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.

Did You Know?

- Approximately 45% of individuals who reported a diagnosis of hip dysplasia between 2020-2024 had Type 1 SMA, which was higher than previous years
- 29% of affected individuals or their caregivers have reported barriers when attempting to schedule an appointment with a healthcare specialist
- 26% of those living with SMA have reported a history of depression
- Since 2021, there has been an increase in the proportion of individuals that reported being treated for depression and/or anxiety within a given year
- To learn more, go check out the **2024 State of SMA**

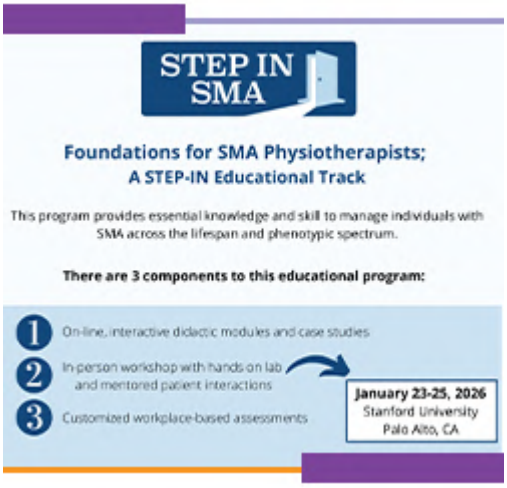
Educational Opportunities

New CME Opportunity Available!

The transition from pediatric to adult care is a critical stage for patients with spinal muscular atrophy (SMA), often marked by challenges in care coordination, psychosocial support, vocational integration, and individualized planning.

In this 30-minute webinar, "**Transitioning to Adult Care in Spinal Muscular Atrophy: How and When**, join Dr. John Brandsema as you explore how to engage patients, caregivers, and providers in seamless pediatric-to-adult spinal muscular atrophy (SMA) care transitions.

This webinar provides .50 CME/CE



Foundations for SMA Physiotherapists; A STEP-IN Education Track

The Foundations track from STEP-IN is a comprehensive, three-part educational program designed to build confidence and competence in physical therapists working with individuals who have SMA:

1.) Online Learning – 17 interactive modules with case studies covering core concepts in SMA physical therapists.

2.) In-Person Workshop – A 2½-day hands-on experience featuring lab sessions, mentored patient interaction, and clinical skill building.

3.) Workplace-Based Assessment – Direct mentorship and evaluation from an experienced SMA PT in the participant's own clinical setting.

The registration fee is $750. To learn more or to apply, visit: **stepinsma.org/foundations**

This initiative is supported by STEP-IN SMA, Cure SMA, THE APNCR network, and additional partner organizations.

For more information on the education Cure SMA provides, head to their **Educational Opportunities** page.

Clinical Resources

Recent Publications

Safety and efficacy of apitegromab in nonambulatory type 2 or type 3 spinal muscular atrophy (SAPPHIRE): a phase 3, double-blind, randomised, placebo-controlled trial

Crawford, Thomas O et al. “Safety and efficacy of apitegromab in nonambulatory type 2 or type 3 spinal muscular atrophy (SAPPHIRE): a phase 3, double-blind, randomised, placebo-controlled trial.” The Lancet. Neurology vol. 24,9 (2025): 727-739. doi:10.1016/S1474-4422(25)00225-X

Risdiplam in Presymptomatic Spinal Muscular Atrophy

Finkel, Richard S et al. “Risdiplam in Presymptomatic Spinal Muscular Atrophy.” The New England journal of medicine vol. 393,7 (2025): 671-682. doi:10.1056/NEJMoa2410120

Presymptomatic Treatment of a Genetic Disease with a Small-Molecule Drug

Sumner, Charlotte J. “Presymptomatic Treatment of a Genetic Disease with a Small-Molecule Drug.” The New England journal of medicine vol. 393,7 (2025): 714-717. doi:10.1056/NEJMe2507195

