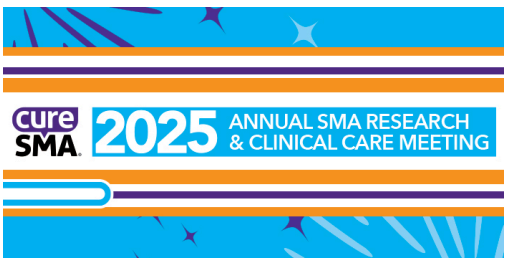
NEWSLETTER FOR HEALTHCARE PROFESSIONALS

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.

Did You Know?

- 43% of children and 26% of adults living with SMA travel over 60 miles for their SMA-related care. (2024 State of SMA)
- 56% of adults living with SMA had issues accessing clinicians with SMA experience. (2024 State of SMA)
- The **Cure SMA Find a Treatment Center Tool** helps individuals living with SMA and their caregivers locate treatment and medical care within local communities.
    - If your center is listed, you can update or remove outdated information.
    - If your center is not included, add your details today!

Educational Opportunities



Annual SMA Research & Clinical Care Meeting is Almost Here

Are you attending our Annual SMA Research & Clinical Care Meeting? **Click here** to find up-to-date information on our agenda, CME information, FAQ, and more!

Explore CME and non-CME education on SMA including:

- STEP-IN Professional Development for PTs
- Stanford Annual Neuromuscular Conference enduring content
- Roundtable Discussions on the latest SMA Update in Best Practices

Visit our **website** to stay informed!

Clinical Resources


Research article

Patient-reported assessment of bulbar function in spinal muscular atrophy (SMA): Validation of a self-report scale

Journal of Neuromuscular Diseases

Maureen A Lefton-Greif, Yong Zeng, Thomas O Cr

Abstract

Keywords

Introduction

Neurol Ther (2025) 14:1083–1092

ORIGINAL RESEARCH

SMA Community Priorities for Future Drug Therapies: Cross-Sectional Survey Findings Representing 410 Adults with SMA

Ilse S. Peterson · Ramaa Chitale · Mary A. Curry · Lisa T. Belter

Received: February 24, 2025 / Accepted: April 11, 2025 / Published online: May 3, 2025

ABSTRACT

Introduction: Despite advances in the therapeutic landscape for spinal muscular atrophy (SMA), unmet needs for those with this condition persist. This study seeks to characterize unmet needs that adults with SMA hope future therapies will address and explores associations between reported needs and health status and demographic characteristics.

Methods: Close-ended questions from 2021 to 2023 Cure SMA Community Update Survey data were used to assess the importance of needs related to muscle and motor function, lung function and bulbar function, and general functioning. Data was stratified by SMA type and mobility status, and Fisher's exact tests were used to assess for statistically significant differences based on these characteristics. Variations in reported needs were further explored with regressions controlling for sex, education, maximum mobility, and drug treatment status.

Results: The sample included 410 adults who answered questions on unmet needs. Most had type 2 or type 3 SMA (48% and 45%, respectively). Gaining muscle strength was the most frequently reported unmet need, and followed by improving daily functioning, achieving new motor function, and stabilizing motor function. Stratifications and regressions identified statistically significant differences in treatment needs based on health status and demographic characteristics ($p<0.05$). Most notably, people with more severe types of SMA and lower mobility were more likely to report items related to lung function and bulbar function as important.

Conclusion: This research highlights treatment priorities for adults with SMA.

Supplementary Information The online version contains supplementary material available at https://doi.org/10.1007/s40120-025-00753-7.

Keywords: Spinal muscular atrophy; Unmet needs; New treatments; Patient-focused drug development

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Recent Publications

SMA Community Priorities for Future Drug Therapies: Cross-Sectional Survey Findings Representing 410 Adults with SMA

Peterson IS, Chitale R, Curry MA, Belter LT. SMA Community Priorities for Future Drug Therapies: Cross-Sectional Survey Findings Representing 410 Adults with SMA. Neurol Ther. 2025 Jun;14(3):1083-1092. doi: 10.1007/s40120-025-00753-7. Epub 2025 May 3. PMID: 40317455; PMCID: PMC12089628.

Patient-reported assessment of bulbar function in spinal muscular atrophy (SMA): Validation of a self-report scale

Lefton-Greif MA, Belter L, Jarecki J, Schroth M, Zeng Y, Crawford TO, Eaton C. Patient-reported assessment of bulbar function in spinal muscular atrophy (SMA): Validation of a self-report scale. J Neuromuscul Dis. 2025 Mar 20:22143602251325741. doi: 10.1177/22143602251325741. Epub ahead of print. PMID: 40111903

2024 State of SMA Report

The 4th annual Cure SMA State of SMA report is now available. This annual report presents a snapshot of the quickly changing landscape of SMA.

