



NEWSLETTER FOR HEALTHCARE PROFESSIONALS

We hope you enjoy the second edition of Cure SMA's Healthcare Professionals Newsletter. Thank you for all you do.

- [Educational Opportunities](#)
- [Clinical Resources](#)
- [Patient Resources](#)
- [Share Your Expertise](#)
- [Latest News: Clinical Trials & Treatment Approvals](#)

Did You Know?

- **15 days** - the average age at first treatment for infants with 1-2 SMN2 copies identified by SMA newborn screening in 2023.
- **0.75 deaths** per 100 individuals with SMA in 2023 compared with 2.4 deaths per 100 individuals with SMA in 2013. Great work, but there is more to do.
- **50%** of all people living with SMA in the U.S. are 18 years of age or older.
- **97%** of surveyed adults living with SMA hope new therapies will help them gain muscle strength. See the new clinical trials progress and pending FDA approvals.

For insights on the SMA community, check out the [Cure SMA 3rd Annual State of SMA Report](#). Stay tuned for updated metrics in the upcoming 4th Annual State of SMA Report!

Educational Opportunities



Annual SMA Research & Clinical Care Meeting

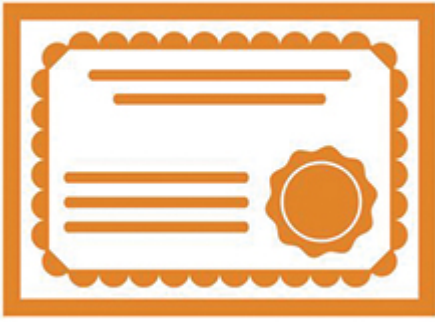
The 2025 [Annual SMA Research & Clinical Care Meeting](#) will be held in Anaheim, California from Wednesday, June 25 – Friday, June 27, 2025, at Disney's Grand Californian Hotel.

Some of this year's topic areas will include:

- Dual Treatment or Combination Therapy
- Scoliosis and Orthopedic Interventions
- Neurocognition in SMA
- Acute Pulmonary Care
- Genitourinary Issues

Through these meetings, we bring together researchers, healthcare professionals, and families to network, learn, and collaborate. If you have any questions, please reach out to SMARCC@curesma.org.

[Click here to register!](#)



Earn CME for a Chance to Attend Conference!

Are you looking to expand your SMA expertise? Check out valuable educational opportunities [here](#).

For an opportunity to be awarded one free registration and 3 nights hotel to the 2025 Annual SMA Research & Clinical Care Meeting, please submit your SMA education CME/CE certificates to patientcare@curesma.org.

2025 Call for MAC Applications

Cure SMA is seeking applicants to join our Medical Advisory Council (MAC) — a leading body of experts dedicated to improving SMA diagnosis, care management, and translating research into clinical practice.

We currently have openings on our Conference Committee, which plays a vital role in shaping content for the Clinical Care Session of the Annual SMA Research & Clinical Care Meeting. Conference Committee responsibilities include:

- Develop the Clinical Care Session by defining key topics, creating special sessions, and overseeing abstract submissions.
- Identify new timely topics and presenters for the Annual SMA Conference.

Apply here: [Medical Advisory Council Application](#)

Deadline: April 25

Announcements: May

Clinical Resources

SMA Quarterly Newsletter

Did you miss a previous newsletter?

Check out our archives on the Cure SMA website by [clicking here!](#)

Recent Publications

To access these publications, [click here.](#)

Development of the SMA EFFORT: A new approach to characterize perceived physical fatigability in spinal muscular atrophy.

Rodriguez-Torres R. et. al. J Neuromuscul Dis. 2025 Jan-Feb;12(1). doi: 10.1177/22143602241313326.

