough function and limit respiratory infections and hospitalizations.

11:30 AM – 12:15 PM

OPTIMIZING TELEHEALTH

Diana Castro, MD
Leslie Nelson, MD
Sarah Stoney, MSW, LSW
Stacey Tarrant, BS, RD, LDN

Come and be part of the discussion surrounding how to best optimize your telehealth appointment and future considerations and best practices.

1:00 PM – 3:00 PM

VIRTUAL EXHIBIT HALL

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3:30 PM – 4:15 PM

GETTING IN THE POOL WITH AND WITHOUT A PANDEMIC

Jennifer Martyn, PT, Wave Therapies, PLLC

This time will look at why and how to safely access therapeutic water. Join this discussion on accessing water, what to look for in a facility, what to do when you get there, as well as what to consider from a health and safety side. This time of COVID provided fresh insights into each of these aspects.

4:00 PM – 4:45 PM

CHANGING YOUR RELATIONSHIP TO YOUR THOUGHTS WITH MINDFULNESS

Tara Davenport, SMA mother

Have you ever thought you cannot stop your thoughts? Good news! You do not have to! Through mindfulness meditation you do not try to stop thoughts, but you can change the way you relate to them, which can bring more peace to your life. And we can all use a little more peace right now! How we relate to our thoughts has a big impact on how we go through our day and relate to those around us. By incorporating mindfulness practices, we can learn ways to improve that relationship, understand how our thoughts can hijack us, and a hack to gently regain control. Join Tara Davenport, mother to a nine-year-old with SMA, as she shares mindfulness ideas on how to change how you relate to your thoughts for more peaceful days.

EVENING SOCIAL EVENT

6:30 PM – 7:30 PM

SMA COMMUNITY CONFERENCE CLOSING SOCIAL

Join the entire SMA community—individuals with SMA, families, researchers, and healthcare providers—in a special end-of-conference celebration. Be sure to wear your 2021 Virtual SMA Conference t-shirt and/or Cure SMA gear as we close out this year's virtual conference together!

SOCIAL EVENTS

MONDAY, JUNE 14TH

5:30 PM – 6:45 PM

KIDS VIRTUAL TALK IT OUT

(KIDS ONLY: AGES 7 - 12)

Al Freedman, moderator, PhD, SMA Dad and Child Psychologist
Angela Wrigglesworth, moderator, Elementary Education Teacher, SMA Adult

Throughout this 75-minute session, children with SMA will have an opportunity to talk with each other about their lives, meet new friends, and catch up with old friends online! Pre-registration is required and will be on a first come, first serve basis.

WEDNESDAY, JUNE 16TH

11:30 AM – 12:45 PM

KIDS VIRTUAL TALK IT OUT

(KIDS ONLY: AGES 7 - 12)

Al Freedman, moderator, PhD, SMA Dad and Child Psychologist
Angela Wrigglesworth, moderator, Elementary Education Teacher, SMA Adult

Throughout this 75-minute session, children with SMA will have an opportunity to talk with each other about their lives, meet new friends, and catch up with old friends online! Pre-registration is required and will be on a first come, first serve basis.

5:30 PM – 7:00 PM

TEENS VIRTUAL TALK IT OUT

(TEENS ONLY: AGES 13 - 17)

Al Freedman, moderator, PhD, SMA Dad and Child Psychologist
Angela Wrigglesworth, moderator, Elementary Education Teacher, SMA Adult

Throughout this 90-minute session, teen with SMA will have an opportunity to talk with each other about their lives, meet new friends, and catch up with old friends online! Pre-registration is required and will be on a first come, first serve basis.

THURSDAY, JUNE 17TH

11:30 AM – 1:00 PM

TEENS VIRTUAL TALK IT OUT

(TEENS ONLY: AGES 13 - 17)

Al Freedman, moderator, PhD, SMA Dad and Child Psychologist
Angela Wrigglesworth, moderator, Elementary Education Teacher, SMA Adult

Throughout this 90-minute session, teens with SMA will have an opportunity to talk with each other about their lives, meet new friends, and catch up with old friends online! Pre-registration is required and will be on a first come, first serve basis.

JOIN US!

SMA COMMUNITY CONFERENCE CLOSING SOCIAL

To cap off the 2021 Virtual SMA Conference and Virtual SMA Research & Clinical Care Meeting, we want to see everyone come together and celebrate. Pop in online to mix and mingle with fellow members of the SMA community and don't forget to wear your conference t-shirt or other Cure SMA gear!

Friday, June 11th at 6:30 p.m. CT

cure
SMA



#VirtualSMAConference and #CureSMA

SMA DRUG PIPELINE

We are funding and directing research with more breadth and depth than ever before. We know what we need to do to develop and deliver new therapies, or even combinations of therapies, to reach our goal of treatments for all individuals with SMA. And we are on the verge of further breakthroughs that will continue to change the course of SMA.

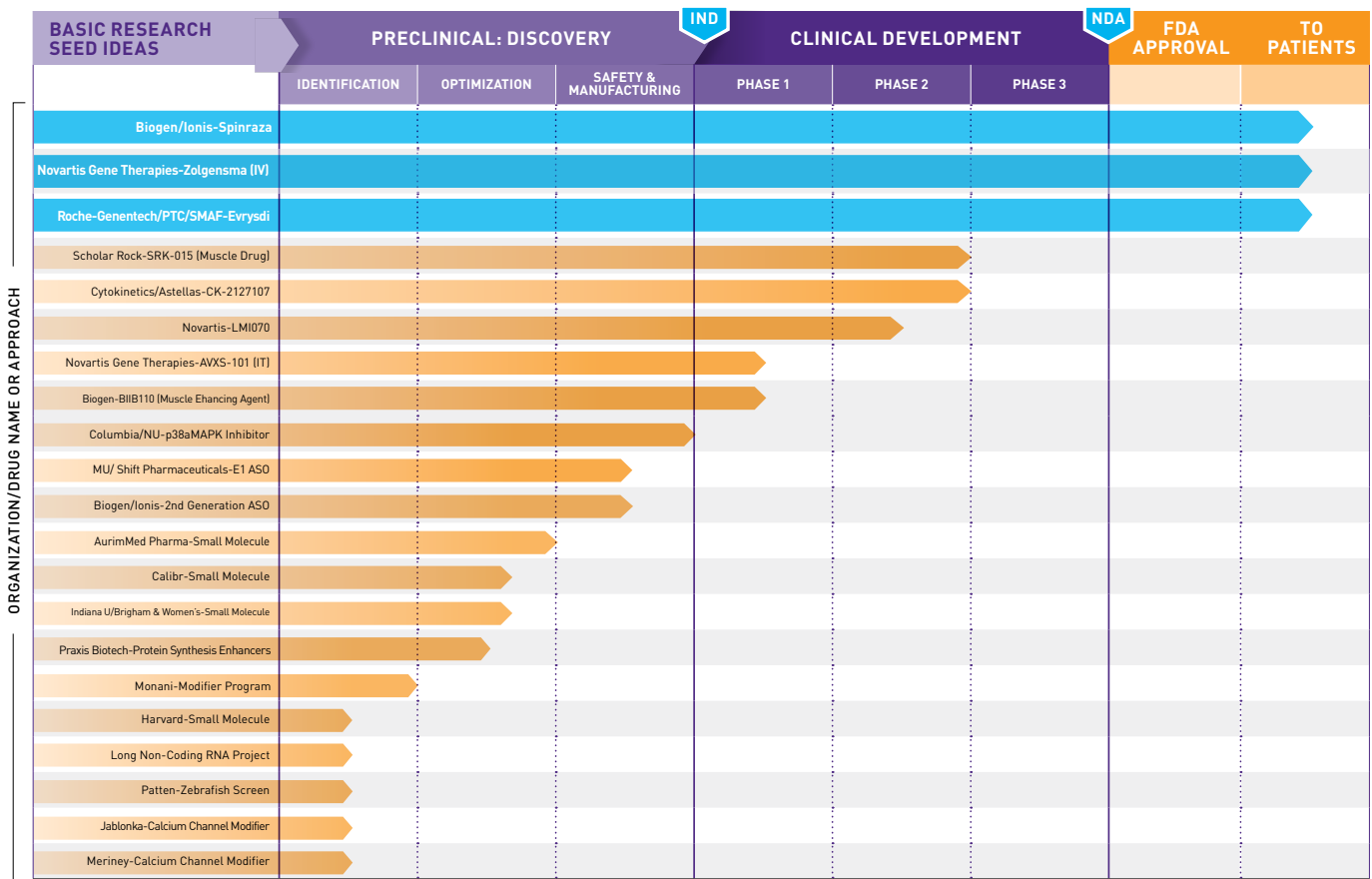
The Cure SMA Drug Pipeline is one of the primary ways we evaluate the success of our research program. It identifies the major drug programs in development and tracks their progress from basic research through U.S. Food and Drug Administration (FDA) approval and beyond. The Cure SMA Drug Pipeline identifies several possible treatment targets:

- Replacement or correction of the faulty SMN₁ gene.
- Modulation of the low functioning SMN₂ “back-up gene.”
- Muscle protection to prevent or restore the loss of muscle function in SMA.
- Neuroprotection of the motor neurons affected by loss of SMN protein.
- Newer approaches that identify additional systems and pathways affected by SMA.