Some of the key findings presented in the 2024 State of SMA report include:

- 51% of the SMA community were adults
- 89% of adults reported gaining muscle strength as their greatest unmet need
- 23% of women living with SMA reported a history of ever being pregnant
- The rates of osteoporosis and chronic pain were higher in the adult SMA community than the U.S. general population
- The average time from diagnosis to first treatment was 18 days for individuals diagnosed in 2024
- The mortality rate of SMA has dropped nearly 80% in the last 10 years

For more insights on the SMA community, check out the **Cure SMA 4th Annual State of SMA Report**

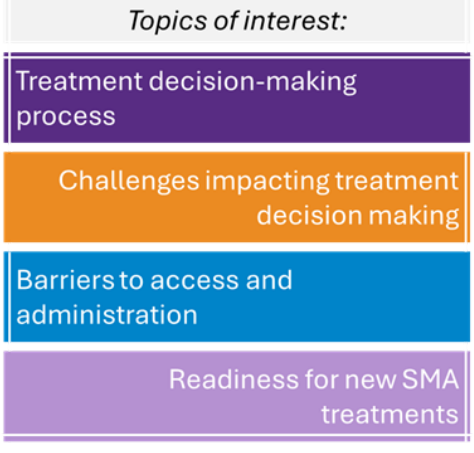
Cure SMA Update

Celebrating Dr. Mary Schroth's Remarkable Legacy

After seven years as Cure SMA's Chief Medical Officer—and decades of unwavering dedication to the SMA community—Dr. Mary Schroth is retiring. Dr. Schroth has been a champion of early diagnosis, newborn screening, and improved clinical care for those with SMA. Beyond her professional contributions, Dr. Schroth's deep compassion and tireless advocacy have left a lasting mark on every person she's served. We thank her and wish her the very best in retirement.

Learn More »

Share Your Expertise



Interested in advising on a Cure SMA project?

We're looking for clinicians who prescribe SMA treatment or those involved in SMA treatment care coordination to collaborate on a project to survey healthcare providers with the goal of understanding treatment decision-making and barriers and capacity challenges that impact a center's ability to prescribe and administer SMA treatment.

There are opportunities for authorship on abstracts and a manuscript if authorship criteria are met.

If interested, more information can be found here: **https://go.curesma.org/e/978203/Barriers-Project-Interest-Form/918nr/1028029108/h/zjKe7ZhVQY4reSB-chW05l7wMgrfh3Gl9k3u8CGOVNo**

*Please note that this is a project funded by the Cure SMA Industry Collaboration. More information can be found **here**.*

Currently Recruiting: Exploration of the Diagnostic Journey of Adult Onset SMA

Cure SMA has developed a short survey to explore the diagnostic journey and unmet needs of adults living with Type 4 SMA. The survey is designed to evaluate gaps in existing research such as time to diagnosis and treatment, comorbidities (prior to and after SMA diagnosis), and ongoing disease management. To help Cure SMA reach more individuals living with Type 4 SMA, please share this **IRB-approved flyer** with your patients as an opportunity for them to share their experiences with Cure SMA. This survey is only intended for those living in the United States, including Puerto Rico.

Patient Resources

Check out our Living Unlimited Series Booklets

Cure SMA's *Living Unlimited* series comes from the desire to talk about topics related to daily life when you or a loved one has SMA.

The first booklet is designed for parents of newly diagnosed children and covers mental health, coping with and overcoming challenges, how to support your child, advice from an adult with SMA, and so much more.

The second booklet is for Adults with SMA and covers topics like travel, treatment access for SMA and primary care, going to school and work, laws and rights that you should know, and more.

To access booklets, **click here**.

To request a hard copy of these booklets, please email

communitysupport@curesma.org.

Understanding Gene Therapy

Cure SMA is committed to providing individuals living with SMA and their caregivers with resources for learning about treatments to aid in their decision-making. As part of this effort, we're pleased to share a new video explaining the mechanism of action (MOA) of gene therapy. While not drug-specific, the video offers a helpful introduction to how gene therapy works in the context of SMA.

To watch the video, **click here**.

This video was created with support from Presenting Sponsors Novartis and Ultragenyx.

Latest News: Clinical Trials & Treatment Approvals

NMD Pharma

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 about this clinical trial at **Study Details | Safety and Efficacy of NMD670 in Ambulatory Adult Patients With Type 3 Spinal Muscular Atrophy | ClinicalTrials.gov.**

Study flyer also available: **https://go.curesma.org/e/978203/Type-3-Phase-2-Study-Flyer-pdf/918pc/1028029108/h/zjKe7ZhVQY4reSB-chW05I7wMgrfh3GI9k3u8CGOVNo**

Scholar Rock

Scholar Rock announced that the U.S. Food and Drug Administration (FDA) has accepted its Biologics License Application (BLA) for apitegromab and granted priority review, setting a Prescription Drug User Fee Act (PDUFA) target action date of September 22, 2025. The company also launched an Expanded Access Program (EAP) in the U.S., with plans to initiate similar programs in Europe. Apitegromab is a muscle-targeted therapy intended to deliver clinically meaningful improvements in motor function for individuals with spinal muscular atrophy (SMA) who are receiving SMN-targeted treatments.

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.