News & Updates March 2026

cure SMA NEWSLETTER FOR HEALTHCARE PROFESSIONALS

Welcome to the March edition of the Cure SMA Healthcare Provider Newsletter, featuring new clinical resources, educational opportunities, and recent publications to support your care for individuals with SMA.

- Educational Opportunities
- Clinical Resources
- Patient Resources
- Latest News: Clinical Trials & Treatment Updates

The Cure SMA Find a Treatment Center website is an online directory used by patients with spinal muscular atrophy (SMA) to connect to sites offering approved treatments.

TAKE A FEW MINUTES TODAY TO REVIEW OR UPDATE YOUR LISTING
www.curesma.org/find-a-treatment-center

The Cure SMA Find a Treatment Center Tool is an online resource used by patients and caregivers to locate sites offering SMA treatment and care.

Is Your Site Currently Listed & Up To Date?

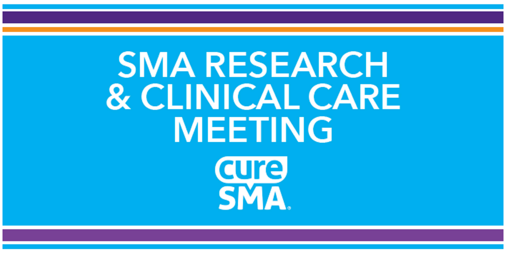
- If your center is listed, please review it for accuracy. You can update or remove outdated information.
- If your center is not included, add your details today!

Why Update or Add Your Site?

- Connect with individuals and families looking for SMA specialists.
- Support community access to SMA treatment by ensuring your center's listing accurately reflects the options available at your site.

Take a few minutes today to review or update your listing by clicking here.

Educational Opportunities



Annual SMA Research & Clinical Care Meeting

The 2026 Annual SMA Research & Clinical Care Meeting will take place June 24–26, 2026, in Orlando, Florida at Disney's BoardWalk Inn. This meeting brings together researchers from academia, government, and industry alongside multidisciplinary healthcare providers to share the latest advances in SMA research, treatment, and clinical care.

Held in parallel with the Annual SMA Conference, these meetings foster collaboration, education, and connection across the SMA community.

For more information, click here.

For questions, please contact SMARCC@curesma.org.

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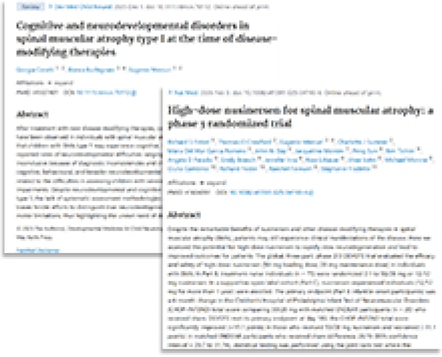
Clinical Resources

Recent Publications



Cognitive and neurodevelopmental disorders in spinal muscular atrophy type I at the time of disease-modifying therapies

Abstract

High-dose nusinersen for spinal muscular atrophy: a phase 3 randomized trial

Abstract

Impaired renal function in patients with spinal muscular atrophy: A longitudinal cohort study

Asselman, F. L., Meijvis, S. C., Wadman, R. I., Cuppen, I., Vernooij, R. W., Vermeer, L. M., van den Berg, L. H., Groen, E. J., & van der Pol, W. L. (2026). Impaired renal function in patients with spinal muscular atrophy: A longitudinal cohort study. Journal of neuromuscular diseases, 22143602251377240. Advance online publication.

Cognitive and neurodevelopmental disorders in spinal muscular atrophy type I at the time of disease-modifying therapies.

Coratti G, Buchignani B, Mercuri E. Dev Med Child Neurol. 2025 Dec 1. doi: 10.1111/dmcn.70112. Online ahead of print. PMID: 41327401

Pregnancy in women with spinal muscular atrophy (SMA): maternal and neonatal outcomes with multi-speciality management

Cohen, D., Nana, M., Hart, N. et al. Pregnancy in women with spinal muscular atrophy (SMA): maternal and neonatal outcomes with multi-speciality management. J Neurol 273, 88 (2026).

