2020 VIRTUAL SMA CONFERENCE

JUNE 8-12, 2020

UNITED, AT HOME

#VIRTUALSMACONFERENCE

PROGRAM GUIDE

cure SMA

Make today a breakthrough.

Dear Families,

During this week of our 2020 Virtual SMA Conference, we are not going to focus on the current COVID-19 situation. Except to recognize how the strength and support of our community has done such a tremendous job in keeping all of us safe and healthy.

Even during this pandemic, we have been able to rapidly provide emergency support programs to thousands of families, while keeping our funding for core programs moving forward. This includes new basic research grants that we recently awarded and the expansion of our Cure SMA Care Center Network to 20 sites. Now, we are also extending our funding towards activities that will provide increased support for clinical research and trials.

Our 2020 Virtual SMA Conference will focus on the continued progress we have made, and the new milestones we are targeting to hit next. During 2020, our goals are to cross over 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 real-world access to treatments, research and 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. One of our most important initiatives in this area is the continued expansion of the SMA Care Center Network and corresponding SMA Clinical Data Registry to improve care and results, as well as increase access to new therapies. We are also increasingly shifting our programs to provide more customized and localized support.

This conference is supported by our generous sponsors. Our special thanks to AveXis, Biogen, and Genentech as the Presenting Sponsors of the 2020 Annual SMA Conference. We would also like to recognize our Visionary Sponsor, Ionis; our Platinum Sponsor, Scholar Rock; and our Silver Sponsors, Accredo and Permobil, for their support.

During the 2020 Virtual Research & Clinical Care Meeting, researchers from industry and academia will meet to create open communication and collaboration, accelerating the pace of research. Multidisciplinary clinicians will share knowledge, ask questions, and network to optimize care and grow our SMA community of healthcare providers.

We thank you for joining us to stay safe and healthy, and for supporting programs that achieve our SMA community's goals

Sincerely,

Kenneth Hobby
President

Colleen McCarthy O'Toole
Vice President, Events
& Family Support

Jill Jarecki, PhD
Chief Scientific Officer

Mary Schroth MD
Chief Medical Officer

JOIN US!

FOR AN END-OF-CONFERENCE CELEBRATION

To cap off the 2020 Virtual SMA Conference week, we want to see the full SMA community come together and show how we are "United, At Home."

Friday, June 12th at 7:00 p.m. CT

What represents "unity" for you? Share this symbol with us on your social channels.

cure SMA

#SMAConferenceAtHome and #CureSMA

Thank you to our generous sponsors for their support of the 2020 Virtual SMA Conference. This opportunity offers a unique experience to work in partnership with one another to enhance groundbreaking research and provide families the support they need today.

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MONDAY, JUNE 8TH (ALL TIMES ARE CT)

10:00AM – 11:00AM

WELCOME: OPENING SESSION

Kenneth Hobby, President, Cure SMA
Richard Rubenstein, Esq., Board Chair, Cure SMA
Nick Farrell, Incoming Board Chair, Cure SMA

Cure SMA would like to welcome the SMA community together as we kick off the 2020 Virtual SMA Conference. This opening session will be an introduction to the week-long conference activities, sessions, and events that are planned.

11:30AM – 12:15PM

CLINICAL CARE AND RESEARCH UPDATES

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

As we continue to kick-off the first day of our Virtual SMA Conference, Cure SMA will provide updates on clinical care and research.

1:00PM – 2:00PM

NEWLY DIAGNOSED VIRTUAL SESSION

(for newly diagnosed families only)

Kenneth Hobby, President, Cure SMA
Colleen McCarthy O'Toole, Vice President, Events & Family Support, Cure SMA
Mary Schroth, MD, FAAP, FCCP, Chief Medical Officer, Cure SMA
Rob Graham, MD
Danyelle Sun, SMA Mother, Type 2 and Type 3
Amanda DeVay, SMA Type 1
Angela Wrigglesworth, Adult with SMA, Type 2
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.

3:00PM – 4:00PM

FAMILY SYMPOSIUM, SPONSORED BY GENENTECH

SMA MY WAY: WHEN RESILIENCE IS A SUPERPOWER – SUPPORT SYSTEMS, CHANGING PERSPECTIVES AND FOCUSING ON THE POSITIVES

with Shane Burcaw and Hannah Aylward

6:00PM – 7:00PM

GRANDPARENTS VIRTUAL MINGLE

Join other grandparents who are affected by SMA as you "mingle" together at home. Connect, network, and chat through Zoom. Pre-registration is required to join.

7:00PM – 9:00PM

ADULTS WITH SMA SOCIAL AT HOME,

SPONSORED BY BIOGEN

Join other adults with SMA as you socialize from home. Connect, network, and chat through Zoom. Pre-registration is required to join.

TUESDAY, JUNE 9TH (ALL TIMES ARE CT)

10:00AM – 10:45AM

AQUATIC PHYSICAL THERAPY

Jennifer Martyn, PT, Wave Therapies, PLLC

The pool offers great benefits for individuals of all ages with SMA. Come spend some time out of the water to hear the whys and hows of aquatic exercise for you or your family member. We will discuss reasons to (and not to) use the water, what to look for in aquatic facilities, safety, equipment, and exercises. This is a great time to come and get your creative juices flowing for how some items might be adapted to be just perfect for your needs.

