



# NEWSLETTER FOR HEALTHCARE PROFESSIONALS

Welcome to the June 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.

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# STATE OF SMA

2025 REPORT

The 2025 State of SMA Report is Now Available!

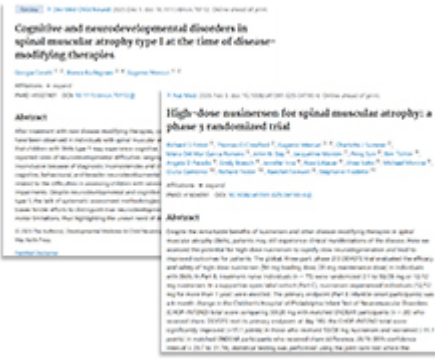
The State of SMA report features insights into the progress and ongoing challenges impacting people with spinal muscular atrophy (SMA) and their families, utilizing data collected by Cure SMA. Previously, the annual report has also been used to advocate for the SMA community by informing the FDA about unmet needs and educating insurers about access barriers.

Key findings in this report include:

- A decline in the number of individuals diagnosed with SMA each year.
- Gaining muscle strength continues to be the greatest reported unmet need among adults living with SMA.
- Adults cite a lack of information on nutrition and appropriate diet types as an unmet need.
- 70% of adults report experiencing incomplete or inadequate care with emergency medicine and preventative care.
- >30% of adults report not being up to date on routine screenings for cervical cancer, colorectal cancer, and prostate cancer.
- 22% of women living with SMA reported a history of pregnancy.
- Caregivers of children with 2 SMN2 copies that received treatment earlier in life reported less trouble swallowing and less soft/weak voice.
- Comorbidities including chronic pain, osteopenia, and osteoporosis were frequently reported among adults living with SMA.
- 22% of those living with SMA reported a history of a depression diagnosis; 34% reported a history of anxiety diagnosis.
- Mortality has declined by nearly 60% over the past ten years.

To learn more, go check out the [2025 State of SMA Report](#)

[Clinical Resources](#)



## [Insights from Wise Warriors: A Needs Assessment and Preventive Parenting Program Recommendations From Parents of Children with Spinal Muscular Atrophy](#)

Andrade IJ, Kim JJ. Insights From Wise Warriors: A Needs Assessment and Preventive Parenting Program Recommendations From Parents of Children With Spinal Muscular Atrophy. *Journal of Child Neurology*. 2026;0(0). doi:10.1177/08830738261447163

## [The phenotypic spectrum and genetic determinants of severe spinal muscular atrophy in individuals with a single SMN2 copy: an international retrospective observational study](#)

Cicala G, Capasso A, Villa M, Coratti G, Arpaia C, Agosto C, Corti S, Ricci F, Bruno C, Matesanz S, Gross B, Mendoza DG, Kuntz N, Kirschner J, Ziegler A, Servais L, Asselman FL, van der Pol L, Castiglioni C, Nascimientto A, Tizzano EF, Mendonça RH, Zanoteli E, Munot P, Scoto M, Finkel R, Pane M, Tiziano FD, Mercuri E; ITASMAc group; International SMA group. *EClinicalMedicine*. 2026 May 7;95:103931. doi: 10.1016/j.eclinm.2026.103931. eCollection 2026 May. PMID: 42163975 Free PMC Article

## [Navigating new motor function trajectories: Consensus recommendations for assessment in the era of newborn screening and early treatment in SMA](#)

Krosschell KJ, Dunaway Young S, Maczek E, et al. Navigating new motor function trajectories: Consensus recommendations for assessment in the era of newborn screening and early treatment in SMA. *Journal of Neuromuscular Diseases*. 2026;0(0). doi:10.1177/22143602261433638

## [Impact of Early Intervention on Motor Milestone Achievement in Spinal Muscular Atrophy: Insights from Cure SMA Survey Data](#)

Lawrence, K.A.K., Belter, L., Curry, M. et

al. Impact of Early Intervention on Motor Milestone Achievement in Spinal Muscular Atrophy: Insights from Cure SMA Survey Data. *Neurol Ther* (2026).  
doi.org/10.1007/s40120-026-00943-x