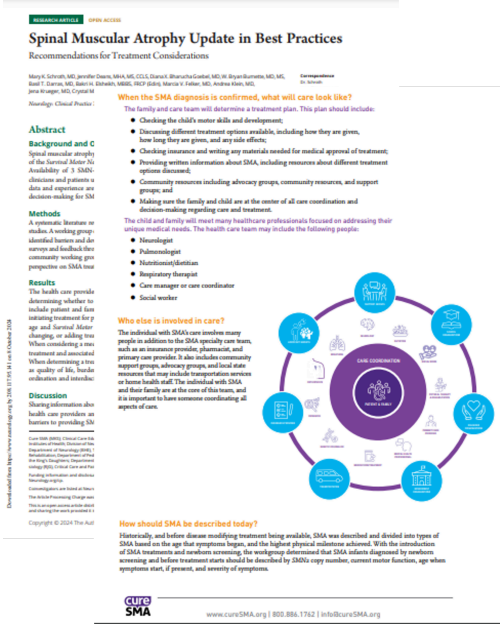
Spinal presentations in children with spinal muscular atrophy type 1 following gene therapy treatment with onasemnogene abeparvovec - The SMA REACH UK network experience.

Wolfe A, et al. Neuromuscul Disord. 2024 Nov;44:104451. doi: 10.1016/j.nmd.2024.104451.

Spinal Muscular Atrophy Update in Best Practices: Recommendations for Treatments Considerations.

Schroth M, et al. Neurol Clin Pract. 2025 Feb;15(1):e200374. doi: 10.1212/CPJ.0000000000200374

Share Your Expertise





Providers: Please Check Your Center's Listing for Accuracy

The Cure SMA Find a Treatment Center website is used by patients, caregivers, and healthcare providers to locate sites offering SMA treatments in the U.S.

This searchable webpage provides the site name, address, contact information, patient age group served, and SMA treatments offered. We invite you to update or add your treatment location.

ACTION ITEM: Please check your center's listing.



SMA Treatment Center Survey

As a valued clinician and member of the SMA community, Cure SMA and Scholar Rock invite you to participate in a short survey, which seeks to understand your treatment center's infusion capabilities. By completing this brief survey, you will help Scholar Rock accelerate patient access strategies and prepare for the potential launch, pending regulatory approval, of an infused treatment for individuals living with SMA.

Your participation in the survey is completely voluntary and all your responses will be kept confidential. The brief 20 question survey will take less than 5 minutes to complete. To take part in the survey, click <https://www.surveymonkey.com/r/DVJVGNM>.

If you have any questions, please feel free to reach out to Scholar Rock at trade@scholarrock.com.



Cure SMA is advocating for additional federal funding for Spinal Muscular Atrophy research and we need your help!

**cure
SMA**

Advancing SMA Research Through Advocacy

Cure SMA is advocating for additional federal funding for spinal muscular atrophy research. In February, we rallied in support of federal SMA research investments to develop new treatments focused on improving muscle strength, respiratory function, fine motor skills, and overall quality of life. View the [signed letter](#) and send it to your [congressional leaders](#) to ensure they see the strong support for SMA research.



Currently Recruiting: Exploration of the Diagnostic Journey of Adult-Onset SMA

Cure SMA has developed a short survey to explore the diagnostic journey and unmet needs of adults living with Type 4 SMA. The survey is designed to evaluate gaps in the existing research such as time to diagnosis and treatment, comorbidities (prior to and after SMA diagnosis), and ongoing disease management.

To help Cure SMA reach more individuals living with Type 4 SMA, please share this [IRB-approved flyer](#) with your patients as an opportunity for them to share their experiences with Cure SMA. This survey is only intended for those living in the United States, including Puerto Rico. The survey ends on April 14, 2025.

Patient Resources

Traveling with SMA

Traveling with SMA is often stressful and can require additional planning so we have pulled together a variety of resources as well as a travel support package to help make traveling easier, safer and more comfortable.

Quick Links

- Support Programs
- Travel Support Package
- Travel Resource Guides
- Custom Wheelchair Tags
- Report Airlines for ACAA Violations
- Advocacy



New Travel Resources

Cure SMA's newly launched [travel webpage](#) offers a variety of resources for the entire SMA community to help make traveling easier, safer, and more comfortable.