11:30AM – 12:15PM

LIFE AFTER DIAGNOSIS: 25 YEARS OF LESSONS LEARNED

Al Freedman, PhD, SMA Dad and Child/Family Psychologist

A diagnosis of SMA brings along with it a wide range of unusual and complicated challenges: medical, social, emotional, and financial, among others. The SMA journey can also offer us some unanticipated positive consequences. In this session, Dr. Freedman, a psychologist and the father of a 25-year-old SMA-affected son, will offer tips to individuals and families on how to navigate the many social and emotional challenges that come with an SMA diagnosis.

2:00PM – 3:00PM

FAMILY SYMPOSIUM, SPONSORED BY BIOGEN

TREATMENT AND MULTIDISCIPLINARY CARE IN SMA

7:00PM – 9:00PM

ADULTS WITH SMA LOUNGE NIGHT,

SPONSORED BY BIOGEN

Drop in to chat with friends this evening. The lounge is open for 2 hours to connect and interact with other SMA affected adults in the community. Pre-registration is required to join.

WEDNESDAY, JUNE 10TH (ALL TIMES ARE CT)

10:00AM – 10:45AM

MEDICAL MANAGEMENT OF ADULTS WITH SMA

Tina Duong, MPT, PHDc
Bakri Elsheikh, MD

This workshop will focus on three key areas: 1) Standards of care related to the adult medical and rehabilitation management of SMA in the current natural history landscape; 2) Challenges related to obtaining Spinraza treatment, present experience, and expectations; and 3) The latest research associated with other combination therapies that may be effective in adults with SMA. Specific attention will be aimed toward the importance of integrating, coordinating, and directing one's own care. The session is ideal for families and participants who are transitioning to adult clinics or adults with SMA.

11:30AM – 12:15PM

HOW TO FIND, HIRE, AND MANAGE YOUR PCAs

Carolyn Barrett, SMA Adult
Paula Barrett, SMA Parent

This workshop is an introductory seminar designed to give you the tools you need to hire your own personal care assistants (PCAs). What to look to for? Where to look? How to describe your needs? In answering these questions, we will strive to help you learn how to recruit, interview, and screen PCA applicants. This workshop is designed for anyone that is looking to bring PCAs into their life for the first time or young adults who may be looking to independently manage their team of PCAs, but all are welcome.

1:00PM – 1:45PM

RELEVANT ASSISTIVE TECHNOLOGY TOOLS FOR INDIVIDUALS WITH SMA: WHERE WE'VE COME AND WHERE WE ARE GOING!

Dan Phillips, AAC/AT Specialist
Hanna Eide, SMA Adult

Technology has become so much a part of all our lives that is seems to be constantly around us. It can seem overwhelming for many of us just to manage our daily lives. For individuals with motor challenges, technology can be a critical piece in independence and opening a world of possibilities. It is often difficult to keep up with assistive technology and determine the most appropriate tools in our ever-changing tech landscape.

Hanna Eide, a 23-year-old individual with SMA, and Dan Phillips, an assistive technology professional, will share their expertise and experience with using a wide variety of assistive technology tools. This workshop will explore assistive technology tools that are relevant for individuals with SMA in a wide variety of areas that include:

- Augmentative and Assistive Communication
- Digital Media Tools
- Social Media
- Reading and Writing
- Artificial Intelligence Tools

3:00PM – 4:00PM

FAMILY SYMPOSIUM, SPONSORED BY AVEXIS

ZOLGENSMA® (ONASEMNOGENE ABEPARVOVEC-XIOI) CLINICAL STUDY UPDATE

7:00PM – 9:00PM

ADULTS WITH SMA SOCIAL AT HOME,

SPONSORED BY BIOGEN

Join other adults with SMA as you socialize from home. Connect, network, and chat through Zoom. Pre-registration is required to join.

THURSDAY, JUNE 11TH (ALL TIMES ARE CT)

10:00AM – 10:45AM

ORAL PLACEMENT THERAPY (OPT): USING MUSCLE-BASED THERAPY WITH CLIENTS WITH THE DIAGNOSIS OF SMA

Renee Hill MS, CCC/SLP COM(R)

This workshop is for participants who are having difficulty with oral control for feeding, speech, saliva control, and/or oral rest posture. During this session, attendees of all SMA types and function levels will learn how using a muscle-based approach to oral function can assist in maintaining and improving motor function, reducing fatigue. This approach focuses on assessing the underlying motor system and developing a plan based on current function.

11:30AM – 12:15PM

BREATHING BASICS

Richard Shell, MD
Richard Kravitz, MD
Oren Kupfer, MD
Jane Taylor, MD

Individuals with SMA are at risk for difficulty breathing due to muscle weakness. This workshop will discuss why muscle weakness causes difficulty breathing during sleep, illness, and after surgery, and how to know when breathing support machines, such as 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.

