



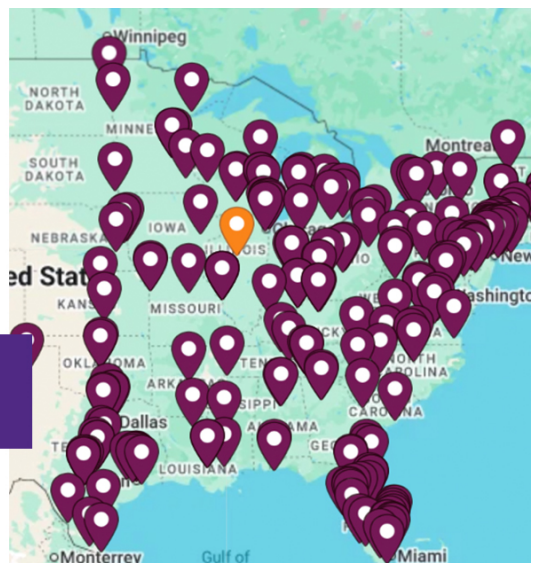
NEWSLETTER FOR HEALTHCARE PROFESSIONALS

Welcome to the September 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.

- [Clinical Resources](#)
- [Share Your Expertise](#)
- [Latest News: Clinical Trials & Treatment Updates](#)

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

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



Clinical Resources



CURE SMA

CARE SERIES BOOKLET

A HEALTH INSURANCE ROADMAP FOR PEOPLE LIVING WITH SPINAL MUSCULAR ATROPHY (SMA) AND THEIR CAREGIVERS

CHOICE AND CONNECTION TO TREATMENT AND CARE

Expanding Access: Health Insurance Roadmap in 13 Languages

Cure SMA's updated Health Insurance Roadmap is now available in 13 languages, expanding access to important health insurance information for individuals with SMA and their families. This resource helps families to better understand and navigate common insurance-related issues, how to talk with insurers, a glossary of key insurance terminology, and steps to consider when facing coverage denials and appeals. Healthcare providers can use the road map as a resource to direct patients and families with the information they need. The latest update also includes information on patient support and access programs for newly approved treatments.

Access our Health Insurance Roadmap [here](#).

Cure SMA Clinical Trials

Cure SMA's Clinical Trials page provides patients and families with information and

SMA Clinical Trials

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SMA.

practical tools to help them better understand and navigate clinical trial participation. Resources explain clinical trial phases, study teams, regulatory oversight, the informed consent process, and current research directions. In addition, the SMA Registry, Clinical Trial Finder, Screening Visit Checklist, and travel planners can help families identify opportunities and prepare for participation.

Healthcare providers are encouraged to share this resource with patients and families who are interested in learning more about clinical trials or considering participation.

To access these resources click [here](#).

Recent Publications

Assessment of Transition Plan Implementation and Provider Engagement Among Adults Living with Spinal Muscular Atrophy (SMA): Findings from a Cross-Sectional Study

A Cure SMA study, published in *Neurology and Therapy*, asked adults aged 18-29 about their experience transitioning from pediatric to adult care. Respondents who described their transition as positive were more likely to have received structured support from their provider, including readiness check-ins, planning conversations, and written transition plans. Nearly all respondents reported difficulty finding adult providers knowledgeable in SMA care. For providers managing SMA transitions, the findings offer a practical starting point for building a structured transition process.

Click [here](#) to access the publication.

Eisenman, L.E., Wise, A.R. & Curry, M.A. Assessment of Transition Plan Implementation and Provider Engagement Among Adults Living with Spinal Muscular Atrophy (SMA): Findings from a Cross-Sectional Survey. *Neurol Ther* (2026).

Strengthening adaptive functioning and participation in the contemporary spinal muscular atrophy paradigm: clinical

[Abstract] | [Full-text \(PDF\)](#) | [Download PDF](#) | [View abstract](#) | [View full-text](#)

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

Alfonso, A. | [View abstract](#) | [Download PDF](#)

Abstract

After treatment with new disease-modifying therapies, it has been observed in individuals with spinal muscular atrophy (SMA) type I that cognitive and neurodevelopmental disorders are reported. The aim of this study was to evaluate the prevalence of cognitive and neurodevelopmental disorders in individuals with SMA type I at the time of disease-modifying therapies. Cognitive and neurodevelopmental disorders were assessed in individuals with SMA type I at the time of disease-modifying therapies. The results of this study are presented in the abstract.