SMA DRUG PIPELINE

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

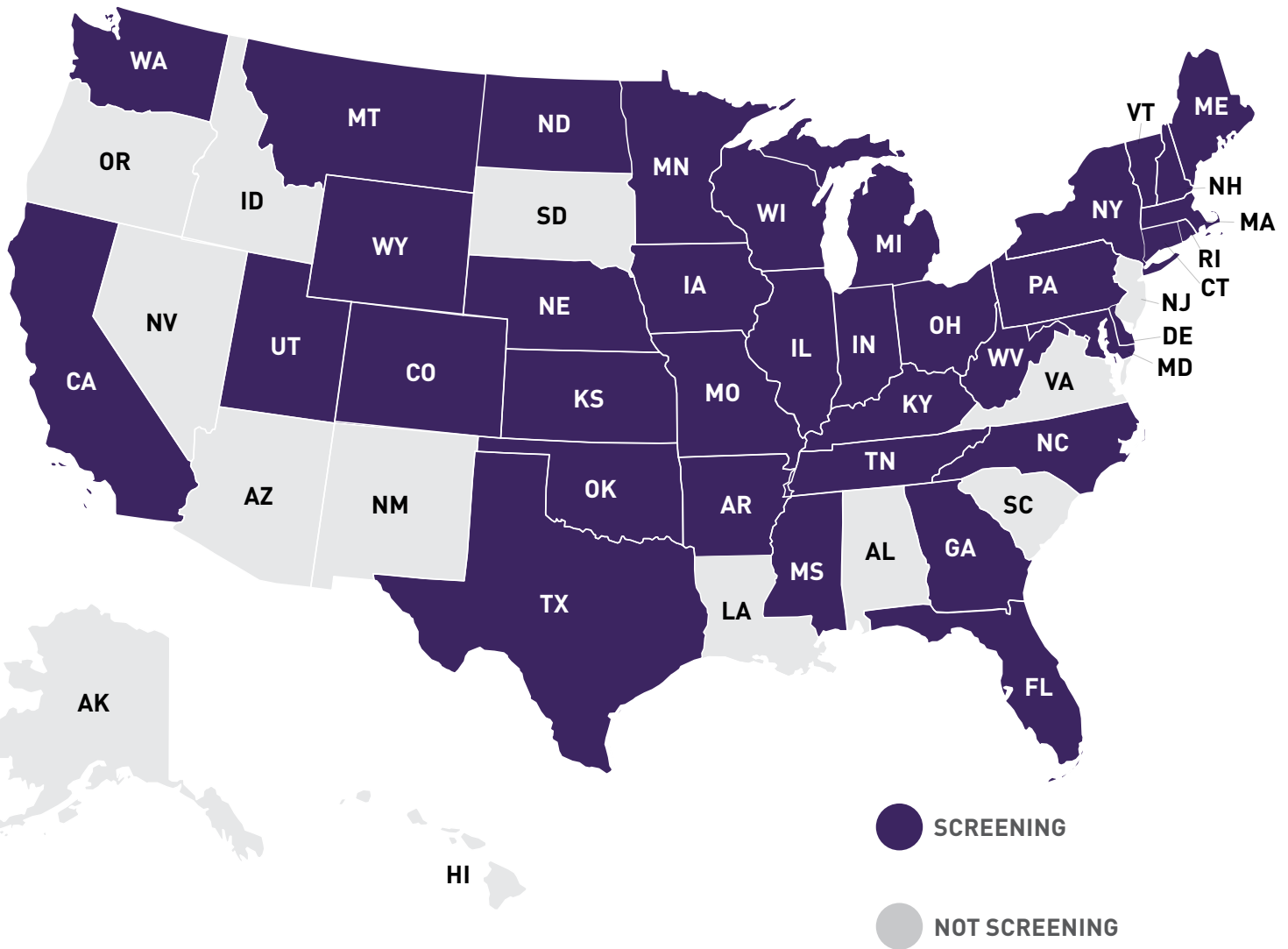


IND = Investigational New Drug NDA = New Drug Application Last updated: April 2021

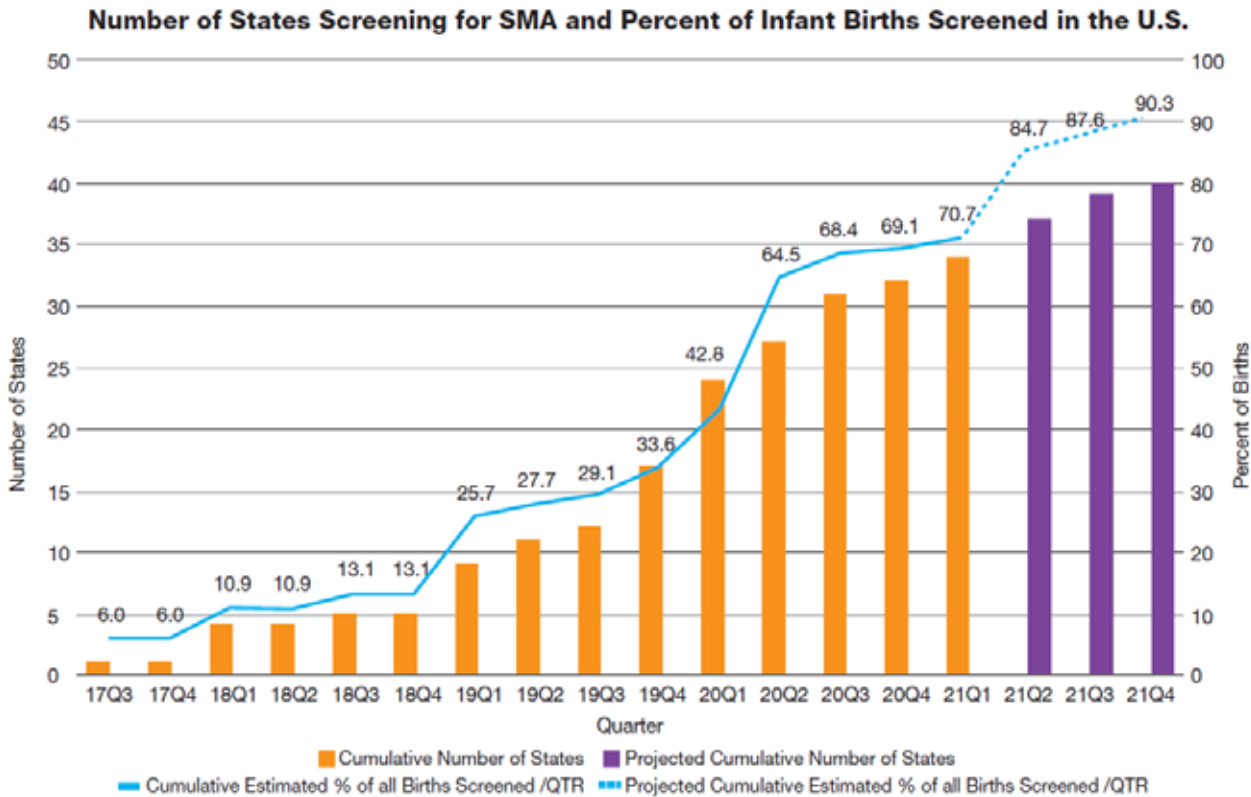
NEWBORN SCREENING UPDATE

Early diagnosis and treatment are key in ensuring the best possible outcomes for a baby with SMA. The most effective way to do this is by screening every newborn for SMA through their state's newborn screening program. The federal government recommends which conditions to screen for, while each state is expected to implement the screening of new disorders based on its local processes.