2:00PM – 2:45PM

SMA CLINICAL TRIALS

Diana Castro, MD
John Brandsema, MD

Two treatments are approved for individuals with SMA, and more treatments are in development and clinical trials. This workshop brings together neurologists who conduct SMA clinical trials for a discussion on how to think about and interpret the results from SMA clinicals trials, and how to think about this information when making decisions about treatments

3:30PM – 4:15PM

WOMEN'S HEALTH IN SMA

Emma Ciafaloni, MD, FAAN
Loralei Thornburg, MD

This workshop will highlight the importance of routine gynecologic care for women with SMA and the management of common gynecologic problems. Given the unique obstetrical issues confronted by patients with SMA, we 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.

FRIDAY, JUNE 12TH (ALL TIMES ARE CT)

10:00AM – 10:45AM

NAVIGATING THE SPECIAL EDUCATION PROCESS: SETTING OUR KIDS UP FOR SUCCESS

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

Special education can be intimidating for parents entering a whole new world of acronyms, timelines, and IEP goals. Knowing what you can expect for your child and asking for appropriate services and accommodations can be difficult. Join our session to learn the ins and outs of special education. Hear the stories of three students and the accommodations and services that benefited them.

11:30AM – 12:15PM

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.

2:00PM – 2:45PM

WHAT IT MEANS TO BE A TEEN ON WHEELS

Angela Wrigglesworth, M. Ed., Elementary Education Teacher, SMA Adult

It is hard to be a teenager today. The combination of rapidly changing hormones, peer pressure, and educational challenges are often too much for most young people to handle. If asked to imagine all these factors combined with a diagnosis of SMA, most would probably picture a disaster waiting to happen. But being a teen with SMA is not impossible and, in fact, can produce some of the most influential and positive times in a young person's life. This facilitated session, led by SMA community member Angela Wrigglesworth, will help to shed light on how to overcome the difficulties of being a teenager and bring forth a smooth segue into adulthood.

2021 ANNUAL SMA CONFERENCE

JOIN US IN AUSTIN, TEXAS!
THURSDAY, JUNE 10 – SUNDAY, JUNE 13, 2021

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

Cure SMA is excited to reunite the SMA community for the 2021 Annual SMA Conference in Austin, Texas from Thursday, June 10 – Sunday, June 13, 2021. Additional conference details will be announced in the upcoming months and registration will launch in the fall of 2020.

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

WE LOOK FORWARD TO SEEING YOU IN AUSTIN NEXT JUNE!

STAY TUNED

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

zolgensma®
(onasemnogene
abeparvovec-xioi)
suspension for intravenous infusion

"I will always remember the day

Adalyne received a one-time dose for SMA. It was like she was having another birthday."

Patti, mother of Adalyne

ZOLGENSMA® (onasemnogene abeparvovec-xioi) 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.

Adalyne, treated at
~2½ months and pictured
at 3 years, has SMA Type 1.

To learn more, talk to your child's doctor about **the one-time-only dose** and visit ZOLGENSMA.com.

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 a patient experiences unexpected bleeding or bruising.

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 AveXis at 833-828-3947.

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

avexis

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. Caregivers should seek immediate medical attention if a patient experiences unexpected bleeding or bruising.

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. Caregivers should 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 you can visit www.ZOLGENSMA.com for Full Prescribing Information.

MANUFACTURED, PACKED, DISTRIBUTED by

AveXis, Inc.
Bannockburn, IL 60015

US-ZOL-20-0121 05/2020

SPINRAZA®
(nusinersen) injection 12 mg/5 mL

10,000+ adults and children worldwide with spinal muscular atrophy (SMA) have been treated with SPINRAZA*

Join us for our Cure SMA Family Symposium

June 9th from 3:00-4:00 pm ET/ 2:00-3:00 pm CT

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 click here to see full Prescribing Information.

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).

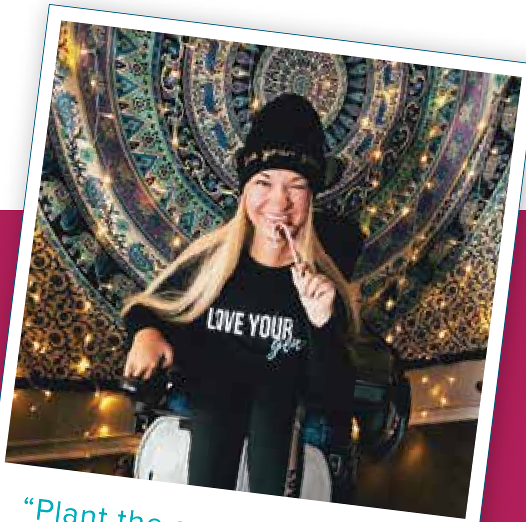
MANUFACTURED FOR

Biogen, Cambridge, MA 02142

*Based on commercial patients, early access patients, and clinical trial participants through December 2019.

225 Binney Street, Cambridge, MA 02142

Biogen.



“Plant the seeds of change.”

ALEX

“Keep laughing!”

SHANE

“You’re doing great!”

KEVAN

sma myway™

Individually driven. Community inspired.

