STATE OF SMA

2025 REPORT

Publication Date: May 13, 2026

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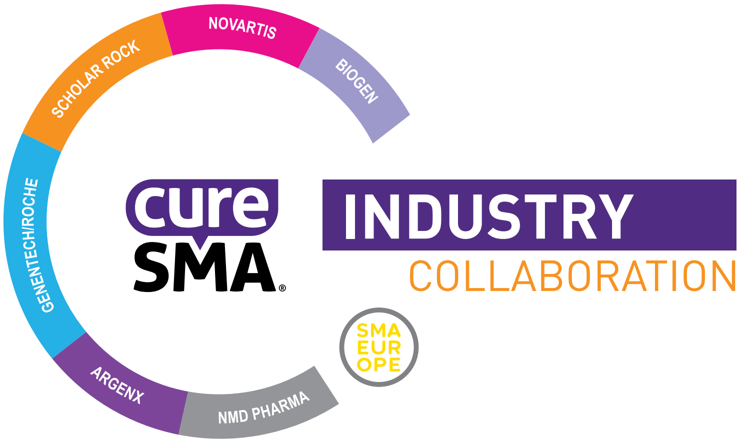
ACKNOWLEDGMENTS

Cure SMA is thankful to all individuals with spinal muscular atrophy (SMA) and their families who have generously shared their data. Their willingness to provide details about how SMA impacts their families and daily lives allows us to advance the understanding of this disease and lays the foundation for continued progress on behalf of our community.

Cure SMA is grateful for the support and funding provided by the Cure SMA Industry Collaboration (SMA-IC) for research initiatives, including this report.

THE CURE SMA INDUSTRY COLLABORATION



The Cure SMA Industry Collaboration (SMA-IC) was established in 2016 to leverage the experience, expertise, and resources of pharmaceutical and biotechnology companies, as well as other nonprofit organizations involved in the development of spinal muscular atrophy (SMA) therapeutics to more effectively address a range of scientific, clinical, and regulatory challenges. Funding for the research included in the State of SMA report was provided by the 2025 SMA-IC; members included Cure SMA, Biogen, Novartis, Scholar Rock, Genentech/Roche Pharmaceuticals, argenx, NMD Pharma, and SMA Europe.

ADDITIONAL ACKNOWLEDGMENTS

- Cure SMA is grateful to the SMA Care Center Network for their commitment to improving care for people with SMA and contributing consented patient data to the Clinical Data Registry.
- Thank you to the clinicians connected to Cure SMA who continue to provide high quality care to individuals with SMA, participate in community initiatives, and lend clinical expertise to projects that fill critical knowledge gaps and advance SMA research.
- Funding for the Cure SMA Care Center Network and Adult & Pediatric Neuromuscular Clinical Research Network (APNCR) has been provided in part by the Erin Trainor Memorial Fund and the Tyler William Orr Memorial Fund.
- Current funding for the SMA Care, Outcomes, and Reported Experiences (CORE) Survey has been provided by Biogen and Novartis.

DEAR CURE SMA COMMUNITY:

We are beyond excited to share with all of you our fifth annual State of SMA report. When we developed our first State of SMA five years ago, we had the goal of sharing a few highlights from the data we had been collecting at Cure SMA. Fast forward five years and we could not be more proud to continue to develop what has now become a valuable data resource for families, healthcare providers, researchers, industry partners, payers and regulators. In the past year alone, the State of SMA report has been used to **educate the FDA** on the ongoing unmet needs of the SMA community, **support new SMA treatment approvals**, and **educate insurers on access barriers of the SMA community.**

To date, Cure SMA has connected with over 11,000 individuals with SMA and their families worldwide and nearly 70% of the U.S. SMA community. Throughout the year, we collect a variety of outcomes from our community members through online surveys, focus groups, and clinical data registries. We are excited to see participation in our various data collection efforts from adults living with SMA increase every year. 2025 marked another year of additional treatment options for the community (see "SMA Treatments in the U.S." section). We are excited to introduce an additional clinical registry that Cure SMA sponsors, the Adult & Pediatric Neuromuscular Clinic Research Network (APNCR), to all of you (see the "Cure SMA Resources" section). Through all of these data sources we aim to capture the patient voice and evaluate the changing landscape of SMA, so Cure SMA can continue to advocate and educate on the ongoing unmet needs of the community.