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

Alfonso, A. | [View abstract](#) | [Download PDF](#) | [View full-text](#)

Alfonso, A. | [View abstract](#) | [Download PDF](#)

Abstract

To explore the neuroprotective benefits of nusinersen and other disease-modifying therapies in spinal muscular atrophy (SMA), patients may not experience clinical manifestations of the disease. These results assessed the potential for high-dose nusinersen to modify disease progression and treat the associated motor deficits. The study design was a phase 3, randomized, controlled trial that evaluated the efficacy and safety of high-dose nusinersen (30 mg/kg) compared with low-dose nusinersen (10 mg/kg) in patients with SMA type I. The primary endpoint was the percentage of patients who achieved a 10% or greater increase in the mean change in the Functional Status Index (FSI) at 12 weeks. The results of this study are presented in the abstract.

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[View abstract](#) | [Download PDF](#)

perspectives and future directions

Farah, E. E., Farrar, M. A., Kariyawasam, D. S., & Paguinto, S. G. (2026). Strengthening adaptive functioning and participation in the contemporary spinal muscular atrophy paradigm: clinical perspectives and future directions. *World journal of pediatrics : WJP*, 10.1007/s12519-026-01063-0. Advance online publication.

Click [here](#) to access the publication.

Efficacy and safety of risdiplam in patients with type 1 spinal muscular atrophy: a 3-year open-label extension of the two-part, phase 2 FIREFISH trial

Mazurkiewicz-Beldzińska, M., Servais, L., Baranello, G., Boespflug-Tanguy, O., Day, J. W., Deconinck, N. P., Klein, A., Masson, R., Mercuri, E. M., Rose, K. J., Vlodavets, D. V., Xiong, H., Zanoteli, E., El-Khairi, M., Gaki, E., Kuthiala, M., Gorni, K., Kletzl, H., Palfreeman, L., Darras, B. T., ... FIREFISH Study Group (2026). Efficacy and safety of risdiplam in patients with type 1 spinal muscular atrophy: a 3-year open-label extension of the two-part, phase 2 FIREFISH trial. *The Lancet. Child & adolescent health*, 10(9), 625–636.

Click [here](#) to access the publication.

Ten years of disease-modifying therapy in spinal muscular atrophy: lessons learned and future directions

Mercuri, E., Finkel, R.S. & Muntoni, F. Ten years of disease-modifying therapy in spinal muscular atrophy: lessons learned and future directions. *Nat Rev Neurol* **22**, 549–563 (2026).

Click [here](#) to access the publication.

289th ENMC international workshop: assessing and managing emerging AAV related toxicities after gene therapy for neuromuscular disorders

Orbach, R., Gil Garzon, M. R., Büning, H., Bönnemann, C., Muntoni, F., & 289th ENMC Workshop participants (2026). 289th ENMC international workshop: assessing and managing emerging AAV related toxicities after gene therapy for neuromuscular disorders, 26 -

Click [here](#) to access the publication.

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

Proud CM, Kichula EA, Matesanz SE, Kumar A, Saito K, Lavery CG, Farrar MA, Bharucha-Goebel DX, Haberlová J, Mundada V, Kwon JM, McMillan HJ. Onasemnogene abeparvovec gene therapy for treatment of patients with spinal muscular atrophy: Updated real-world practical considerations. *J Neuromuscul Dis*. 2026 Jul;13(4):587-601. Epub 2025 Nov 10. PMID: 41212681; PMCID: PMC13434951.

Click [here](#) to access the publication.

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Share Your Expertise



SMA Patient Validation Survey

The International Consortium for Health Outcomes Measurement (ICHOM) SMA Working Group has proposed a minimum set of health outcomes that matter to people living with SMA. To explore whether these outcomes are meaningful to the community, ICHOM launched the SMA Patient Validation Survey. This survey collects feedback on the outcomes included in the SMA Set from adults with lived experience of SMA, as well as from caregivers of children or adults with SMA.

