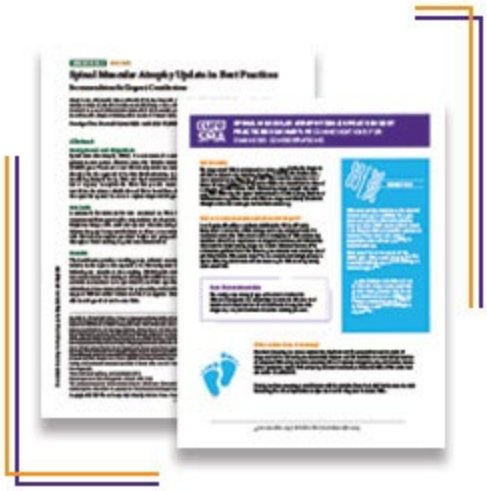
NEWSLETTER FOR HEALTHCARE PROFESSIONALS

Congratulations to all for advancing care, treatment and research for individuals living with SMA! We hope you enjoy the first edition of Cure SMA's Healthcare Professionals Newsletter. Thank you for all you do.



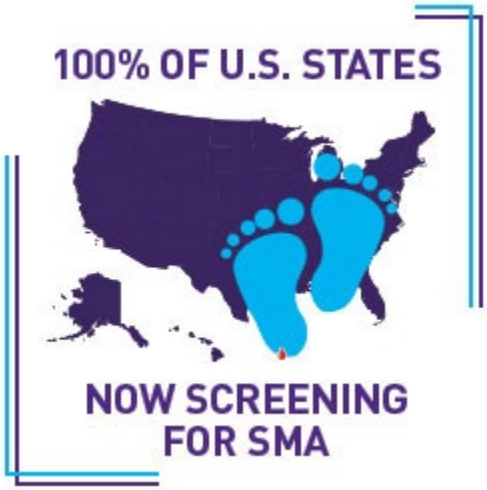
SMA Diagnosis and Treatment Best Practice Recommendations Updated

In collaboration with healthcare professionals across the U.S and Europe and the SMA community, Cure SMA published updated best practice guidelines on SMA diagnosis. This publication adds information about SMA newborn screening recommendations and provides criteria to consider for the diagnosis of adult onset SMA or SMA type 4. Remember that SMA can be diagnosed in ~95% of individuals with simple genetic testing for SMN1.

Cure SMA also published recommendations for treatment considerations. The driving recommendations when considering SMA treatments for a patient are the patient and family perspective and safety and side effects of the treatments. Please see the publication for a table of all three currently available FDA and EMA-

approved SMN-enhancing treatments. Note that a table of the pivotal clinical trials is also available as supplementary materials.

Both articles are available here. A companion plain language summary for each publication is also available to share with your patients. Click here to access.

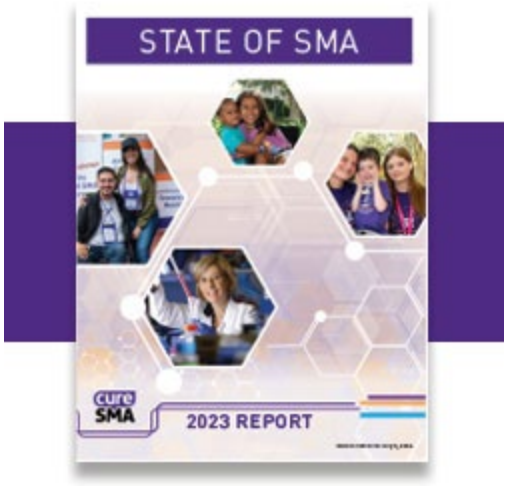


SMA Newborn Screening

In January 2024 SMA newborn screening was fully implemented across the U.S. This is an amazing achievement! Thank you to all for your advocacy and perseverance.

SMA NBS data provided by 30 state public health laboratories through 2021 revealed a revised incidence of SMA in the US of 1 in 14,694 live births as described in the publication below:

Newborn Screening and Birth Prevalence for Spinal Muscular Atrophy in the US



State of SMA 2023 Report Highlights

1. Cure SMA estimates 9000-9500 individuals with SMA are living in the U.S.
2. 50% of people living with SMA in the U.S. are adults.
3. 60-70% of individuals with SMA are estimated to have received an FDA-approved treatment.
4. Treating infants with SMA and two SMN2 copies between 0-14 days of life results in better outcomes at 2 years of age compared to infants treated between 15 and 30 days of life or older. Treating infants with SMA as early as possible results in the best outcomes.
5. 97% of adults with SMA report continuing unmet needs and they hope new therapies will help them gain muscle strength.