**Clinical evaluation of ThecaFlex DRx, a novel implantable catheter-port for intrathecal nusinersen delivery in spinal muscular atrophy: Initial results from the PIERRE-IDE study**

Mesina-Estarrón I, Abruzzo M, Gomes FC, Thaxton M, Yengo-Kahn A, Stone S, Northam W, Snyder B, Cahill P, Bauer D, Muhonen M.J  
*Neuromuscul Dis*. 2026 Mar

13:22143602261432303. doi:

10.1177/22143602261432303. Online ahead of print.

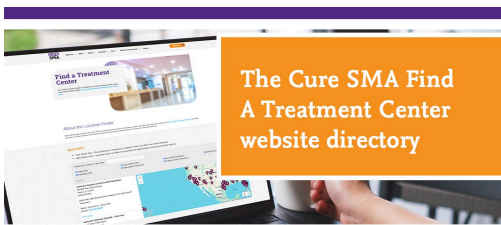
**Adult SMA REACH: A UK clinical network and real-world data collection study for adults living with spinal muscular atrophy**

Page J, Karkkainen E, Segovia S, Fitzsimmons S, Verdu-Diaz J, Murphy L, Benesperi G, Carver A, Simms L, Tanner S, Tasca G, Moat D, Michell-Sodhi J, Lofra RM, Marini-Bettolo C;  
Adult SMA REACH clinical network. *J*

*Neuromuscul Dis*. 2026 Mar

13:22143602261433509. doi:

10.1177/22143602261433509. Online ahead of print.



TAKE A FEW MINUTES TODAY TO REVIEW OR UPDATE YOUR LISTING [WWW.CURESMA.ORG/FIND-A-TREATMENT-CENTER](http://WWW.CURESMA.ORG/FIND-A-TREATMENT-CENTER)

## What is the Cure SMA Find a Treatment Center Tool?

The Cure SMA Find a Treatment Center website is an online directory used by patients and caregivers to locate sites offering spinal muscular atrophy (SMA) treatment and care. Opt-in and update requests may be submitted via a short survey that is also accessible on the Find a Treatment Center page.

Why Opt In?

- Connect individuals and families with SMA specialists.
- Support access to SMA treatment by ensuring your center's information accurately reflects the care available at your site.

### Check Your Center's Listing

- If your center is not currently listed, please add your center to help patients and families find and access care.
- If your center is already listed, please review and update your listing to ensure the information is accurate and current.

To access the website and review your site, click [here](#).

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## Educational Opportunities



### STEP-IN SMA

STEP-IN SMA offers educational resources for physiotherapists who provide clinical care or research for SMA patients. The next educational opportunity in their series is on June 16 in partnership with Cure SMA.

This presentation is a sneak peek of Rehab Focus poster presentations from the Cure SMA Annual Research & Clinical Care Meeting. To register or to learn more about the STEP-IN SMA professional development series, click [here](#).

# Annual SMA Research & Clinical Care Meeting

The 2026 Annual SMA Research & Clinical Care Meeting will take place June 24–26, 2026, in Orlando, Florida, at Disney’s BoardWalk Inn. This meeting brings together researchers from academia, government, and industry alongside multidisciplinary healthcare providers to share the latest advances in SMA research, treatment, and clinical care.

Held in parallel with the Annual SMA Conference, these meetings foster collaboration, education, and connection across the SMA community.

For more information, click [here](#).

For questions, please contact [SMARCC@curesma.org](mailto:SMARCC@curesma.org).

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## [Share Your Expertise](#)

### Request for Clinician Input on Real World Data Analysis Methodology

Cure SMA is seeking a clinician with expertise in motor function assessments (e.g., HFMSE, RULM, etc.) to provide input on the methodological approach for a real-world data analysis. The project aims to characterize demographic and clinical features of teens and adults with SMA, stratified by post-treatment motor function assessment trajectories using the Cure SMA Clinical Data Registry.