High-dose nusinersen for spinal muscular atrophy: a phase 3 randomized trial.

Finkel RS, Crawford TO, Mercuri E, Sumner CJ, Garcia Romero MDM, Day JW, Montes J, Sun P, Tichler B, Paradis AD, Boesch E, Inra J, Littauer R, Sohn J, Monine M, Gambino G, Foster R, Farewell R, Fradette S. Nat Med. 2026 Feb 3. PMID: 41634391

Spinal Muscular Atrophy Carrier Screening: Assessment of Provider Knowledge and Clinical Practice

Riegel, Melissa, Whitney Bender, Elizabeth Critchlow, and Lorraine Dugoff. 2026. “ Spinal

Muscular Atrophy Carrier Screening: Assessment of Provider Knowledge and Clinical Practice.," Prenatal Diagnosis: 46. no. 2), 229–23

An updated review of the SMA clinical trial landscape in the United States: Findings from analysis of recruitment targets on ClinicalTrials.gov and a survey of SMA clinical trial sites on factors affecting site capacity and readiness
Sarr, F., Peterson, I., Glascock, J., & Curry, M. (2026). An updated review of the SMA clinical trial landscape in the United States: Findings from analysis of recruitment targets on ClinicalTrials.gov and a survey of SMA clinical trial sites on factors affecting site capacity and readiness. Contemporary Clinical Trials Communications, 49, 101601

Examination of the Peripheral Nervous System in Children With Spinal Muscular Atrophy: A High-Resolution Ultrasonographic Study
Wurster J, West E, Meier S, Bürke NP, Jansen L, Broser PJ. Examination of the Peripheral Nervous System in Children With Spinal Muscular Atrophy: A High-Resolution Ultrasonographic Study. Brain Behav. 2026 Feb;16(2):e71234. PMID: PMC12876041.

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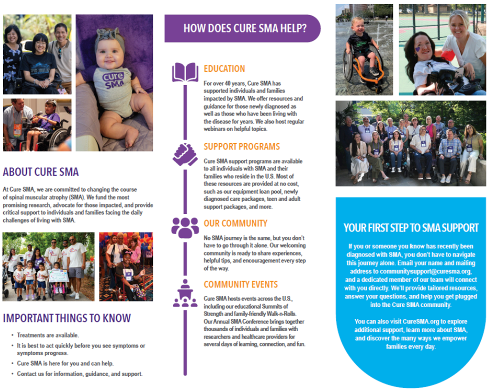
Patient Resources

SMA Patient Resources Available for Request

Explore our resources for healthcare providers. The Clinical Resources page now features direct links to request forms for Cure SMA patient resource guides and educational materials.



ABOUT CURE SMA

At Cure SMA, we are committed to changing the course of spinal muscular atrophy (SMA). We fund the most promising research, advocate for those impacted, and provide critical support to individuals and families facing the daily challenges of living with SMA.

IMPORTANT THINGS TO KNOW

- Treatments are available.
- It is best to act quickly before you see symptoms or symptoms progress.
- Cure SMA is here for you and can help.
- Contact us for information, guidance, and support.

HOW DOES CURE SMA HELP?

EDUCATION

For over 40 years, Cure SMA has supported individuals and families impacted by SMA. We offer resources and guidance for those newly diagnosed as well as those who have been living with the disease for years. We also host regular webinars on helpful topics.

SUPPORT PROGRAMS

Cure SMA support programs are available to all individuals with SMA and their families who reside in the U.S. Most of these resources are provided at no cost, such as our equipment loan pool, newly diagnosed care packages, teen and adult support packages, and more.

OUR COMMUNITY

No SMA journey is the same, but you don't have to go through it alone. Our welcoming community is ready to share experiences, helpful tips, and encouragement every step of the way.

COMMUNITY EVENTS

Cure SMA hosts events across the U.S., including our educational Summits of Strength and family-friendly Walk-n-Rolls. Our Annual SMA Conference brings together thousands of individuals and families with researchers and healthcare providers for several days of learning, connection, and fun.