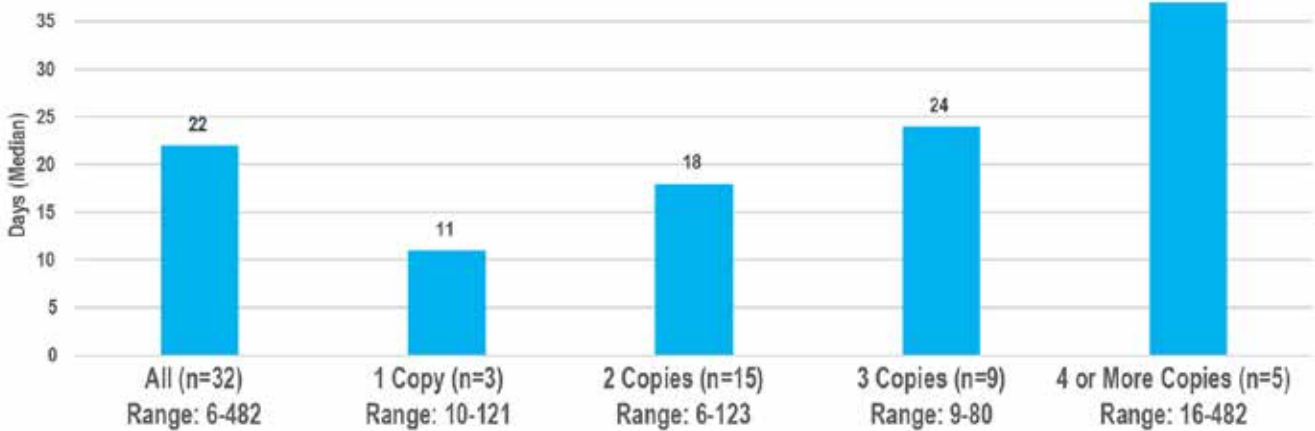
Cure SMA continues to advocate for every state to implement newborn screening for SMA, and thanks to the hard work of our families and advocates, we have made tremendous progress. In 2020, 17 new states began screening newborns for SMA, making it the most successful year to date in implementation of newborn screening for SMA. Currently, 37 states have added SMA to their newborn screening panel, meaning approx. 85 percent of all infants born in the U.S. are now screened for SMA.



NEWBORN SCREENING UPDATE (CONT.)



MEDIAN DAYS FROM DIAGNOSIS TO TREATMENT

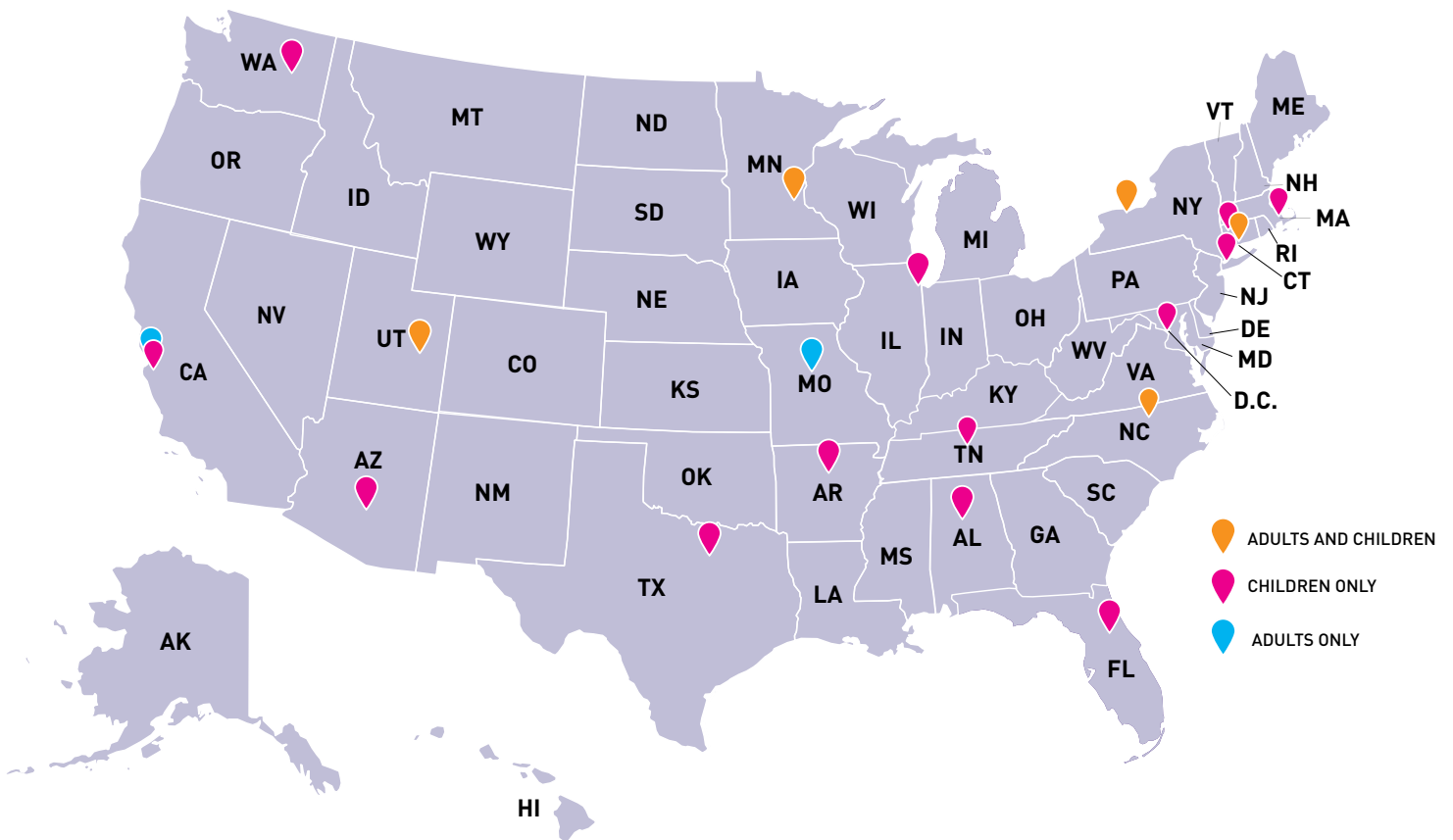


To better understand the impact of SMA newborn screening over time, Cure SMA has established an SMA Newborn Screening Registry. Families with an infant diagnosed with SMA via newborn screening, or prenatally, are encouraged to enter data in Cure SMA's Newborn Screening Registry. Families can also consent for their providers to enter data on their behalf. As of June 1, 2021, there were 40 newborns in the Registry. More information is available at www.curesma.org/newborn-screening-for-sma/ or www.curesma.org/nbsr.

CURE SMA CARE CENTER NETWORK

The Cure SMA Care Center Network is made up of 19 sites geographically dispersed throughout the U.S., representing SMA care for both children and adults. These Care Centers—a collection of neuromuscular clinics partnering with Cure SMA—provide multidisciplinary care for people with SMA and contribute consented SMA patient electronic health record data to the SMA Clinical Data Registry to achieve the following goals:

1. Quality improvement of SMA clinical care and disease management leading to creation of evidence to support a robust standard of care for SMA.
2. Standardize care across the U.S. to facilitate more rapid therapeutic development.
3. Expand clinical care center capacity to deliver new therapies to individuals with SMA, increase patient access to new treatments, and increase the number of sites for SMA clinical trials.
4. Resource for local patient services and family support and regional healthcare providers.



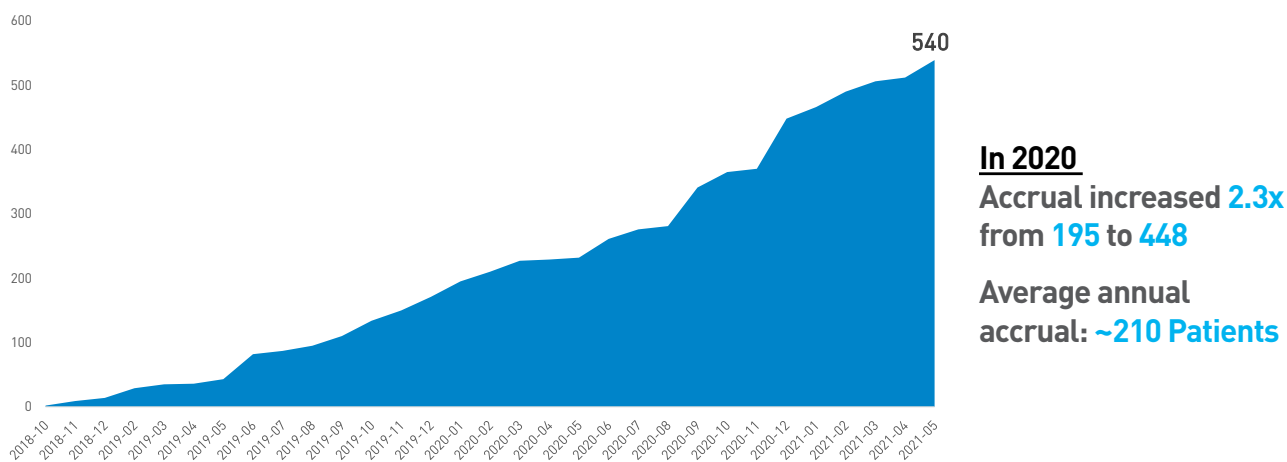
- Advocate Children’s Hospital Park Ridge, IL
- Arkansas Children’s Hospital Little Rock, AR
- Boston Children’s Hospital Boston, MA
- Children’s of Alabama Birmingham, AL
- Children’s National Medical Center Washington, DC
- Columbia University New York, NY
- Connecticut Children’s Medical Center Hartford, CT
- Duke University Medical Center Durham, NC
- Gillette Children’s Specialty Healthcare St. Paul, MN
- Phoenix Children’s Hospital Phoenix AZ
- Stanford Health Palo Alto, CA
- Stanford Children’s Health Palo Alto, CA
- Seattle Children’s Hospital Seattle, WA
- University of Missouri Health Care Columbia, MO
- University of Rochester Medical Center Rochester, NY
- University of Utah, Utah Program for Inherited Neuromuscular Disorders Salt Lake City, UT
- University of Texas Southwestern/ Children’s Health Dallas, TX
- Vanderbilt University Medical Center Nashville, TN
- Yale Pediatric Neuromuscular Disorder Clinic New Haven, CT