Title: What matters most when it comes to Spinal Muscular Atrophy Validation Survey

Time: one-time commitment of 10-15 minutes

Qualifications:

- Must be an adult (age of majority or

What matters most when it comes to Spinal Muscular Atrophy?



ICHOM – an international non-profit organization that aims to improve global healthcare – is developing a set of outcomes most important to people with spinal muscular atrophy. A Working Group (WGG) of globally diverse multidisciplinary experts consisting of providers, researchers, and those with lived experience has identified outcomes to be included in our recommendation.

To explore whether these outcomes are meaningful and capture what people with lived experience find most important, we invite you to complete the validation survey which intends to capture your thoughts on the level of importance of each identified outcome. The results of this survey will be used to guide the WG's final recommendations.

Provide us with your feedback to improve the quality of care today!

Validation Survey	Time One-time commitment of 10 – 15 minutes	Qualifications • Must be an adult (age of majority or greater). • Interested in sharing your opinions about what really matters when it comes to spinal muscular atrophy. • Be an adult with lived experience of spinal muscular atrophy or a carer to someone with spinal muscular atrophy. • Able to participate in an online survey.
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greater).

- Interested in sharing your opinions about what really matters when it comes to spinal muscular atrophy.
- Be an adult with lived experience of spinal muscular atrophy or a carer to someone with spinal muscular atrophy.
- Able to participate in an online survey.

If you have any patients who would be interested, please provide them with this link to complete the [survey](#).

To learn more, click [here](#) for an informational flyer.



Real-World Air Travel Experiences

Cure SMA has launched a survey capturing real-world air travel experiences — from booking through landing — to show lawmakers how different flying is for wheelchair users with SMA compared to other passengers. Findings will support Cure SMA's advocacy for more accessible air travel policies, including the ultimate goal of allowing passengers to remain in their wheelchair inside the cabin of the plane. Feel free to share this [survey](#) with patients and families.

Lenox Hill Hospital Adult SMA Oral Health Survey

The Adult SMA Multidisciplinary Clinic at Lenox Hill Hospital is working to improve access to high-quality oral health and dental care for adults living with spinal muscular atrophy (SMA).



In collaboration with our adult SMA Patient Advisory Board and Northwell Health dental professionals, we have developed a brief anonymous survey to better understand oral health practices, experiences, and barriers to dental care among adults with SMA.

Who: Adults (18 years and older) with SMA

What: Anonymous Oral Health Survey

When: One-time online survey (approximately 5 minutes to complete)

[Survey Link](#)

This study is **IRB-approved**, and all survey responses are **de-identified and anonymous**.

If you have any questions or experience issues accessing the survey, please contact:

Dr. Aaron Zelikovich

Lenox Hill Hospital

azelikovich@northwell.edu

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



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](#)

Biogen



SMA PIERRE PK Study

This 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.

STELLAR-1 Study

This [study](#) is evaluating the effects of a new investigational drug called salanersen. The study drug salanersen is given once per year and is designed to help the body produce more SMN protein. This study is currently enrolling newborns under the age of 6 weeks who have been diagnosed with SMA, but are not yet showing symptoms. Please contact clinicaltrials@biogen.com for more information.

STELLAR-2 Study

This [study](#) is focused on learning about the safety and effects of salanersen (BIIB115) when given to babies with spinal muscular atrophy (SMA) who were previously treated with onasemnogene abeparvovec. Please contact clinicaltrials@biogen.com for more information.

SOLAR Study

The SOLAR [study](#) is for individuals 15 to 60 years old with spinal muscular atrophy (SMA) and are currently taking risdiplam or who have not yet taken any treatment. This study is evaluating a study drug called salanersen to understand its effects and safety in individuals ages 15 to 60 with SMA. Please contact clinicaltrials@biogen.com for more information. You can also [click here](#) to get started.



Scholar Rock

Scholar Rock announced that it has received approval from the U.S. Food and Drug Administration (FDA) for ISEMBYLD™ (apitegromab-mstn) to treat spinal muscular atrophy (SMA). To learn more, read [here](#).

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.

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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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