YOUR FIRST STEP TO SMA SUPPORT

If you or someone you know has recently been diagnosed with SMA, you don't have to navigate this journey alone. Email your name and mailing address to communitysupport@curesma.org, and a dedicated member of our team will connect with you directly. We'll provide tailored resources, answer your questions, and help you get plugged into the Cure SMA community.

You can also visit CureSMA.org to explore additional support, learn more about SMA, and discover the many ways we empower families every day.

The Cure SMA Resource Request Form includes adaptive sports and recreation guides, driving and vehicle guides, state resource guides, and more.

The Cure SMA Care Series Publications & Clinic Materials Form allows you to request printed materials, including our Care Series Booklets and a new informational trifold on SMA and ways Cure SMA can help.

To access click here.

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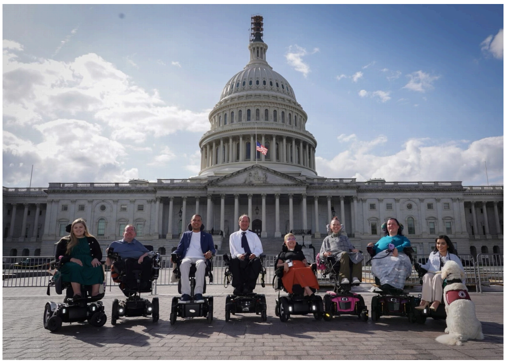
Latest News: Clinical Trials & Treatment Approvals



SMA Community Research & Legislative Priorities Finalized in New Federal Law

On February 3, Congress approved and the President signed into law key research and legislative priorities that Cure SMA and individuals with SMA and their families have been advocating for over the past year. The **Consolidated Appropriations Act of 2026** includes the following provisions that will benefit children and adults with SMA and their families:

- Increased federal research funding for SMA
- Extension of the Rare Disease Research & Development Program
- A streamlined process for accessing specialized care

To learn more, click **here.**

Scholar Rock

The SMA OPAL Study is a Phase 2 clinical study that will test multiple doses of apitegromab in children under 2 years old with 5q SMA who show delayed motor development. The study will closely examine how the drug works in the body, as well as its safety, tolerability, and potential benefits. Please contact **clinicaltrials@scholarrock.com** for more information. Or **click here** for more information.

Biogen

The **STELLAR-1 Study** is a Phase III clinical trial evaluating the effects of a new investigational drug called salanersen. The study drug salanersen is administered once per year and works by helping the body produce more SMN protein. The STELLAR-1 study will enroll newborns who have been diagnosed with spinal muscular atrophy (SMA) but are not yet showing symptoms. **Explore the Study**

The **SMA PIERRE PK Study** looks at how the body absorbs and processes nusinersen when it is delivered through an implanted device called the ThecaFlex DRx system, compared to the standard spinal injection. The device uses a small tube and port placed under the skin to deliver the medication. People with SMA who are already participating in the PIERRE study may also take part in this research. Please contact **clinicaltrials@biogen.com** for more information. You can also **click here** to get started.

argenx

The Sparkle Study is a clinical study aimed at assessing if the study drug, called ARGX-119 is safe and can help children and teenagers who have spinal muscular atrophy (SMA). ARGX-119 is designed to help keep the connection between nerves and muscles and improve the signalling between them. This may improve muscle strength and reduce tiredness. **Explore the study**

Genentech
A Member of the Roche Group

Genentech

Genentech recently released an update on their continued collaboration with the SMA community and Evrysdi® tablet updates. They will continue to provide timely updates as they become available. **Read the full community update letter here**

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About Cure SMA

Cure SMA leads the way to a world where everyone impacted by spinal muscular atrophy (SMA) is empowered to lead independent, successful, and fulfilling lives. We strive to create a community where every individual is heard and feels welcomed. Cure SMA provides practical support programs for our community and advocates for their needs. We fund and direct comprehensive research that drives breakthroughs in treatment, and we advance access to high quality care. We will not stop until we have a cure.

cure SMA®

DONATE ♥

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