SMA CLINICAL DATA REGISTRY

The core of the Cure SMA Care Center Network is the SMA Clinical Data Registry. Clinical information from these sites is integrated into the SMA Clinical Data Registry, offering real-world evidence that will be used to guide best care and create evidence-based standards of care for SMA. The Registry continues to grow in partnership with the Cure SMA Care Center Network, with more than 540 patients reporting data to date.

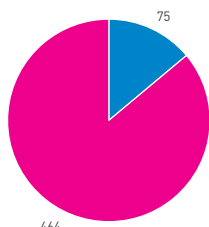
Growth of the SMA Clinical Data Registry, as well as the Cure SMA Care Center Network, will be supported by Cure SMA's recently launched Real-World Evidence Collaboration. The primary goal of the RWEC is to improve quality, and increase volume, of real-world evidence data collection and lead efforts to raise the standard of care for SMA. Activities for 2021 include:

- 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.



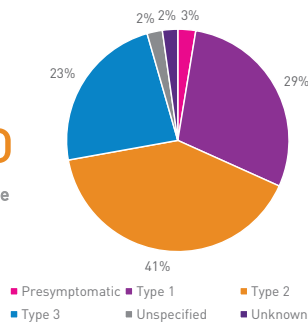
86%

of CDR patients have
a **REPORTED SMA Treatment**



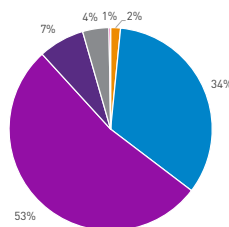
41%

of CDR patients have
SMA Type 2



53%

of CDR patients have **3 copies of the SMN2 gene (N=382)**



SMART MOVES CAMPAIGN

Developmental delays can be early signs of a serious medical condition. With SMA, early diagnosis and early treatment is key. SMARt Moves is an education campaign geared toward new parents and healthcare providers to help them recognize delayed milestones and to bridge the gap to diagnosis for those infants for whom SMA newborn screening is not available. The program emphasizes the early warning signs of SMA and provides demonstration videos for healthcare providers and families, as well as a checklist and a diagnostic toolkit.

Central to the SMARt Moves effort is an easy-to-use website that encourages parents to trust their instincts if they suspect a motor delay, because missed milestones may be a sign of a serious medical condition like SMA. On the site, parents improve their understanding of the early signs of motor delays, watch instructional videos, and download a helpful checklist to share with their doctor and help address their concerns.

The website also includes a dedicated page for healthcare providers, which details current diagnostic criteria, educational resources, and the latest treatment options and protocols. Available resources for healthcare providers include:



SMA Diagnostic Toolkit: Summarizes clinical trial data supporting early treatment, provides a table of clinical signs and symptoms by SMA type, and features a list of disorders to consider in the differential diagnosis of SMA.



SMA 1-Page Quick Reference Guide: An abbreviated version of the SMA Diagnostic Toolkit.



Know the Warning Signs Video: A series of videos that break down some of the hallmark symptoms of SMA.



SMA CME HubSpot: Free and accredited SMA related activities for healthcare professionals.



EARLY ACTION, EARLY TREATMENT, SAVES LIVES.

More information about the SMARt Moves campaign can be found at www.curesma.org/smartmoves/.

CURE SMA INDUSTRY COLLABORATION

No single group can research and develop a treatment for SMA alone. One of the most distinctive aspects of Cure SMA's research program is the SMA Industry Collaboration. Established in 2016, the SMA Industry Collaboration is a multi-faceted partnership that brings together pharmaceutical companies, Cure SMA, and other nonprofit organizations, to share information, ideas, and data. The SMA Industry Collaboration works together to address scientific, clinical, and regulatory topics that are critical to advancing drug development in SMA and will benefit the broader SMA community.

Through the SMA Industry Collaboration, we fund research to ensure that effective, safe treatments can progress through clinical trials quickly and gain approval from the U.S. Food and Drug Administration (FDA) and international regulators. Our research also ensures these treatments address 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 Collaboration consists of four topic groups:

- Regulatory interaction and outcome measure development.
- Education and awareness.
- Clinical trials.
- Patient reported data project.

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





THIS MOMENT COMES FROM WITHIN

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

SPINRAZA IS APPROVED FOR CHILDREN AND ADULTS WITH SMA

INDICATION

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

IMPORTANT SAFETY INFORMATION

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

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

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

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

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

Please see full [Prescribing Information](#).

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



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225 Binney Street, Cambridge, MA 02142

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FROM **3 days*** TO **80 years old^{†‡}**

there's someone from almost every age group
who has taken SPINRAZA

*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 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.

Attend our SPINRAZA Symposia to learn about the latest
independent, observational studies in adults with SMA

JUNE 8TH 2:00 - 3:00 PM CDT

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

Disclaimer: The acceptance of Exhibitors and Sponsors does not constitute or imply endorsement by Cure SMA of any company, product or service. Cure SMA accepts no responsibility for any claims made by any outside party.



Evrysdi in action

Over 1,300 people in the US are taking Evrysdi since approval*

- Join our panel of community members on **Monday, June 7, from 2 PM – 3 PM Central Time.**
- You'll hear community members discuss living with SMA, different ways they take action to manage SMA every day, and their experiences with Evrysdi.

*Number of people taking Evrysdi since August 2020 (approval) through April 2021.

What is Evrysdi?

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

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

Important Safety Information

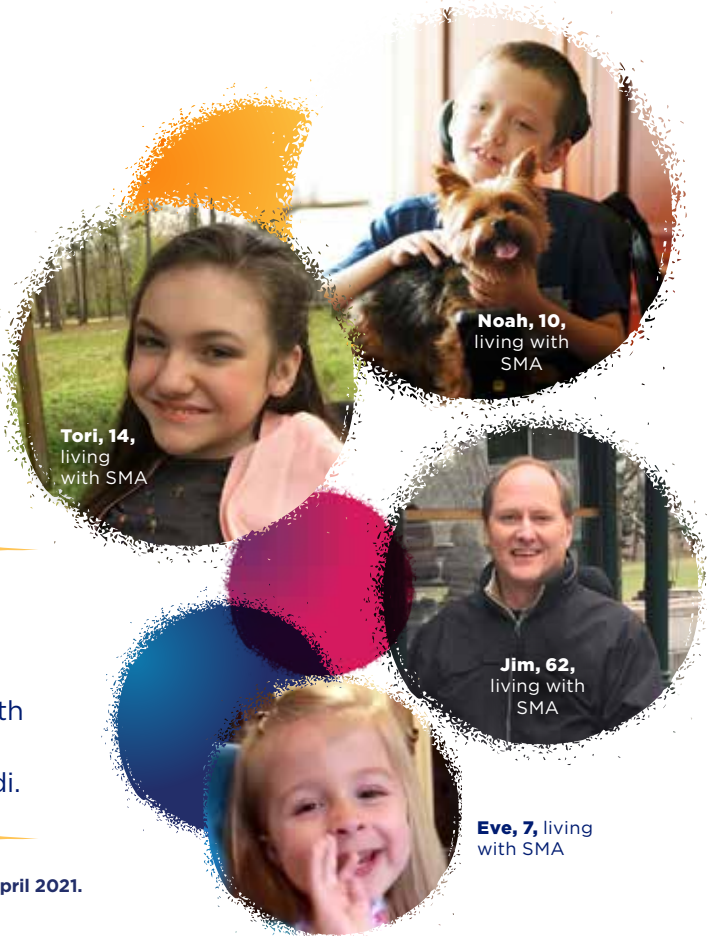
- Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:
 - are pregnant or plan to become pregnant. If you are pregnant, or are planning to become pregnant, ask your healthcare provider for advice before taking this medicine. Evrysdi may harm your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

- **Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine
- You should receive Evrysdi from the pharmacy as a liquid that can be given by mouth or through a feeding tube. The liquid solution is prepared by your pharmacist. If the medicine in the bottle is a powder, **do not use it.** Contact your pharmacist for a replacement
- Avoid getting Evrysdi on your skin or in your eyes. If Evrysdi gets on your skin, wash the area with soap and water. If Evrysdi gets in your eyes, rinse your eyes with water
- The most common side effects of Evrysdi include:
 - For later-onset SMA: fever, diarrhea, rash
 - For infantile-onset SMA: fever, diarrhea, rash, runny nose, sneezing, sore throat, and cough (upper respiratory infection), lung infection, constipation, vomiting

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

You may report side effects to the FDA at **1-800-FDA-1088** or www.fda.gov/medwatch. You may also report side effects to Genentech at **1-888-835-2555**.