Hear from fellow life travelers living with SMA, including authors, entrepreneurs, students, partners and more, and see how they’re approaching life goals and celebrating individuality.

Visit our website to explore these stories and more.

SMAMyWay.com

Genentech
A Member of the Roche Group


the reality of yes
Another future made better
by Ionis innovation.

ScholarRock

At Scholar Rock, our spirit of inquiry goes beyond traditional therapeutic approaches to uncover enlightened solutions for patients. Our investigational medicines include a novel muscle-directed treatment for SMA.

We want to thank the SMA community for their support of the TOPAZ trial.

Enrollment in the TOPAZ trial has been completed and we look forward to keeping the SMA community updated on our progress.

TOPAZ

ALCYONE LIFESCIENCES INC.

Innovating safe, convenient and cost-effective patient-centric CNS Drug Delivery

We are awed by the everyday courage, determination and grace of individuals and families living with SMA.

As proud sponsors of the Cure SMA 2020 Annual Conference, our Alcyone family is honored to recognize the amazing strength of the SMA community during the current public health emergency.

Please Stay Strong and Be Safe!

… an FDA designated Breakthrough Device* uniquely designed to enable chronic intrathecal therapy delivery and access, even during challenging times. SMA patients deserve safer and more convenient access to proven, best-in-class treatment with Spinraza. Ask your doctor about upcoming ThecaFlex DRx System clinical trials …

**An Investigational Device not yet approved by the US Food and Drug Administration*

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FOUNDATION

Our mission

The Permobil Foundation is dedicated to enhancing the quality of life by empowering strength and independence through community support, employee engagement and grant funding. We work in partnership with non-profit organizations and agencies to provide support and services so individuals can live a life without limitations.

Permobil Explorer Mini
Power mobility device designed to facilitate self-initiated movement, enable early exploration and support developmental milestones for young children with mobility impairments.

permobilfoundation.org |

Carolyn Barrett works full-time in marketing communications for the biggest university in Boston, Boston University. Specifically, she works with their College of Education and Human Development, managing social media, public relations, and internal/external communications. Carolyn is also in the process of completing her graduate degree in Public Relations. She graduated from Boston College in 2016 with her Bachelor's in Political Science. Carolyn lived on campus, worked in admissions, campus ministry, and managed more than a dozen personal care assistants. Carolyn loves to ski, spend time with friends, and drive her adapted vehicle. Her favorite days are spent enjoying her Boston neighborhood with her Service Dog, Shadow. She has been coming to the conference for as long as she can remember and is always happy to share her story and learn from others. Carolyn has SMA Type 2.

Paula Barrett is leadership development consultant and a partner at Leadership Partners, LLC. Paula is an experienced human resource professional with a history of success leading strategic and operational initiatives. She has worked with all types and sizes of organizations. Her specialties are in performance improvement, leadership development, employee engagement, and coaching leaders. Paula has a bachelor's degree in Business from Merrimack College and a master's degree in Business Administration from the D'Amore-McKin School of Business at Northeastern University. Paula also served as Chairman of the Board for Cure SMA for 5 years. Prior to this, she served as Secretary of the New England Chapter for 10 years. Paula lives in Massachusetts with her husband, Mike, and daughter, Carolyn. Carolyn has Type 2 SMA, recently graduated from Boston College, and is working full-time in Boston.

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.

Diana Castro, MD, is a specialist in Pediatric Neuromuscular Medicine and Child Neurologist at the University of Texas Southwestern. Dr. Castro is the co-director of the Pediatric Muscular Dystrophy Association (MDA) Clinic at Children's Health in Dallas, and the director of the Cure SMA Care Center Network. 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, following the current guidelines and new data from research trials. She is very interested in education for patients and other physicians nationally and Internationally.

Emma Ciafaloni, MD, FAAN, is a Professor of Neurology, Pediatrics, and Obstetrics/Gynecology at the University of Rochester. She is also the Director of Pediatric Neuromuscular Medicine and Director of the Cure SMA-sponsored SMA clinic. Dr. Ciafaloni has devoted her 25-year career to the diagnosis and treatment of adult and pediatric patients with neuromuscular diseases, particularly patients with muscular dystrophies, SMA, Duchenne, and myasthenia gravis. As Program Director of the Neuromuscular Medicine Fellowship for the past 17 years, she also takes pleasure in training residents and fellows in the diagnosis and treatment of patients with neuromuscular diseases. Her research interests have focused on experimental therapeutics in neuromuscular disease and she has been involved in clinical trials in pediatric and adult neuromuscular diseases for the past 20 years. She has also been involved in surveillance for muscular dystrophies through the Centers for Disease Control and Prevention-funded MD STARnet group and has done studies on pregnancy outcome in women with neuromuscular diseases.

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 and a master's in Educational Administration and Mid-Management from Texas A&M University. 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 program's 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.