Resources include:

- Travel Related Support Programs
- Cure SMA Travel Resource Guides
- Helpful Travel Resources and Links
- Custom Wheelchair Tags
- Reporting Airlines for ACAA Violations
- Accessible Air Travel Advocacy

If you are interested in requesting a Travel Support Package, please fill out the [request form](#). Applicants must have a confirmed SMA diagnosis, be 18 years of age or older, and reside within the United States.

Resources Guide

General Guides:

- ❖ National Resource Guide
- ❖ State Specific Resource Guide

Insurance Guides:

- ❖ Insurance Letter of Medical Necessity Checklist
- ❖ Insurance Appeals Checklist

School Guides:

- ❖ DEI Children's Book Recommendations
- ❖ Educator's Guide to SMA
- ❖ Sample School Letter Guide
- ❖ Guide to Talking with Children about Disabilities

Travel Related Guides:

- ❖ Traveling with SMA Resource Guide
- ❖ Air Travel Resource Guide
- ❖ Air Travel Checklist
- ❖ Airplane Cargo Hold Dimensions
- ❖ Know Your Rights when Traveling by Airplane
- ❖ Guide to Creating your own Custom Wheelchair Tag

Resource Guides

Cure SMA offers resource guides for Individuals with SMA related to:

- Insurance
- School
- Travel
- Equipment
- Home Modification
- Driving & Vehicles
- Financial Guides
- PCAs
- And More

Request any of these guides on our website by [clicking here](#).

Have an idea for a useful resource guide? Send your ideas to communitysupport@curesma.org.

Latest News: Clinical Trials & Treatment Updates

Research

Risdiplam for Prenatal Therapy of Spinal Muscular Atrophy

Finkel RS, et al. N Engl J Med. 2025. DOI: <https://doi.org/10.1056/NEJMc2300802>

First-in-human study of epidural spinal cord stimulation in individuals with spinal muscular atrophy

Prat-Ortega G, et al. Nat Med. 2025. DOI: <https://doi.org/10.1038/s41591-024-03484-8>.

Genentech

On February 12, 2025, [Genentech announced that the U.S. Food and Drug Administration approved a New Drug Application for an Evrysdi® \(risdiplam\)](#), 5 mg tablet for people living with SMA. The tablet may be taken whole with water or dispersed in one teaspoon (5 mL) of room temperature non-chlorinated drinking water. The Evrysdi liquid will continue to be available. For more information, see [Evrysdi Tablet](#).

Biogen

[Biogen announced](#) that the U.S. Food and Drug Administration (FDA) has accepted their supplemental New Drug Application (sNDA) and the European Medicines Agency (EMA) has validated the application for a higher dose regimen of nusinersen for spinal muscular atrophy (SMA).

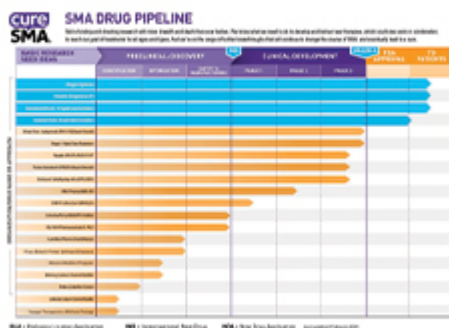
Scholar Rock

Scholar Rock [announced the submission of a Biologics License Application \(BLA\)](#) to the U.S. Food and Drug Administration (FDA) for apitegromab, a muscle-targeted therapy designed and developed to provide clinically meaningful improvement in motor function for people living with SMA who are receiving SMN-targeted treatments.

SMA Drug Pipeline

The SMA Drug Pipeline is how Cure SMA evaluates the success of our spinal muscular atrophy (SMA) research program. It identifies the major drug development programs and tracks their progress from basic research through FDA approval and beyond.

Please see the [SMA Drug Pipeline](#) for more information.



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.