Please see accompanying brief summary for additional Important Safety Information.



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

What is EVRYSDI?

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

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

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

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

How should I take EVRYSDI?

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

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

Taking EVRYSDI

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

Reusable Oral Syringes

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

What are the possible side effects of EVRYSDI?

The most common side effects of EVRYSDI include:

- **For later-onset SMA:**
 - fever
 - diarrhea
 - rash
- **For infantile-onset SMA:**
 - fever
 - runny nose, sneezing, sore throat, and cough (upper respiratory infection)
 - 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 registered trademark of Genentech, Inc.
 M-US-00007143(v2.0)
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 All rights reserved.

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

This Patient Information has been approved by the U.S. Food and Drug Administration.

Approved: 4/2021

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Join us in raising our voices for SMA

Wednesday, June 9, from 2:00 to 3:00 PM CT



Emily, mother of Ellie
Diagnosed in utero



Victoria, mother of Olivia and Jaxon
Diagnosed in utero



Mike, father of Aaron
Diagnosed through newborn screening

While every case of spinal muscular atrophy (SMA) is unique, caregivers still share one powerful resource: **each other.**

We are excited to bring together 3 SMA caregivers for our first virtual **Caregiver Roundtable Discussion**, covering topics like:

- Prenatal testing and newborn screening for SMA
- The importance of early treatment
- Feeling empowered to advocate for your child
- Why they chose ZOLGENSMA® (onasemnogene abeparvovec-xioi)

Visit **ZOLGENSMA.com** to watch inspiring family videos.

Indication and Important Safety Information

What is ZOLGENSMA?

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

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

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

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

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

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

What do I need to know about vaccinations and ZOLGENSMA?

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

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

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

What are the possible or likely side effects of ZOLGENSMA?

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

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

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

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



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IMPORTANT FACTS ABOUT ZOLGENSMA® (onasemnogene abeparvovec-xioi)

USE

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

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

WARNINGS

Acute Serious Liver Injury and Elevated Liver Enzymes

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

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

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

OTHER IMPORTANT INFORMATION

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

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

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

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

COMMON SIDE EFFECTS

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

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

QUESTIONS?

To learn more, talk to your doctor and visit www.ZOLGENSMA.com for Full Prescribing Information.

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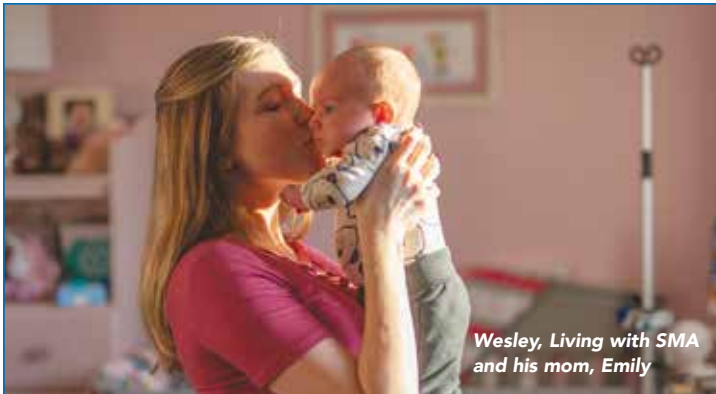
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Wesley, Living with SMA and his mom, Emily

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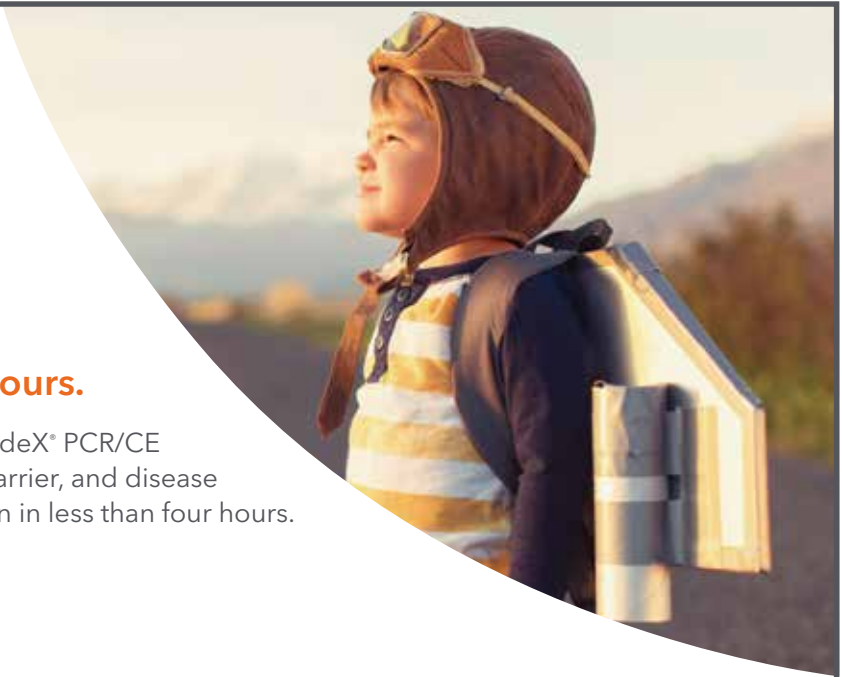
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Ahmed Ibrahim Ahmed, MD, MSc, FACOG, RDMS, RDCS, is an assistant professor of obstetrics and gynecology, maternal fetal medicine, and reproductive genetics at the University of Rochester, Rochester, NY. He graduated top of class with honor at Faculty of Medicine, Alexandria, Alexandria, Egypt. He obtained his MSc degree in reproductive science at the same university. Dr. Ahmed did his OBGYN residency training at Maimonides Medical Center, Brooklyn, NY. He also finished a combined maternal fetal medicine/medical genetics and genomics fellowship at Wayne State University, Detroit, MI.

Kristen Allison, PhD, CCC-SLP, is an assistant professor and director of the Speech Motor Impairment and Learning (SMILe) Lab at Northeastern University, Boston, MA. Her research focuses on improving assessment and treatment of motor speech disorder in children, with a particular emphasis on pediatric dysarthria.

Snigdha Alur-Gupta, MD, MSCE, is Assistant Professor of OBGYN, University of Rochester, Rochester, NY, as well is a Reproductive Endocrinologist and Infertility Specialist. Dr. Alur-Gupta completed her residency at the University of Rochester followed by fellowship at the University of Pennsylvania, Philadelphia, PA. During her fellowship, she also obtained a Master's in Clinical Epidemiology and Biostatistics. Dr. Alur-Gupta is passionate about multiple aspects of infertility care with research interests in IVF, PCOS, and disparities in care.

Vanessa Battista, DNP, MBA, MS, RN, CPNP-PC, CHPPN, is a Pediatric Nurse Practitioner (PNP) on the Neuromuscular Team at The Children's Hospital of Philadelphia. She holds a B.A. from Boston College, a B.S. and M.S. from Columbia University School of Nursing, a certificate in Pastoral Ministry from the Boston College School of Theology and Ministry, and a DNP and MBA from Johns Hopkins University. Vanessa previously served as a research coordinator, nurse, and nurse practitioner at the SMA Centers at Columbia University Medical Center and Children's Hospital Boston. She then relocated to Philadelphia to be a part of the Pediatric Advanced Care Team (PACT), prior to returning to neuromuscular care. Vanessa is a member of the Cure SMA Medical Advisory Council (MAC).

Diana Castro, MD, is a specialist in Pediatric Neuromuscular Medicine at the University of Texas Southwestern, Dallas, TX. Dr. Castro is the director of the neuromuscular division at Children's Health in Dallas and co-director of the Pediatric Muscular Dystrophy Association Clinic and Parent Project Muscular Dystrophy. Dr. Castro is also the director of the Cure SMA Care Center Network at Children's. She takes care of a large population of patients with SMA from newborns to 21 years of age. Her goals are to provide the best care possible for patients with SMA, with disease modifying therapies, standards of care, and research trials. She is very interested in education for patients and other healthcare providers nationally and internationally.