Tina Duong, MPT, PhD, is a physical therapist at Stanford University in Palo Alto, CA. She has worked in neuromuscular disease for 18 years as a clinician and researcher in Washington, DC. Currently, she works in the multidisciplinary clinic and the research division for both adults and pediatrics. She practices evidence-based medicine to learn from research and find ways to translate it into clinical practice while addressing the patient focused goals. She has been a member of the Cure SMA Medical Advisory Council since 2018. She is an aspiring skier and in her free time she enjoys dancing, biking, running, and hiking.

Hanna Eide is an individual with SMA and has been using assistive technology since she was 3 years old. She is now pursuing a career in freelance web development after graduating from college in May of 2018. She is also an amateur photographer who specializes in animal photography and photo editing. Hanna loves sharing her experiences with technology to be helpful for anyone who is involved with someone with a disability or has a disability themselves.

Bakri Elsheikh, MBBS, FRCP, is an Associate 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, the Clinical Neurophysiology Fellowship, and the Adult SMA clinic at Ohio State University. His research focus is on the outcome measures and treatment of adults with SMA.

Albert Freedman, PhD, is a child and family psychologist at Freedman Counseling Associates in 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 health care 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 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 children and families with SMA.

Richard M. Kravitz, MD, is a Professor of Pediatrics in the Division of Pediatric Pulmonary and Sleep Medicine at the Duke University Medical Center in 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 Assistant 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 specialist, she has a broad range of experiences working with families and students who have Individualized Education Program ((IEPs). Her first-hand 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 plunged her into aquatics, with a group physical therapy program for children with a variety of diagnosis. Additionally, she provided clinical-based physical therapy and staffed the regional MDA 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, as well as improving functional skills. When not in the water Jennifer loves being with her family, biking, kayaking, gardening, and sewing.

Dan Phillips is a speech and language pathologist and assistive technology specialist in northern California. He is currently the director of the Technology Resource Center of Marin (www.trcmarin.org), a site-based assistive technology center that was created and designed by Dan in 2001, and currently serves the needs of more than 4,000 students in both special and general education in Marin County, CA. He has been working as an assistive technology specialist for the past 25 years. He teaches at three Universities in the San Francisco Bay Area, working with special needs teachers, occupational therapists, and speech pathologists. Dan has received Honors of the Association from the California Speech and Hearing Association, the highest honor in California for speech and language pathologists, as well as Outstanding Program for the Technology Resource Center. He has received three California Golden Bell teaching awards for his work within his organization for exemplary teaching. Last year, he was awarded Schoolmaster of the Year, as well as Marin County Teacher of the Year. He was a top 10 finalist for California Teacher of the Year. He was awarded Best School Technology Director from Tech Advocates and is nominated for the national award for Outstanding International Contributions by the American Speech and Hearing Association.

Renee Roy Hill, MS, CCC-SLP, has provided therapeutic assessments and program planning for adults and children with oral placement, feeding, and motor speech deficits for more than 20 years. She is the owner of Crossroads Therapy Clinic in New Braunfels, TX, and a member of the TalkTools® speakers bureau. Renee has been an invited speaker for American Speech-Language-Hearing Association (ASHA) state conventions and has received specialized training in speech/oral motor/feeding therapy, Apraxia, sensory processing disorders, NDT training and PROMPT. She is also a Certified Orofacial Myologist. She is the creator of the TalkTools® Apraxia Program.

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 New York State Bar Association; National Society of Financial Services Professionals; National Association of Insurance and Financial Advisors; Chairman, National Board of Directors, Cure SMA; Greater New York Chapter, Cure SMA; Town of Yorktown, Group Home Committee; and Chairman, Town of Yorktown, Board of Ethics. He currently resides in Yorktown Heights, NY, with his wife, Michele, 14-year-old daughter, Emma, and dog, Ike. He makes this presentation in loving memory of Max, "my special boy" (April 24, 2005–February 8, 2009).

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 the University of Texas Southwestern. 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.

Jane B. Taylor, MD, MsCR, is a Pediatric Pulmonologist in the Division of Pulmonary and Sleep Medicine at University of Pittsburgh Medical Center–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 for family reasons. She is now the pulmonologist in the multidisciplinary neuromuscular clinic at CHP and has started the CHP Family Medical Advocacy and Advisory Board, where she 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.

Loralei Thornburg, MD, is the James Woods Jr. Professor of Obstetrics and Gynecology at the University of Rochester, where she did both her OB/GYN residency and Maternal-Fetal Medicine fellowship, and now serves as both the Division and Fellowship Director for Maternal-Fetal Medicine. A native of western Michigan, she went to medical school at Wayne State in Detroit. She teaches annually at the SMFM/Banner Obstetrical Critical Care course, including both lecture and simulation—where she teaches on neurologic care for patients during pregnancy. She started a "rare disease" course in 2020 at the annual meeting for MFM to better help specialists be aware of the complex care needs during pregnancy for patients with uncommon challenges. She is lives in Rochester, NY with her husband, two children, a rabbit, and a flame gecko.

Angela Wrigglesworth, a third-grade teacher from Houston, 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 sixteen-year member of the National Task Force on Public Awareness through MDA 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 years' Virtual SMA Conference!

GET INVOLVED WITH YOUR LOCAL CHAPTER

Cure SMA has 36 volunteer chapters throughout the United States.