The data collected by Cure SMA is not only presented in this report. In 2025 alone, our data was published in two peer-reviewed scientific journals and presented in eight poster presentations at clinical research conferences. These publications were made possible because of the SMA community and your willingness to share your stories, experiences, hopes, and needs with Cure SMA.

Every participant counts and lends insight into further educating the community on the ongoing unmet needs and supporting new drug approvals. Thank you all for your continued commitment to Cure SMA.

Sincerely,

Lisa Belter, MPH
Vice President,
Data Analytics

Sarah Whitmire, MS
Director,
Data Analytics

Erin Welsh, MPH
Specialist,
Data Analytics

ABOUT THIS REPORT

This report is based on internal data from Cure SMA data sources and output from the SMA Model, which Cure SMA created to estimate demographic and clinical characteristics of individuals with SMA in the United States.

Both the patient-reported data and the clinician-reported data are presented in this report. In some of our analyses, we have combined the data sources. Even though data comes from multiple sources and perspectives, previous analyses have shown high reliability between the patient-reported and clinician reported data.[1]

The data in this report includes individuals who are:

- Diagnosed (both self-reported and/or clinically confirmed) with 5q SMA
- Included in one or more of our databases as of December 31, 2025
- Residents of the U.S.

The data in the report describes individuals as:

- Children (ages 0-12 years)
- Teens (ages 13-17 years)
- Pediatric (ages 0-17 years) and
- Adults (ages 18 years and older)

If it was reported that an individual with SMA was identified by screening, they must have a diagnosis date after 1994 for prenatal screening and a diagnosis date after 2015 for newborn screening.

The Cure SMA databases are dynamic and rely on the community's participation to share up to date information on the lived experiences of SMA. This report reflects the most current information housed in Cure SMA's databases, but there may be delays in collecting new information, and differences in numbers presented in this report and in previous and future State of SMA reports may be observed.

Additionally, all the analyses within this report are descriptively showing what we see in the data, but they do not adjust for factors that may bias the results. Caution should be taken when interpreting the results.

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1. Belter L, et al. Comparing Spinal Muscular Atrophy (SMA) outcomes between a patient-reported membership database and a clinical data registry. Poster presented at the American Academy of Neurology in Boston, MA in April 2023.

DEFINITIONS

Here are a few terms that you will see as you read this report:

STATISTICAL TERMS

Mean:
The average value in a dataset.

Median:
The middle value in a set of numbers.

Mortality Rate Per Year:
The frequency of the occurrence of death within a subgroup of individuals within a one-year period.

Qualitative Data:
Non-numerical or descriptive data, such as information gathered from interviews, focus groups, etc.[1]

SMA Birth Prevalence:
The proportion of individuals born in a specific time period that have SMA.

SMA Incidence:
The number of individuals who are diagnosed with SMA per year.

SMA Prevalence:
The number of individuals that are currently living with SMA.

Standard Deviation (SD):
A measure of how far away the data points are from the average value in a dataset.[2]

TREATMENT TERMS

Combination/Concurrent Treatment:
Initiation of an additional therapy (or "add on") while continuing treatment of the original therapy. This includes initiating Spinraza or Evrysdi after initiation of Zolgensma.[3]

Effectiveness:
How well a treatment works in people who use it after it has been approved by regulatory bodies such as the FDA.[4]

Efficacy:
How well a treatment works in a controlled setting, such as a clinical trial.[5]

Sequential Treatment:
Discontinuing a treatment at or shortly after administration of a different treatment.[3]

CLINICAL TERMS

Clinician Reported Data:
Data that is gathered from clinician reported medical records about a patient seeking care, commonly through medical records, case report forms, or surveys.

Durable Medical Equipment (DME):
Medical devices or supplies that are prescribed for repeated long-term use, such as a wheelchair or BPAP machine.

Patient- or Caregiver- Reported Data:
Data that is gathered directly from a patient or caregiver, commonly through online surveys and questionnaires.