Spinal Muscular Atrophy Functional Composite Score Revised (SMA-FCR) in Untreated and Nusinersen-Treated Patient Cohorts

Pasternak A, McDermott MP, Montes J, et al. Spinal Muscular Atrophy Functional Composite Score Revised (SMA-FCR) in Untreated and Nusinersen-Treated Patient Cohorts. Neurology. 2025;105(2):e213839. doi:10.1212/WNL.0000000000

Supporting Individuals with SMA Through Legislative Advocacy

Recognizing that state and federal policies impact nearly all aspects of life for individuals with SMA, Cure SMA has developed an **advocacy toolkit** of resources to help SMA advocates, including healthcare providers, to educate their legislators on their needs and goals.

Cure SMA advocacy resources include:

• **SMA Issues Fact Sheets**

• **Advocacy How-To Videos**

• **SMA State Fact Sheets**

• **Advocacy Action Center**

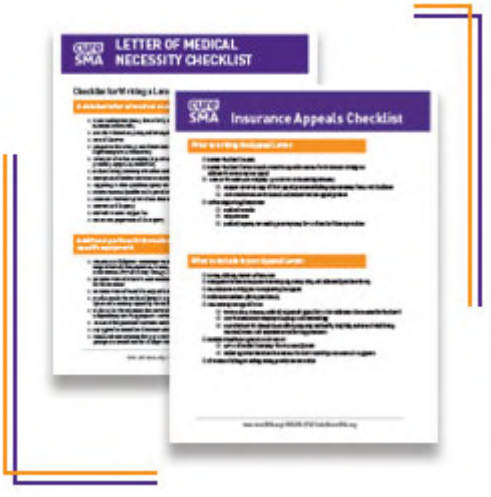
Over 600,000 messages have been sent to state and federal legislators through Cure SMA tools, supporting SMA community priorities like research, caregiving, transportation, housing, and access to healthcare. Healthcare providers can join the effort—become a Cure SMA Advocate at: **https://www.votervoice.net/CureSMA/Register**.

Updated Health Insurance Road Map Now Available – Just in Time for Open Enrollment!

Cure SMA's **Health Insurance Road Map** has been newly updated to help patients, families, and providers navigate the complexities of health insurance with greater clarity and confidence. The resource offers essential tools to simplify challenging insurance topics and promote informed decision-making. The Roadmap includes:

- Clear definitions of commonly used insurance terms

- Guidance for evaluating health plans, including key questions to consider
- Strategies for managing denials and appeals for SMA treatment
- Information on pharma-sponsored patient support and access programs
- Resources for financial assistance



Explore Cure SMA's Insurance Resources

Did you know that Cure SMA has a dedicated website with insurance-related tools for individuals with SMA and healthcare providers?

Our **Insurance Resources** webpage includes:

- The updated Health Insurance Roadmap
- State Specific Resource Guides
- Checklists for Letters of Medical Necessity and Insurance Appeals
- Payer Outreach Resource Letters

We encourage you to take a moment to explore these tools and share with your patients and care teams.

Patient Resources

Newly Diagnosed Support from Cure SMA

Did you know Cure SMA offers a free, comprehensive information packet to anyone newly diagnosed with SMA in the U.S., including our SMA Care Series Booklets on treatment, genetics, daily life, and more. Families also receive a Newly Diagnosed Care Package with age-appropriate toys suggested by other SMA parents.

If your patient hasn't yet connected with us, have them email **communitysupport@curesma.org** with their name and mailing address, and someone will reach out to provide support.

You can also explore our full range of support in the **Community Support Services Booklet**.

Share Your Expertise

Spinal Muscular Atrophy (SMA) & Pregnancy

Researchers at Stanford University School of Medicine Neuromuscular Division are studying pregnancy complications and outcomes in individuals with SMA who were receiving disease modifying treatments. If you know of a patient with SMA that has or is planning to become pregnant and have taken a disease modifying treatment such as nusinersen or risdiplam, please contact the research team at **shsmith@stanford.edu** for more information.

