



NEWSLETTER FOR HEALTHCARE PROFESSIONALS

Welcome to the October 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**](#)
- [**Share Your Expertise**](#)
- [**Latest News: Clinical Trials & Treatment Updates**](#)

Recent Publications



Respiratory management in spinal muscular atrophy: development of a global outcome measure

Edel, L., Civitello, M., Muntoni, F., Finkel, R., Mercuri, E., Baranello, G., & Mayer, O. (2025). Respiratory management in spinal muscular atrophy: development of a global outcome measure. *Neuromuscular disorders : NMD*, 56-57, 106253. Advance online publication. doi:10.1016/j.nmd.2025.106253

A qualitative, mixed-method approach to reaching consensus on function, fatigue, and fatigability outcomes in teens and adults living with spinal muscular atrophy

Glascok, J., Belter, L. T., Burk, M. M., Tingey, J. J., & Curry, M. A. (2025). A qualitative, mixed-method approach to reaching consensus on function, fatigue, and fatigability outcomes in teens and adults living with spinal muscular atrophy. *Orphanet journal of rare diseases*, 20(1), 519. doi:10.1186/s13023-025-04047-x

Onasemnogene abeparvovec gene therapy for treatment of patients with spinal muscular atrophy: Updated real-world practical considerations

Proud CM, Kichula EA, Matesanz SE, et al. Onasemnogene abeparvovec gene therapy for treatment of patients with spinal muscular atrophy: Updated real-world practical considerations. *Journal of Neuromuscular Diseases*. 2025;0(0). doi:10.1177/22143602251391258

Open-label phase 4 trial evaluating nusinersen after onasemnogene abeparvovec in children with spinal muscular atrophy

Proud, C. M., Finkel, R. S., Parsons, J. A., Masson, R., Brandsema, J. F., Kuntz, N. L., Foster, R., Li, W., Littauer, R., Sohn, J., Fradette, S., Youn, B., & Paradis, A. D. (2025).

Open-label phase 4 trial evaluating nusinersen after onasemnogene abeparvovec in children with spinal muscular atrophy. The Journal of clinical investigation, e193956. Advance online publication. doi:10.1172/JCI193956

Pregnancy in spinal muscular atrophy in the treatment era

Walter MC, Günther B, Astrid B, et al. State of the art: Pregnancy in spinal muscular atrophy in the treatment era. Journal of Neuromuscular Diseases. 2025;0(0).

doi:10.1177/22143602251370414



Find A Treatment Center Tool

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](#).

SMA RESEARCH & CLINICAL CARE MEETING



2026 Annual SMA Research & Clinical Care Meeting

This year's meeting will be held from Wednesday, June 24th – Friday, June 26th at Disney's Boardwalk Inn Hotel in Orlando, FL.

The Annual SMA Research & Clinical Care Meeting brings together researchers from academia, government, and biotech/pharmaceutical companies with multi-disciplinary healthcare providers who are diagnosing and caring for people with SMA. The event is held in conjunction with Cure SMA's Annual SMA Conference.

To stay up to date on conference information and abstract submission, please access our [**Annual Research & Clinical Care Meeting**](#) website.

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Patient Resources



Launched New Virtual SMA Parent Socials!

All parents of children with SMA are invited to join these Zoom get-togethers to connect with other parents across the U.S. and spend a fun evening together! These hour-long events will take place on the following Wednesdays at 8:00pm ET / 7:00pm CT / 5:00pm PT. Simply click on the links to learn more and register:

- [**Wednesday, January 21**](#)
- [**Wednesday, March 4**](#)

Please email [**communitysupport@curesma.org**](mailto:communitysupport@curesma.org) for more information.



Virtual Adults with SMA Socials are Back!

These online get-togethers are just for adults with SMA will take place at 8:00pm ET / 7:00pm CT / 5:00pm PT! Simply click on the links to learn more and register:

- [Tuesday, January 13](#)
- [Thursday, February 12](#)
- [Wednesday, March 11](#)
- [Tuesday, April 14](#)

Please email

communitysupport@curesma.org for more information.



Cure SMA Advocacy Seeks Everyday Living Stories & Experiences from SMA Community

Cure SMA has launched an [advocacy survey](#) to gather first-person stories and experiences from adults with SMA and families of children with SMA about their everyday living challenges and goals. Cure SMA regularly features these stories in its advocacy [reports](#), [statements](#), and [letters](#). We encourage you to invite your SMA patients and their families to share their perspectives and make their voice heard.



Navigating Your Clinical Trial Journey

Cure SMA has developed tools designed to empower clinical trial decision-making amongst patients and families. They include:

- **SMA Registry and Clinical Trial Finder**

- **Screening Visit Checklist**
- **Decoding Informed Consent**
- **Travel Planners (Air and Ground)**

To learn more and to share these resources with your patients and families, click [here](#).

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We Need Your Expertise!



SMA Physician Survey Research on Bisphosphonate Use

You are invited to complete a survey out of the Johns Hopkins Departments of Orthopaedic Surgery and Pediatrics assessing physician opinions regarding the use of bisphosphonates in the treatment of SMA. We are requesting your participation at the link below. This survey should take less than three minutes to complete. If you do not treat patients with SMA, we also would greatly appreciate it if you could please forward this email to your colleagues who do.

Survey Link [Here](#)

This survey will help us gain more insight into physician beliefs and practices regarding SMA treatment. Your time and participation are greatly appreciated.

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[Latest News: Clinical Trials & Treatment Approvals](#)



Novartis

Novartis announced that it has received approval from the U.S. Food and Drug Administration (FDA) for Itivisma (onasemnogene abeparvovec-brve) to treat adults and children 2 years of age and older with spinal muscular atrophy (SMA). Learn more [here](#).



Biogen

Biogen announced plans to initiate Phase 3 development to evaluate the effectiveness and safety of salanersen, an investigational drug for the treatment of spinal muscular atrophy (SMA). Phase 3 initiation follows the announcement of positive interim results of the Phase 1 study of salanersen, which informed the design of the studies.

For the full community statement from Biogen, please [click here](#).



Scholar Rock

Scholar Rock recently held an in-person Type A meeting with the FDA on November 12 to discuss the Biologics License Application (BLA) for apitegromab, a potential treatment for children and adults with SMA.

Cure SMA was present and active at this FDA meeting to represent our community to ensure that the patient voice and the patient need were heard and factored into decisions. In particular stressing the urgency needed to address unmet needs across the entire age spectrum in SMA. Read the full press release [here](#).



NMD Pharma, a clinical-stage biotech company, is seeking participants in their SYNAPSE-SMA clinical trial. SYNAPSE-SMA is an investigational SMA independent treatment for adults with Type 3 SMA that may enhance the activation of signals between nerve cells and muscles. [Learn more.](#)

Learn about this clinical trial [here](#).

Study flyer also available [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.



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