Social Determinants Of Health (SDOH):
The conditions in which people are born, grow, live, work, and play that affect their health and well-being.

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1. Qualitative Data. NNLM. https://www.nnlm.gov/guides/data-glossary/qualitative-data.
2. Standard Deviation - an overview | ScienceDirect Topics. https://www.sciencedirect.com/topics/mathematics/standard-deviation.
3. Proud CM, 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).
4. Effectiveness. The Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard. (2025, September 24). https://mrctcenter.org/glossaryterm/effectiveness/.
5. Spahn J. Clinical trial efficacy: what does it really tell you? J Allergy Clin Immunol. 2003 Nov;112(5 Suppl):S102-6.

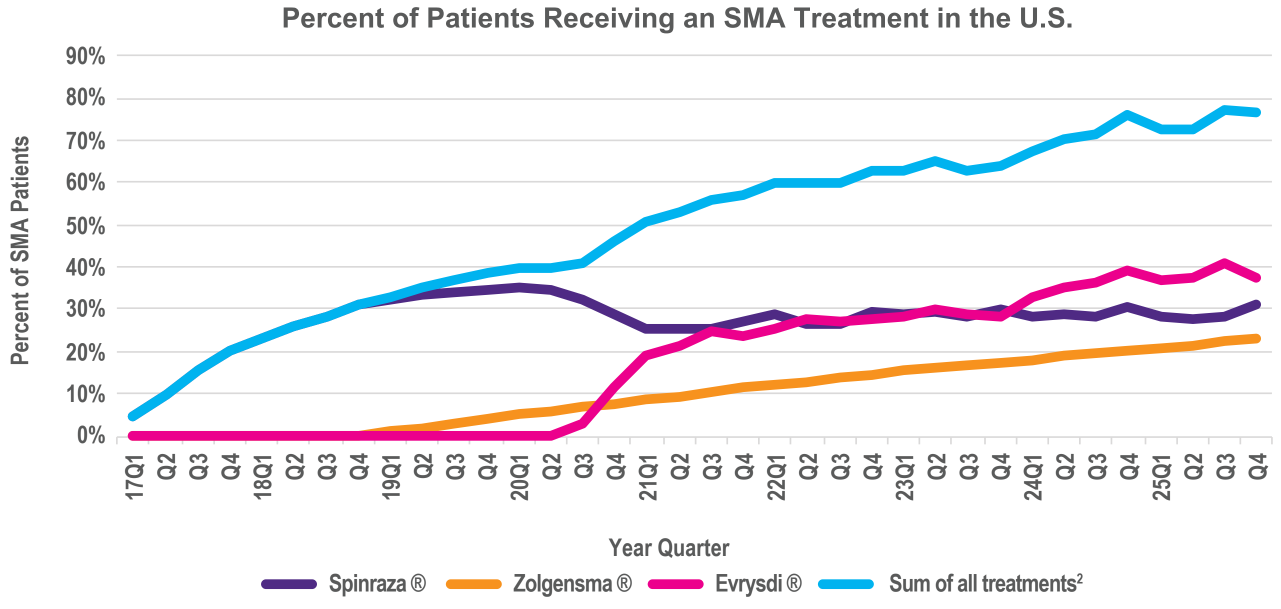
SMA TREATMENTS IN THE U.S.

The SMA Drug Pipeline is how Cure SMA evaluates the success of our SMA research program. It identifies the major drug development programs and tracks their progress from basic research through FDA approval and beyond.
Please visit curesma.org/sma-drug-pipeline to learn more.