The time commitment is less than one hour, and there is an opportunity to be included as an author on an abstract if interested. This project

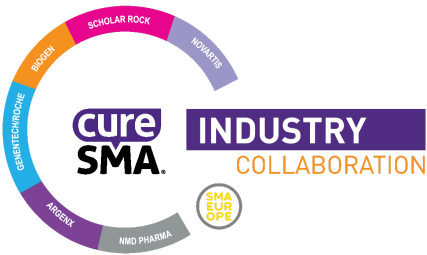


is funded by the 2026 SMA Industry Collaboration (SMA-IC).

To learn more, please email [sarah.whitmire@curesma.org](mailto:sarah.whitmire@curesma.org)

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## Cure SMA Latest News



### Cure SMA Shares Research Insights with Scientists and Clinicians at Spring 2026 Conferences

The SMA Industry Collaboration (SMA-IC) is a partnership that unites Cure SMA, other nonprofit organizations, and pharmaceutical partners to achieve the common goals of improving treatment outcomes and quality of life for individuals with SMA and their families. By sharing data and expertise, SMA-IC members work together to address scientific, clinical, and regulatory challenges. At research conferences this spring, Cure SMA presented SMA-IC research findings based on the real-world experiences of the SMA community. Click [here](#) to learn more.



### SMA Added as a Priority Research Topic in DOD PRMRP

Earlier this year, Congress [designated](#) SMA as a priority research topic within the U.S. Department of Defense's Peer Reviewed Medical Research Program (PRMRP), making SMA one of only 51 conditions eligible for this competitive federal research funding program. This designation creates new opportunities to advance promising SMA research and therapies. The

designation was the result of a nationwide advocacy campaign, where Cure SMA mobilized SMA advocates in all 50 states through letters, online actions, and in-person meetings with lawmakers to support SMA's inclusion. The addition of SMA to the FY26 [eBRAP Program Portfolio](#) highlights the impact of coordinated grassroots advocacy and federal engagement by the SMA community. The Department of Defense plans to host upcoming webinars related to these Congressionally Directed Medical Research Program opportunities. Those details will be posted on the DOD website: <https://cdmrp.health.mil/>.

## Cure SMA Policy Brief

Cure SMA has published a new policy brief to educate policymakers about the importance of Medicaid home care in promoting health and independence for people with SMA. Many individuals with SMA rely on Medicaid-funded caregiving for complex daily needs, including respiratory care, nutrition support, personal hygiene, and safe mobility transfers. Cure SMA developed the brief in response to funding [cuts](#) and recent [statements](#) that mischaracterize these essential services as unnecessary. Healthcare providers and advocates are encouraged to read and share the brief [here](#).

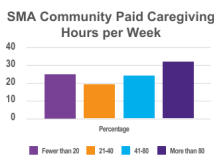
### **Cure SMA** PROTECT MEDICAID HOME CARE FOR PEOPLE WITH SPINAL MUSCULAR ATROPHY

*Self-Directed Care and Paid Family Caregiving are Essential to Independence, Health, and Work*

Individuals with spinal muscular atrophy (SMA), a rare neuromuscular disease, rely on Medicaid-funded home care to maintain their health, independence, and ability to live in and contribute to their communities. Bipartisan policies expanding Medicaid home and community-based services (HCBS) have improved health outcomes, reduced institutionalization, and enabled participation in school and work. Federal and state proposals that reduce access to or limit eligibility for Medicaid home care, including self-directed care and paid family caregiving, would have serious consequences for individuals with SMA and their families.

#### SPINAL MUSCULAR ATROPHY AND THE SMA COMMUNITY'S CAREGIVING NEEDS

SMA is a neurodegenerative disease that causes severe muscle weakness and loss of motor function. As a result, most individuals with SMA rely on one or more caregivers to assist with nearly all activities of daily living, including eating, bathing, dressing, mobility, and respiratory care. Many individuals utilize paid caregivers, such as private duty nurses (PDNs) or personal care attendants (PCAs), to meet these needs, including 96% who receive more than 41 hours of paid care each week. One adult with SMA shared, "I am no longer ambulatory. I have limited upper body strength. I need assistance with many activities, basically any part of my day that requires me to do something using the major muscles of my body." Another adult with SMA said, "Paid caregivers allowed me the freedom to work in person, opening up opportunities for socialization that would not be possible were I stuck in the house."