Kimberly Cook, as a special education professional, focuses on access to the curriculum for all learners. She serves educators, students, and parents through her work with Access Curriculum Together and as an adjunct instructor at Texas A&M University in Corpus Christi, TX. She holds a bachelor's in Advertising from the University of Texas in Austin, TX, and a master's in Educational Administration and Mid-Management from Texas A&M University in Corpus Christi. She worked as a member of the Texas Assistive Technology Network and the Texas Low Incidence Disability Network as a consultant with the Education Service Center, Region 2. Her diverse experience includes teaching in a general education and special education environment, teaching students with dyslexia and autism, and serving as a federal programs director. Kimberly's primary areas of interest include assistive technology, access to curriculum, and low incidence disabilities. Kimberly lives in Corpus Christi with her husband Kresten and dog, Cooper.

Tara Davenport, began the life-changing journey of learning about and practicing mindfulness meditation 10 years ago. She is passionate about sharing how her mindfulness practice has helped her. Tara is a former civil engineer who has returned to her alma mater, New Mexico State University, Las Cruces, NM, to work towards a master's degree in social work. She lives in Albuquerque, New Mexico with her husband Patrick and their four children.

Mary Anne Elhert, CFP, is a financial professional. She is also a mother, sister, and a daughter to her loved ones with disabilities. She is highly regarded as a specialist in working with families of individuals with disabilities and the elderly and speaks at conferences, hosts webinars, and runs virtual training on future planning. Mary Anne's journey began in senior management positions within the banking industry, including Deutsche Credit Corp, Heller Financial, and Citicorp. For more than 20 years she was a leader in the corporate world, leaving in 1990 to create Ehlert Financial Group and Protected Tomorrows, businesses driven to assist individuals and families to develop both short- and long-term financial strategies to help them find peace of mind. Over the years, she found that many individuals and families were receiving transactional advice, rather than overall planning, and thus she set about to become a true personal advisor to her clients. The mission to become that needed resource to her clients has driven the overall purpose and strategy of the firm. As President and Founder of Protected Tomorrows and Ehlert Financial Group, as well as a partner of Forum Financial Management, Mary Anne has served on the Board of Directors of Special Olympics Illinois, Gateway to Learning School for Special Needs, Illinois St. Coletta, as well as Vice-Chair for the National Disability Institute in Washington, DC. She currently serves on the board for the Cornelia de Lange syndrome National Foundation, Special Olympics Illinois Foundation Board, the Advisory Board of Tails for Life, and the Advisory Counsel of Integrative Touch for Kids.

Bakri Elsheikh, MBBS, FRCP, is a Professor of Neurology at the Ohio State University Wexner Medical Center. He is board-certified in Neurology, Neuromuscular Medicine, and Clinical Neurophysiology. He is the medical director of the EMG Laboratory and the director of the Neuromuscular Medicine and Clinical Neurophysiology Fellowships and the Adult SMA clinic at Ohio State University. His research focus is on the outcome measures and treatment of adults with SMA.

Julia Feinberg, is pursuing her doctorate in clinical psychology at the California School of Professional Psychology, where she specializes in improving the mental health of people with disabilities. Her dissertation is researching barriers people with SMA encounter when accessing mental health services, and what mental health services would be most valuable to add to hospital based SMA clinics. She trained at the Oakland Veteran's Administration Outpatient Clinic, the UCSF Alliance Health Project for the LGBTQ+ and HIV+ communities, and a middle and high school counseling program. Prior to beginning her doctoral program, Julia completed her MEd at Harvard in higher education administration, and her BA from Stanford in Political Science. She has worked in various fields including at a Medicaid management company and the Senate H.E.L.P Committee's Office of Disability Policy. Julia currently lives in San Francisco and grew up in the Philadelphia suburbs. She has Type 2 SMA and uses a wheelchair.

Albert Freedman, PhD, is a child and family psychologist at Freedman Counseling Associates near Philadelphia, PA, and a member of the Cure SMA's Medical Advisory Council. Dr. Freedman provides counseling support for children with special needs and their families, and serves as a consultant to healthcare organizations, rare disease advocacy groups, businesses, and schools. He has spoken and written widely on the topic of caring for children and families challenged by complex medical conditions. Al is the father of two adult children, Jack and Cara. Jack was diagnosed with SMA Type 1 in 1995.

Robert Graham, MD, a specialist in Critical Care Medicine, is the Clinical Director for Clinical Care, Anesthesiology, Perioperative Extension (CAPE) and Home Ventilation Programs at the Children's Hospital Boston in Massachusetts. He is dedicated to both the acute and long-term care of children with SMA. Through his outpatient, home visit, and educational programs, he hopes to improve the care and quality of life for individuals with SMA and their families.

Debra Guntrum MS, FNP, ANCC Certified, has a BSN from SUNY Brockport, as well as an MSN from University of Rochester School of Nursing. She is the lead nurse practitioner and clinic coordinator for the Pediatric and Adult Multidisciplinary Neuromuscular Clinic. Debra has worked in the Neuromuscular Disease Center at the University of Rochester for 21 years and cares for children and adults with neuromuscular diseases.

Becky Hurst Davis, MS, RD, CSP, CD, CNSC, is a registered dietitian working with Intermountain Health Care, Salt Lake City, UT. She graduated with her Master of Science in Nutrition from the University of Utah. Becky is a certified specialist in pediatric nutrition, as well as a certified nutrition support clinician. She is passionate about providing the best nutrition care for individuals with neuromuscular diseases and has been involved with Cure SMA for over 12 years.

Richard M. Kravitz, MD, is a Professor of Pediatrics in the Division of Pediatric Pulmonary and Sleep Medicine at the Duke University Medical Center, Durham, NC. He has a long-standing interest in caring for patients with neuromuscular weakness and helps to advocate for their needs on a local, state, and national level. He is co-director of the Duke Comprehensive Neuromuscular Clinic, where he provides pulmonary care to children and adults with neuromuscular weakness in a multi-disciplinary setting. He has co-authored several position papers on the management of the respiratory complications of patients with neuromuscular weakness, as well as spoken nationally and internationally on these topics. He is a member of the Cure SMA Medical Advisory Council.

Oren Kupfer, MD, is a Pediatric Pulmonologist and Inpatient Medical Director at the Children's Hospital Colorado Breathing Institute and Associate Professor of Pediatrics at the University of Colorado School of Medicine. His passion is the respiratory care of children with SMA and other neuromuscular disorders, developing evidence-based clinical care guidelines for respiratory illnesses and post-operative care, and improving quality of life and survival in SMA. He is a member of the Cure SMA Medical Advisory Council.

Tina Lewis, joined the Cure SMA community in 2007 after her son, Julian, was diagnosed with SMA at the age of 3 years. Immediately after her son's diagnosis, Tina became a parent advocate for her son and other children with SMA. Her continuous advocacy at the preschool, elementary, middle, and now high school levels has ensured a positive impact on her son's educational success. Educating Julian's team of physical therapists, occupational therapists, physical education teachers, classroom teachers, and administrators about his physical needs has been essential in ensuring equitable access to the learning environment. At the broader level, Tina has supported families through Cure SMA, as they work to ensure the same experiences for their own children. For the past 24 years, Tina has been an educator in the state of Maryland. As an elementary classroom teacher and reading support teacher, she has a broad range of experiences working with families and students who have IEP's. Her firsthand experiences as an educator have made a direct impact on her son's progress in school. It is her passion to ensure that all children are provided an environment that maximizes their potential for learning.

Jennifer Martyn, PT, studied physical therapy at the University of Washington, graduating in 1995. Her first job at Mary Bridge Children's Hospital in Tacoma, WA plunged her into aquatics, doing a group physical therapy program for children with a variety of diagnoses. Additionally, she provided clinical-based physical therapy and staffed the regional Muscular Dystrophy Association clinic. In 2001, she began Wave Therapies, where she works with both adults and children in a warm water environment with a focus on improving strength and range of motion and improving functional skills. When not in the water, Jennifer loves being with her family, biking, kayaking, gardening, and sewing.

Katlyn McGrattan, MD, is an Assistant Professor in the Department of Speech-Language-Hearing Science at the University of Minnesota, with a clinical appointment at Masonic Children's Hospital. She completed doctoral training in Health & Rehabilitation Science at the Medical University of South Carolina, and post-doctoral training in Neonatal Gastroenterology at Nationwide Children's Hospital and Pediatric Otolaryngology at Medical University of South Carolina. Her research focuses on the use of refined physiologic assessment to identify impairments in neonatal upper aerodigestive physiology and apply targeted therapeutic interventions to maximize treatment effect.