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

VOLUNTEER OPPORTUNITIES ARE AVAILABLE NATIONALLY

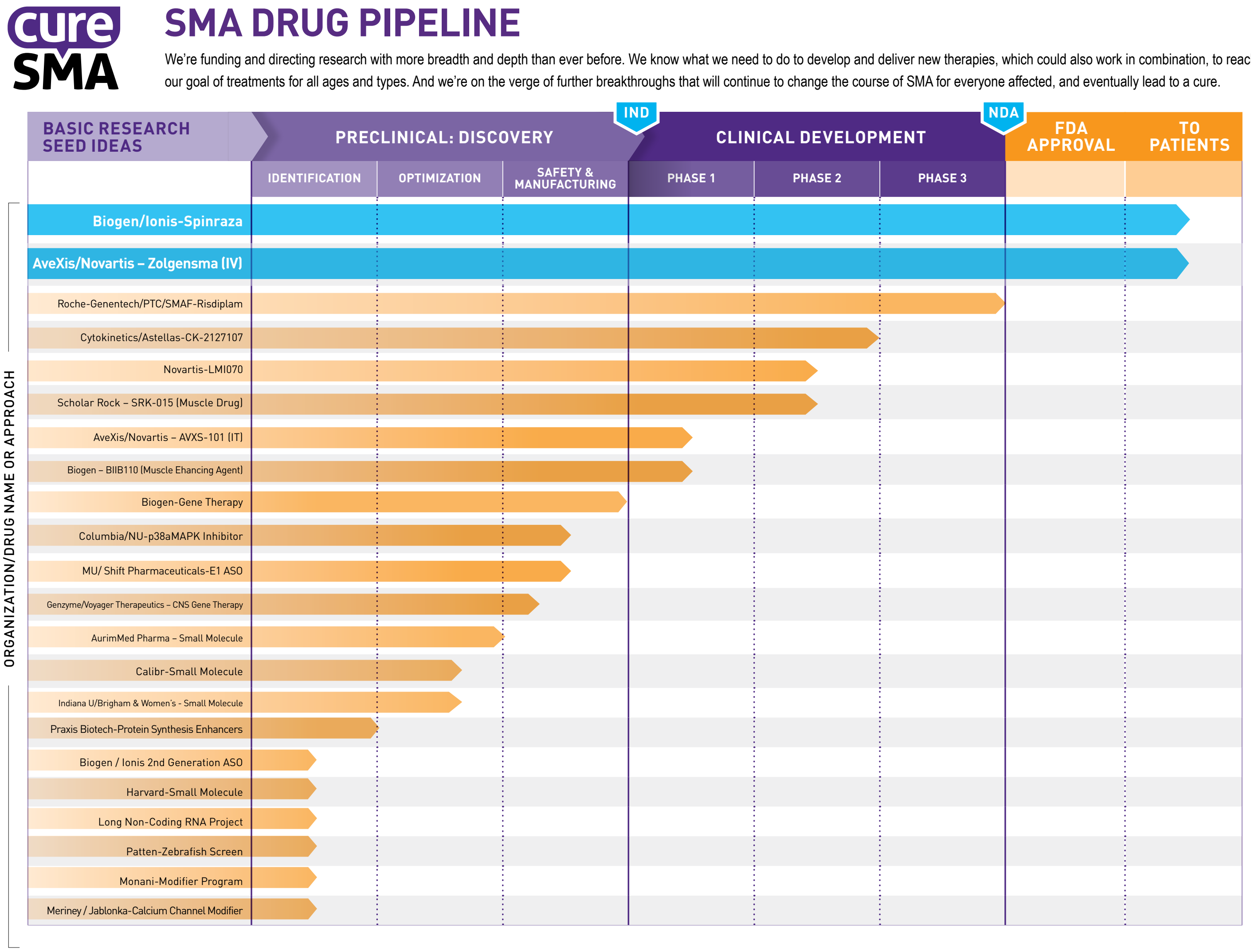
Please contact fundraising@curesma.org for more information.
Visit www.curesma.org/chapters

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, which could also work in combination, to reach our goal of treatments for all ages and stages of SMA. And we are on the verge of further breakthroughs that will continue to change the course of SMA for everyone affected, and eventually lead to a cure.

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 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