We Need Your Help! Participate in the HCP Treatment Decision Making & Barriers Survey

Cure SMA is seeking U.S. clinicians who treat individuals with SMA to participate in a 15–20-minute research survey to understand treatment decision-making and barriers/capacity challenges that impact a center's ability to prescribe and administer SMA treatment.

Clinicians that have prescribed an FDA-approved disease modifying treatment to at least 1 individual with 5q SMA in the last 5 years and are licensed to practice in the United States are eligible to participate.

If interested, please click **here**.

The data collected will be leveraged by Cure SMA to inform the development of future resources to support the delivery of SMA treatment and care.

Latest News: Clinical Trials & Treatment Approvals

Cure SMA Provides Updated Information with FAQ's Related to Treatment Delays and Next Steps

Cure SMA is working closely with Scholar Rock, Biogen, and the FDA to understand the next steps and possible timelines. As we learn more, Cure SMA will share updates on our **Treatment Delays FAQ page** to answer our community's questions and keep you informed on the latest updates.

We will also be sharing any outreach efforts that we will be taking, including reinforcing the urgent needs and goals of the SMA community to

encourage the FDA to move swiftly to respond once resubmissions occur.

Biogen

Biogen announced that the U.S. Food and Drug Administration (FDA) has delayed the approval with a Complete Response Letter (CRL) for the supplemental New Drug Application (sNDA) for the high-dose regimen of nusinersen (SPINRAZA) for people living with SMA.

The CRL requests updates to the technical information included in the Chemistry Manufacturing and Controls (CMC) module. Importantly, the FDA did not identify any deficiencies in the clinical data for the high-dose regimen.

Biogen plans to resubmit the application promptly using readily available information and is working closely with regulatory authorities worldwide to advance this high-dose option, which was recently approved in Japan and is under review by the European Medicines Agency (EMA) and other global regulators.

While this update is unexpected, the clinical data supporting the high-dose regimen remains strong. Cure SMA will continue to monitor the process and share updates with the community as Biogen works toward approval.

Scholar Rock

Scholar Rock announced that the U.S. Food and Drug Administration (FDA) has delayed the approval with a Complete Response Letter (CRL) for the apitegromab Biologics License Application (BLA) for the treatment of people living with spinal muscular atrophy (SMA).

The CRL is related to observations identified during a routine inspection of Catalent Indiana LLC, a third-party fill-finish facility, and is not

specific to apitegromab. Importantly, the FDA did not cite any other approvability concerns related to apitegromab's efficacy, safety data, or the third-party drug substance manufacturer.

Scholar Rock has stated its intention to resubmit the apitegromab BLA once Catalent Indiana has successfully resolved the FDA's observations. The company believes that the FDA will be able to act expeditiously on the application following resubmission.

While this is disappointing news, the issues identified by the regulator appear to be due to an unrelated manufacturing plant issue rather than a problem with the clinical data or apitegromab itself. Cure SMA will be working with the FDA and Scholar Rock to identify a timeline for the resolution of these issues and will communicate more information to the community soon.

To read Scholar Rock's full community statement, please click **here**.

Novartis

Novartis filed OAV101 IT in the US and expects a regulatory decision in H2. **Learn more**

Genentech

The MARLIN Study is enrolling and is seeking participants. The MARLIN study is an observational study aimed at understanding the fertility journey of men diagnosed with spinal muscular atrophy (SMA) who are actively attempting to conceive or have conceived in the past and are taking or have taken risdiplam (Evrysdi).

This is a fully virtual study: it doesn't require in-person visits, treatments or procedures such as laboratory tests. The study consists of questionnaires to be completed remotely (online).

Learn more about this study at **ISRCTN Clinical Study Registry** or visit the **IRB-approved MARLIN** study website.

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.