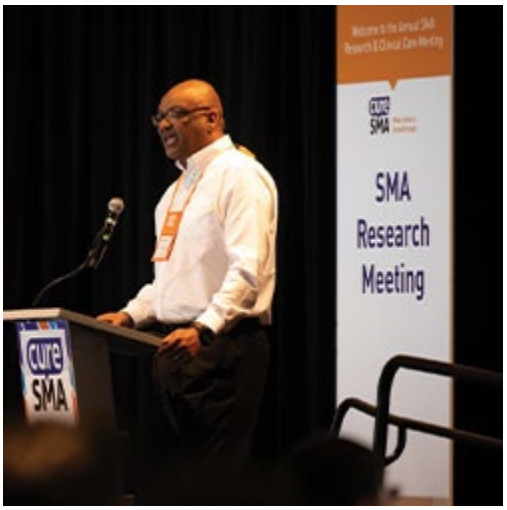
Access the full 2023 Report here.

Healthcare Professional Educational Opportunities

The following educational opportunities can be found on Cure SMA's website. **Click here to access**.

- Stanford University's 6th Annual Neuromuscular Conference, January 25, 2025 (*CME Credits Available*)
- STEP-IN Professional Development Series for Physical Therapists (*CME Credits Available*)
- Care and Management of Adults with SMA (*CME Credits Available*)
- Update in Best Practices: Diagnosis Recommendations Discussions
- SMA Educational Resources POD-NMD for Physical Therapists
- Support and care for children with SMA after gene replacement therapy

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**.



Annual SMA Research & Clinical Care Meeting

The Annual SMA Research & Clinical Care Meeting brings together researchers from academia, government, and biotech/pharmaceutical companies and multi-disciplinary healthcare providers who are diagnosing and caring for people with SMA to network and learn about the latest updates in SMA. The 2025 Annual SMA Research & Clinical Care Meeting will be held at the Grand Californian Hotel and Spa in Anaheim CA June 25 to 27, 2025. 5.5 hours of CME/CE will be provided.

This meeting is held concurrently with the Annual SMA Conference for people living with SMA, their caregivers and families.

Check out the 2025 Annual SMA Research & Clinical Care Meeting website for updates on registration and abstract submission.

STEP-IN SMA Clinical Evaluator Delphi Survey

The STEP-IN Advisory Board is looking for all stakeholders familiar with the work of a clinical evaluator to provide feedback on a **proposed list of competencies** (the essential characteristics of an excellent SMA clinical evaluator). This includes PTs, PIs, Industry Representatives, Clinic Directors and all members of the Multidisciplinary Team.

The request is to complete a survey providing feedback on each competency's clarity, importance and value and suggestions for missing competencies. The goal is to achieve consensus on these competencies and develop curriculum for a STEP-IN Clinical Evaluator track.

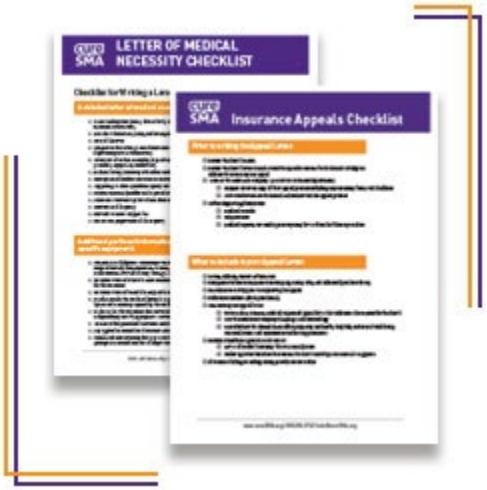
Please complete the survey by **December 1, 2024**. Access the survey here.

If you have any questions about the survey, please email Elizabeth at erh2171@cumc.columbia.edu.

Cure SMA Find a Treatment Center

The Cure SMA Find a Treatment Center tool is used by patients and caregivers, healthcare providers, and payers to locate SMA care and treatment centers 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.

ACTION ITEM: Please check your center's listing for accuracy here.



Insurance Resources

Resources designed to assist with managing insurance related to SMA care and offer guidance on the drafting of letters of medical necessity and navigating the process of appealing insurance denials.

Access insurance resources here.

Headlines from Clinical Trials

SAPPHIRE (Apitegromab - Scholar Rock) clinical trial met primary end point; U.S. Biologics License Application (BLA) planned in Q1 2025

DEVOTE (higher dose nusinersen - Biogen) clinical trial data supports the clinical benefits of a higher dose regimen of nusinersen (50/28 mg) in both individuals previously treated and treatment-naïve to nusinersen.

RAINBOWFISH (risdiplam – Genentech/Roche) clinical trial 2-year follow up data shows efficacy and safety in children first treated pre-symptomatically before 6 weeks of age, with most achieving typical motor milestones.

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.