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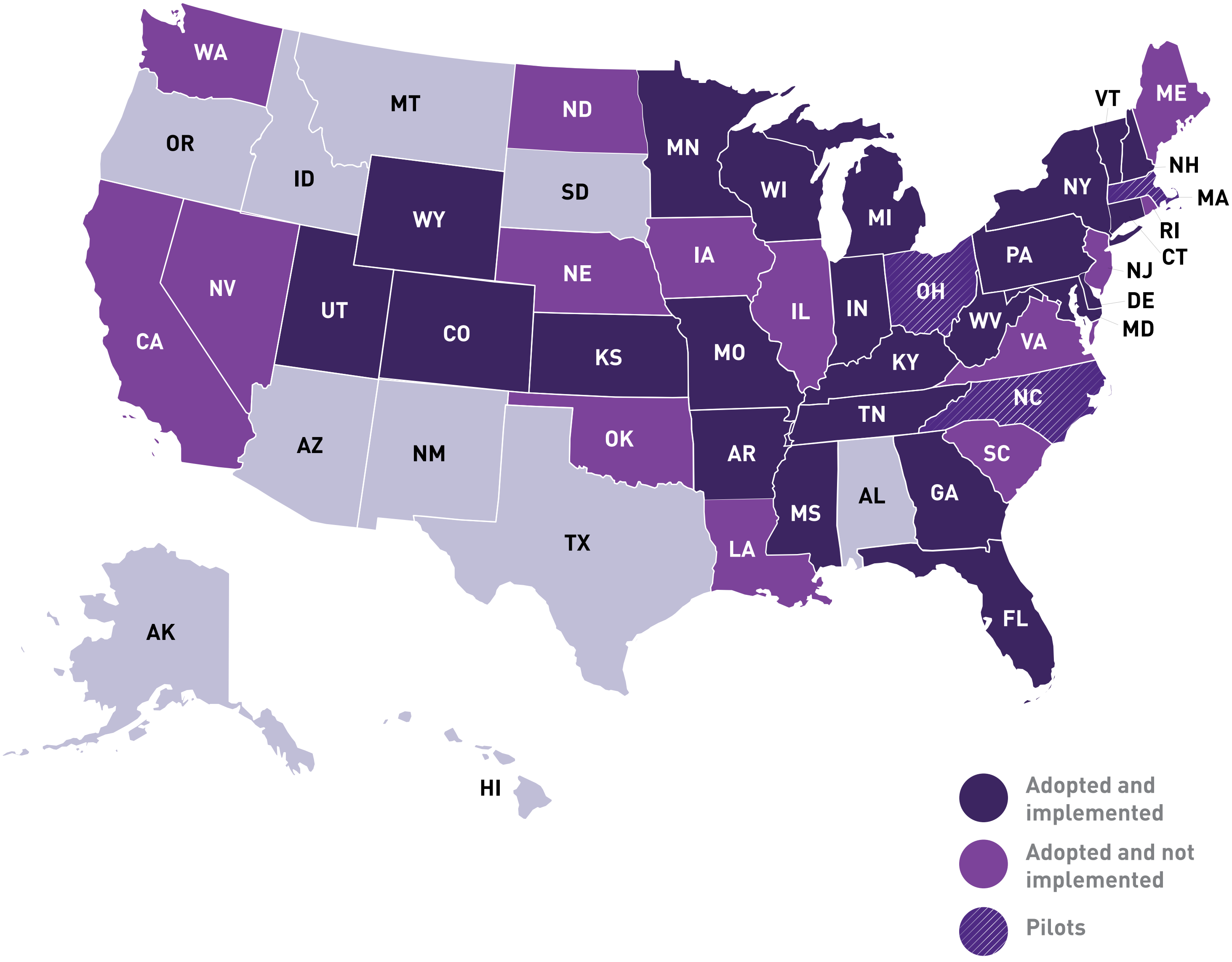
IND = Investigational New Drug **NDA** = New Drug Application Last updated: November 2019

NEWBORN SCREENING UPDATE

Early diagnosis and treatment are key in the fight against SMA. The best way to do this is through screening every newborn for SMA through their state's newborn screening program. Each state decides what conditions to screen for in these tests. Cure SMA has been working to ensure that every state screens for SMA, and thanks to the hard work of our families and advocates, we have made tremendous progress.

SMA newborn screening is being rapidly implemented throughout the U.S. As of May 1, 2020, 23 states have permanently implemented SMA newborn screening, 3 states have pilot SMA newborn screening programs, and 14 states have adopted and plan to implement SMA newborn screening programs soon. Currently, approximately 48 percent of all children with SMA are being screened for SMA.