Diane Murrell, LCSW, is a licensed clinical social worker in the neurology division at Texas Children's Hospital, Houston, TX. She works in the Blue Bird Circle Clinic with families who have children with a chronic illness or disability. She is the dedicated social worker for the neuro-muscular clinic and serves as their transition coordinator. Diane sits on the hospital's clinical ethics committee. Her work in qualitative research includes autism, SMA, transition, and the psychosocial impact of diagnosis related loneliness for a parent and/or child. Diane founded and manages the Houston Fireballs, the first power soccer program in Texas. The soccer program seeks to provide an athletic and team experience for those in power wheelchairs (www.houstonfireballs.com).

Leslie Nelson, PT, PhD, OCS, is an Assistant Professor in the Department of Physical Therapy at University of Texas Southwestern, Dallas, TX. She has a broad background in physical therapy with a focus over the past 13 years on infants and children with neuromuscular disorders and muscle diseases. In addition to her clinical expertise, she has been involved in both investigator-initiated grants and industry sponsored clinical trials for studies involving neuromuscular disorders. Leslie has served as a mentor in the Cure SMA Therapist Mentoring Program. She also serves on the Cure SMA Medical Advisory Committee.

Julie Parsons, MD, did her medical training at the University of Colorado. She is board certified in child neurology. Dr. Parsons was in private practice from 1993 to 2000, then joined the faculty at University of Colorado School of Medicine, where she is a Professor of Pediatrics and Neurology. Dr. Parsons was named the inaugural Haberfeld Family Endowed Chair in Pediatric Neuromuscular Disorders. She is principal investigator on several clinical trials for muscular dystrophy and SMA. Dr. Parsons is Co-Director of the multidisciplinary Neuromuscular clinic at Children's Hospital Colorado.

Samuel Rosenfeld, MD, is the director of the Neuromuscular Clinics at CHOC Children's Hospital. He is a graduate of the Pennsylvania State University School of Medicine. Dr. Rosenfeld completed his Orthopaedic Surgery Residency at the University of California, Irvine and Fellowship in Children's Orthopaedics and Rehabilitation at Rancho Los Amigos National Rehabilitation Center. Dr. Rosenfeld is Board Certified by the American Board of Orthopaedic Surgery and is a Fellow of the American Academy of Orthopaedic Surgery, Fellow of the American Academy of Pediatrics, and Fellow of the American Orthopaedic Association. Dr. Rosenfeld has been on staff at CHOC Children's Hospital and clinical faculty at the University of California, Irvine since 1983. Dr. Rosenfeld is on the Professional Advisory Committee of the Spina Bifida Association, as well as the Medical Advisory Committee for Cure SMA. Dr. Rosenfeld is a member of the Pediatric Orthopaedic Society of North America, American Academy of Pediatrics, American Orthopaedic Association, AACPD, ACPOC, COA, WOA, CMA, and Orthopaedic Rehabilitation Association.

Richard M. Rubenstein, Esq., is an attorney and financial advisor, and a father of a child with special needs who suffered from SMA. He is well versed and has a heartfelt commitment to assist families who require special needs planning. Richard concentrates his legal practice in estate planning and elder law. At Strategies for Wealth, Richard works to develop long-term relationships with his clients and base them on integrity and commitment to them, their families, friends, and businesses. He begins by coordinating and integrating all aspects of their personal and business finances. His role becomes that of a trusted attorney and financial advisor, providing professional counsel to afford his clients with the financial confidence necessary to maximize efficiency and effectiveness in achieving great success, wealth, and peace of mind. Richard is a Member of the American Bar Association; National Society of Financial Services Professionals; National Association of Insurance and Financial Advisors; Past Chairman, National Board of Directors, Cure SMA; Greater New York Chapter, Cure SMA; Town of Yorktown, Group Home Committee; and Past Chairman, Town of Yorktown, Board of Ethics. He currently resides in Yorktown Heights, NY, with his wife, Michele, 17-year-old daughter, Emma, and dogs, Rippley and Sophie. He makes this presentation in loving memory of Max, "my special boy" (April 24, 2005–February 8, 2009).

Gina Santucci, MSN FNP APRN-BC, is a nurse practitioner in pediatric palliative care. She has co-authored several articles and chapters including Comprehensive Pediatric Hospital Medicine (2017), the Oxford Textbook of Advance Practice Nursing (2015) and Nursing Ethics and Advance Practice (2013). She is the editor of the Core Curriculum for Pediatric Palliative Care Nurse 1st and 2nd editions. Ms. Santucci has lectured nationally and internationally on pediatric palliative care and continues to provide education to clinicians.

Peter Schochet, MD, is a board-certified pediatric pulmonologist who has been dedicated to the respiratory management of children with neuromuscular disease. He is a Clinical Assistant Professor of Pediatrics at University of Texas Southwestern, Dallas, TX. He has been an active member of the neuromuscular team at Children's Health Medical Center Dallas since 1996. He is actively involved in the care of SMA patients undergoing scoliosis surgery at Texas Scottish Rite Hospital for Children. He is committed to non-invasive ventilation and management of patients with neuromuscular weakness. He has lectured on the medical management of patients with SMA. He prefers a collaborative style of medical management with open communication with team members, patients, and their families.

Brian D. Snyder, MD, PhD, is a Maurice Mueller Professor of Orthopaedic Surgery, Harvard Medical School and Research Professor of Bioengineering at Boston University, Department of Bioengineering. An attending orthopaedic surgeon at Boston Children's Hospital, his clinical practice focuses on congenital and acquired deformities about the hip and spine related to neuromuscular conditions as well as pediatric trauma. As a clinician-scientist funded by NIH, DoD, private foundations, and industry, his translational research focuses on improving the practice of orthopaedic surgery by applying engineering principles to solve clinical problems. In recognition for his translational research, Dr. Snyder received a Kappa Delta Award from the American Academy of Orthopaedic Surgeons, the Russell Hibbs Award from the Scoliosis Research Society, and the A. Clifford Barger Excellence in Mentoring Award from Harvard Medical School. Dr. Snyder is member of the board for Cure SMA.

Harvey J. Stern, MD PhD, FACMG, FAAP, is currently the Director of Reproductive Genetics and the Preimplantation Genetic Diagnosis program at the Genetics & IVF Institute, Fairfax, VA. He is board certified in Medical Genetics and Pediatrics and has subspecialty certification in clinical, biochemical, and molecular genetics. Dr. Stern has participated in the Annual SMA Conference for more than 10 years.

Sarah Stoney, MSW, LSW, graduated from West Chester University with a Master of Social Work in 2014, after she received her bachelor's degree in the same discipline in 2006. She joined The Children's Hospital of Philadelphia's (CHOP) Division of Neurology in 2014. Previously, she worked in the emergency department of Penn Medicine's Chester County Hospital and as a family counselor at The Lincoln Center for Family and Youth. At CHOP, she assists patients and their families as they navigate the complex medical system, advocates with families in their schools and throughout the community, and provides ongoing support to patients and families in the neuromuscular, leukodystrophy, and multiple sclerosis communities.

Anne Stratton, MD, is a pediatric physiatrist (Rehabilitation doctor) at Children's Hospital Colorado. She is originally from Ohio and received her M.D. from the University of Cincinnati. She then completed dual residencies in Pediatrics and Physical Medicine and Rehabilitation at the University of Colorado, followed by a Pediatric Rehabilitation Medicine Fellowship. She has enjoyed being one of the pediatric physiatrists actively involved in the multidisciplinary Neuromuscular Clinic at Children's Hospital since 2010. She joined the Cure SMA Medical Advisory Committee in 2015. Clinically, she feels it is important to consider the whole patient, their lifestyle, and aspirations when making medical recommendations. She is also the mother of two young girls and enjoys doing some running in her free time.

Stacey Tarrant, BS, RD, LDN, is a clinical nutrition specialist at Boston Children's Hospital, Boston, MA. She has been providing medical nutrition therapy and nutrition counseling to individuals with SMA for the past 13 years within Boston Children's Hospital multidisciplinary SMA clinic. Her other specialty areas include dietary therapy for children with inborn errors of metabolism, including fatty acid oxidation disorders, and the ketogenic diet for children with intractable epilepsy. For the past 6 years, she has been an active member of the Cure SMA Medical Advisory Council.