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



## Biogen

The FDA has approved the High Dose regimen of SPINRAZA (nusinersen) for the treatment of spinal muscular atrophy (SMA), with broad label approval for all those living with SMA. The High Dose regimen is designed to further enhance

SMN protein levels beyond what the existing regimen provides, offering new possibilities for improved outcomes and individualized care. Cure SMA is working closely with Biogen and healthcare providers to support timely education and access to this new dosing option. To view Biogen's latest community statement on this approval, click [here](#).

The STELLAR-1 Study is a Phase III clinical trial evaluating the effects of a new investigational drug called salanersen. The study drug salanersen is administered once per year and works by helping the body produce more SMN protein. The STELLAR-1 study will enroll newborns who have been diagnosed with spinal muscular atrophy (SMA) but are not yet showing symptoms. [Explore the Study](#)



Researchers at Stanford University School of Medicine are studying pregnancy complications and outcomes in individuals with SMA who were receiving disease modifying treatments. This study would be a good fit for those who have:

- A genetic confirmation and diagnosis of SMA.
- Been, are currently, or plan to become pregnant.
- Taken a disease modifying treatment such as nusinesen or risdiplam within 14.5 months of last menstrual period before pregnancy, or at any point in time during pregnancy.

#### RESEARCH DESCRIPTION & PURPOSE

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

#### WOULD THE STUDY BE A GOOD FIT FOR ME?

This study would be a good fit for you if:

- You have genetic confirmation and diagnosis of spinal muscular atrophy (SMA)
- You have been, are currently, or plan to become pregnant
- You have taken disease modifying treatment such as nusinesen or risdiplam within 14.5 months of last menstrual period before pregnancy, or at any point in time during pregnancy

#### WHAT WOULD HAPPEN IF I TOOK PART IN THE STUDY?

- Researchers will collect data from your electronic medical record retrospectively up to 10 years in the past, and up to 10 years following enrollment in the study
- There will be no required on-site visits

#### CONTACT INFORMATION

To take part in this study or get more information:

CALL: 650-272-8141

EMAIL: [shsmith@stanford.edu](mailto:shsmith@stanford.edu)

The principal researcher for this study is [Jacinda Sampson MD, PhD](#)

For general information about participant rights, contact 1-866-680-2906.



Researchers will collect data from your electronic medical record retrospectively up to 10 years in the past, and up to 10 years following enrollment in the study. There will be no required on-site visits. To take part in this study or get more information:

**Call: 650-272-8141**



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](#)



## Scholar Rock

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](mailto:clinicaltrials@scholarrock.com) or click [here](#) for more information.

Additionally, Scholar Rock shared that the U.S. Food and Drug Administration (FDA) has accepted its Biologics License Application (BLA) for apitegromab, an investigational treatment for spinal muscular atrophy (SMA), with a Prescription Drug User Fee Act (PDUFA) action date of September 30, 2026. Learn more about Scholar Rock's recent announcement [here](#).



## Genentech

Genentech announced the discontinuation of Emugrobart (an investigational anti-myostatin antibody, also known as GYM329) into Phase III development for spinal muscular atrophy (SMA). The decision follows a comprehensive review of

data from Part 1 of the MANATEE (NCT05115110) study, a global Phase II/III trial evaluating emugrobart in combination with risdiplam. Results showed that emugrobart did not consistently deliver improvements in muscle growth and motor function compared to treatment with risdiplam alone.

Cure SMA, along with Genentech, extends our sincerest thanks to the individuals and families who took part in the phase 1 trial of emugrobart (MANATEE). [Learn More »](#)

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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.