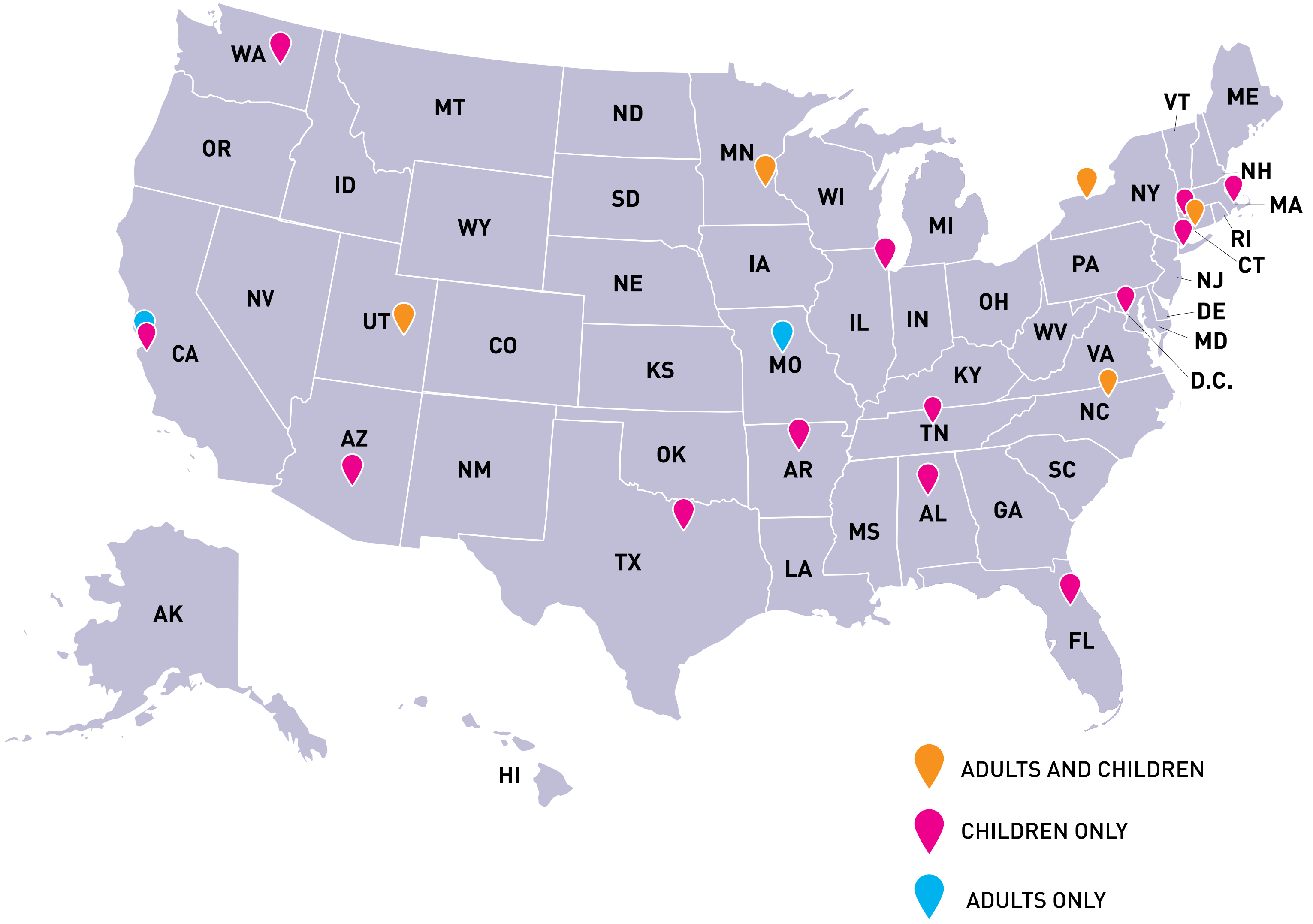


CARE CENTER NETWORK

The Cure SMA Care Center Network is made up of clinics who are partnering with Cure SMA to share consented electronic health record data with 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.

The Cure SMA Care Center Network now includes 20 centers geographically dispersed throughout the U.S., representing 14 pediatric centers, 2 adult centers, and 4 combined pediatric and adult centers. To date, 16 of these centers have fully integrated into the SMA Clinical Data Registry, where we have 274 patients enrolled. Patient enrollment is ongoing. The next expansion phase for the Cure SMA Care Center Network is planned for late 2020, and data collected in the SMA Clinical Data Registry will be utilized to evaluate current care practices and develop accreditation standards for the Care Centers.



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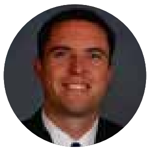
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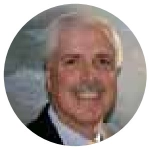
Gillian Mullins – Secretary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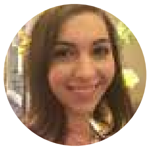
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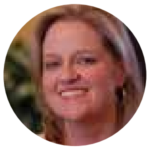
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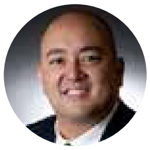
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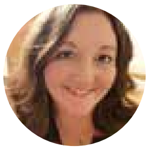
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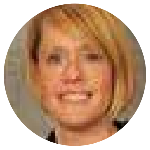
Medical Advisory Council (MAC)

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.

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

Tina Duong, MPT, PhDc
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
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Melissa Gibbons, MS, CGC
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Occupational Therapist at Cincinnati Children's Hospital Medical Center in Cincinnati, OH.

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

Chamindra Konersman, MD
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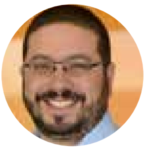
Richard M. Kravitz, MD
Pediatric Pulmonologist and Sleep Medicine Physician at Duke University in Durham, NC.

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.

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Genetic Counselor at Athena Diagnostics in Marlborough, MA.

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

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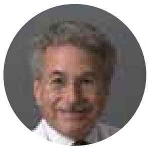
Pediatric Neurologist at Children's Hospital Colorado, associated with the University of Colorado in Denver, CO.

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



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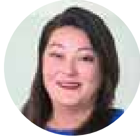
Chien-Ping Ko, PhD, Professor, University of Southern California, SMA Biology/Neurobiology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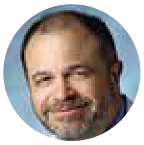
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