Jane B. Taylor, MD, MsCR, is a Pediatric Pulmonologist in the Division of Pulmonary and Sleep Medicine at UPMC – Children's Hospital of Pittsburgh (CHP). She had previously been the director of pulmonary neuromuscular clinic at Children's Mercy Kansas City for 10 years and moved to Pittsburgh in 2019. She is now the pulmonologist in the multidisciplinary neuromuscular clinic at CHP and has started the CHP Family Medical Advocacy and Advisory Board, is working on neuromuscular pulmonary clinical care guidelines for CHP, and continues to be actively involved in pulmonary research. Dr. Taylor is a board member for the local American Lung Association, advocating for pediatric lung health on the local, state, and national levels. She is also the Pediatric Web Director for the American Thoracic Society and incorporates neuromuscular topics into the curriculum. She is a member of the Cure SMA Medical Advisory Council.

Laura Watne, MS RD CSP, is a Registered Dietitian and Board-Certified Specialist in Pediatric Nutrition. She joined the Children's Hospital Colorado multidisciplinary Neuromuscular Clinic team in 2011. Laura specializes in providing medical nutrition therapy for infants, children, and adults with SMA and a variety of other neuromuscular disorders. Laura has a passion for helping patients with SMA thrive and improve their quality of life through evidence-based nutrition plans. Her other areas of expertise include medical nutrition therapy for patients with spina bifida, spinal cord injury, Rett syndrome, as well as young athletes. She is excited to be a member of the Cure SMA Medical Advisory Council.

Angela Wrigglesworth, a third-grade teacher from Houston, TX holds an undergraduate degree from Texas A&M University and a master's degree in special education from the University of St. Thomas. She is the founder of the Ms. Wheelchair Texas Foundation, was a 16-year member of the National Task Force on Public Awareness through the Muscular Dystrophy Association and sits on the advisory board of the Camp for All Foundation. Angela enjoys speaking about living with SMA Type 2 and sharing John Wooden's philosophy that, "Things turn out best for those who make the best of the way things turn out."

Thank you to each and every speaker who helped create and adapt the conference to its virtual platform this year. These individuals volunteer their time to help educate the community on the latest information related to SMA. Thank you for your time and expertise for this year's Virtual SMA Conference!



GET INVOLVED WITH YOUR LOCAL CHAPTER

Cure SMA has 36 volunteer chapters throughout the United States.

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



VOLUNTEER OPPORTUNITIES ARE AVAILABLE NATIONALLY

Please contact fundraising@curesma.org for more information.

Visit www.curesma.org/chapters.

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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!

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President, Cure SMA**

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Medical Advisory Council (MAC)



Mary Schroth MD

Mary is the Chief Medical Officer for Cure SMA. She brings 25 years of experience as a Pediatric Pulmonologist to Cure SMA and is Professor Emeritus at the University of Wisconsin School of Medicine and Public Health. As a specialist in SMA respiratory care and an educator, Dr. Schroth is a leader in the SMA community and with Cure SMA.



Alison Ballard, RN, MSN

Pediatric Nurse Practitioner and Neuromuscular Care Coordinator at Children's Hospital Colorado in Denver, CO.



Vanessa Battista, RN, MS, CPNP – Palliative Care and Quality of Life Subcommittee Chair

Palliative Care Pediatric Nurse Practitioner at Children's Hospital of Philadelphia in Philadelphia, PA.



William Bell, BS Pharm, MBA, MSCC, RPh

Director of Clinical Client Services in Chalfont, PA.



John Brandsema, MD

specializes in Pediatric Neuromuscular Neurology at Children's Hospital of Philadelphia, associated with the Perelman School of Medicine at The University of Pennsylvania in Philadelphia, PA.



Jin Yun (Helen) Chen, MS, CGC

Genetic Counselor at Massachusetts General Hospital in Boston, MA.



Thomas Crawford, MD

specializes in Pediatric Neurology at Johns Hopkins Hospital, associated with Johns Hopkins University in Baltimore, MD.



Tina Duong, MPT, PhD

Physical Therapist at Stanford University in Stanford, CA.



Bakri Elsheikh, MBBS, FRCP

specializes in Neurology, Neuromuscular Medicine, and Clinical Neurophysiology at The Ohio State University Wexner Medical Center in Columbus, OH.



Albert Freedman, PhD

specializes in Psychology in independent practice in Philadelphia, PA.



Melissa Gibbons, MS, CGC

Genetic Counselor at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



Jennifer Hubbell, OTR/L

Occupational Therapist at Cincinnati Children's Hospital Medical Center in Cincinnati, OH.



Becky Hurst Davis MS, RD, CSP, CD

Pediatric Nutritionist at Intermountain Healthcare in Salt Lake City, UT.



Chamindra Konersman, MD

Neurologist at Rady Children's Hospital, associated with the University of California San Diego in San Diego, CA.



Richard M. Kravitz, MD

Pediatric Pulmonologist and Sleep Medicine Physician at Duke University in Durham, NC.



Kristin J. Krosschell, PT, DPT, MA, PCS

Pediatric Physical Therapist at Northwestern University in Chicago, IL.



Oren Kupfer, MD

Pediatric Pulmonologist at Children's Hospital Colorado, associated with the University Of Colorado School Of Medicine in Denver, CO.



Khalida Liaquat, MS, LCGC

Genetic Counselor at Athena Diagnostics in Marlborough, MA.



Diane Murrell, LCSW – Care Coordination, Case Management, & Nursing Subcommittee Chair

Social Worker at Texas Children's Hospital in Houston, TX.



Leslie Nelson, PT, PhDc, OCS

Pediatric Neuromuscular Physical Therapist at Children's Health, associated with the University of Texas Southwestern in Dallas, TX.



Julie Parsons, MD – Neurology Subcommittee Chair

Pediatric Neurologist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



Silvana Ribaldo, MD – Genetics, OB/GYN, & Family Planning Subcommittee Chair
Obstetrician and Gynecologist at Columbia University in New York City, NY.



Samuel Rosenfeld, MD
Orthopedic Surgeon at CHOC Children's Hospital in Orange, CA.



Peter Schochet, MD
Pediatric Pulmonologist at Children's Health, associated with the University of Texas Southwestern in Dallas, TX.



Perry Shieh, MD, PhD – Adult Care Subcommittee Chair
Neurologist at UCLA Medical Center in Los Angeles, CA.



Edward C. Smith, MD
Pediatric Neurologist at Duke University in Durham, NC.



Anne Stratton, MD
Pediatric Physiatrist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.



Stacey Tarrant, BS, RD, LDN
Nutritionist at Boston Children's Hospital in Boston, MA.



Jane B. Taylor, MD, MsCR
Pediatric Pulmonologist at the UPMC Children's Hospital of Pittsburgh, associated with the University of Pittsburgh School of Medicine in Pittsburgh, PA.



Fred W. Troutman, PhD, RN
Nurse Educator, Professor Emeritus at Walla Walla University in Portland, OR.



Laura Watne, MS RD CSP
Nutritionist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.

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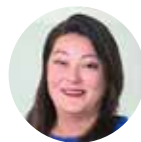


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Chien-Ping Ko, PhD, Professor, University of Southern California, SMA Biology/Neurobiology



Michael Vellard, PhD, Vice President of Research, Ultragenyx Pharmaceutical, Preclinical Drug Development



Thank You DR. ARTHUR BURGHEES

On the 25th anniversary of the SMA Researcher Meeting, we would like to share our gratitude to Dr. Arthur Burghes. He was instrumental in launching this important meeting in its early years and remains committed to ensuring its success year-after-year. Moreover, his long-term support of Cure SMA, as well as his enduring and seminal scientific contributions to SMA, have helped lead the way in our understanding and treatment of SMA.

~ Dr. Jill Jarecki, Chief Scientific Officer, Cure SMA

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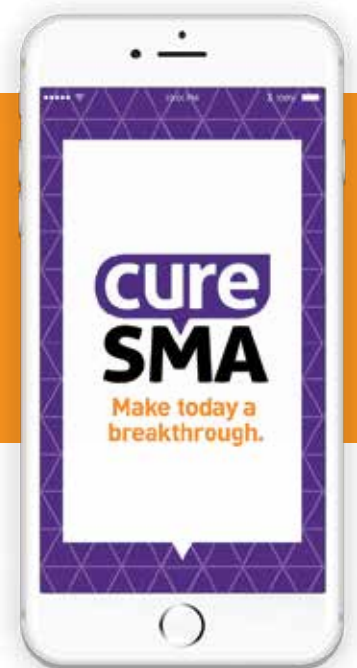


www.linkedin.com/company/families-of-sma



And don't forget about our SMA community support app.

This gives you instant access to Cure SMA's useful tools and resources, as well as information related to SMA care and support for when you are on-the-go. The app is available to download from your Apple or Android device.



Cure SMA would like to thank Genentech for generously funding a grant to support the Cure SMA app resource for the SMA community!

THANK YOU!



SEE YOU ALL NEXT YEAR

**cure
SMA**