SMA Approved Treatments and Investigational Therapies[1]

Organization/Drug Name	Mechanism of Action	Age Coverage	SMA Type Coverage	Stage of Approval		
				FDA Submission	FDA Approval Date	To Patients
Biogen – Low Dose Regimen SPINRAZA®	Modulation of *SMN2*	All ages	All SMA Types		12/23/2016	
Novartis - ZOLGENSMA® (IV)	Gene Therapy	Individuals < 2 years old	All SMA Types		5/24/2019	
Genentech-Roche - EVRYSDI® (oral solution)	Modulation of *SMN2*	All ages	All SMA Types		8/7/2020	
Genentech-Roche - EVRYSDI® (tablet formulation)	Modulation of *SMN2*	Individuals ≥ 2 years old	All SMA Types		2/12/2025	
Novartis - ITVISMA® (IT)	Gene Therapy	Individuals ≥ 2 years old	All SMA Types		11/24/2025	
Biogen – High Dose Regimen SPINRAZA®	Modulation of *SMN2*	All ages	All SMA Types		03/30/2026	
Scholar Rock – apitegromab	Muscle-directed therapy					

Approximately 77% of individuals with SMA in the U.S. were utilizing an FDA-approved treatment in Q4 2025[2-4]



1. Treatments shown in pipeline have been filed with the FDA or approved as of print of this report.
2. The sum of all treatments data is accounting for an estimated 16% of concurrent treatment use.
3. Anyone treated with Zolgensma was categorized as "currently on treatment" for all quarters following treatment.
4. Data here is presented by standard calendar quarters: January, February, and March (Q1) April, May, and June (Q2) July, August, and September (Q3) October, November, and December (Q4).

Analysis Notes:

- Data from https://www.curesma.org/cure-sma-clinical-trials/ and published industry earnings reports from Biogen, Roche, and Novartis. These are estimates and may over or underrepresent treatment utilization.

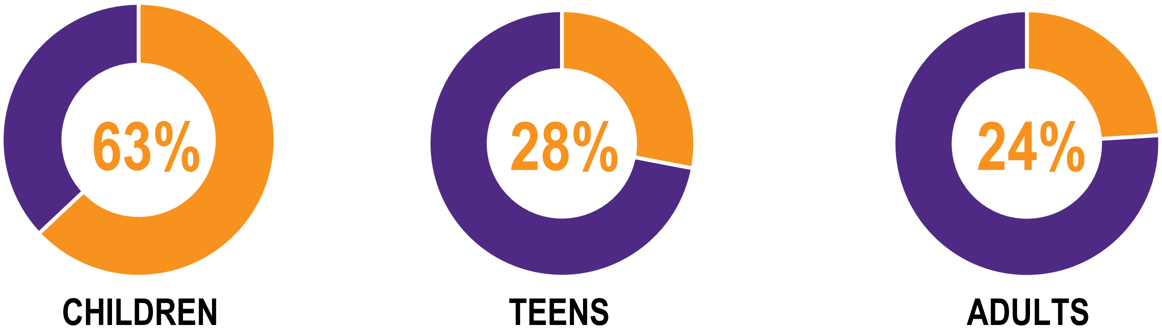
2025 KEY FINDINGS IN CURE SMA DATA

An estimated **9,000-9,500** individuals are living with SMA in the U.S., with **64%** being adults

Representation of teens and adults has increased over the last 10 years, highlighting more individuals with SMA living into adulthood

The mortality rate of SMA has dropped nearly **60%** in the last 10 years

PERCENT OF INDIVIDUALS WITH SMA WHO ARE CURRENTLY AMBULATORY



SMA IS BEING DIAGNOSED EARLIER

MEDIAN AGE AT SMA DIAGNOSIS

2017: 1.2 years | 2020: 248 days | 2025: 7 days

AVERAGE TIME FROM DIAGNOSIS TO FIRST TREATMENT IS DECREASING

DATA AMONG THOSE DIAGNOSED IN A GIVEN YEAR

2017: 196 days | 2020: 63 days | 2025: 28 days

INDIVIDUALS CURRENTLY ON TREATMENT

96% Children | 90% Teens | 78% Adults

INDIVIDUALS UTILIZING 2+ TREATMENTS

38% Children | 33% Teens | 29% Adults

72% of adults currently on treatment worry that their treatment may stop working

UNMET NEEDS STILL REMAIN WITHIN THE SMA COMMUNITY

Approximately half of children and adults have received an insurance denial for their SMA treatment

56% of single adults worry about getting married due to the impact on public benefits

Individuals reporting the greatest difficulty with swallowing were children 6-12 years old and teens 13-17 years old

49% of durable medical equipment (DME) users have received an insurance denial for their equipment

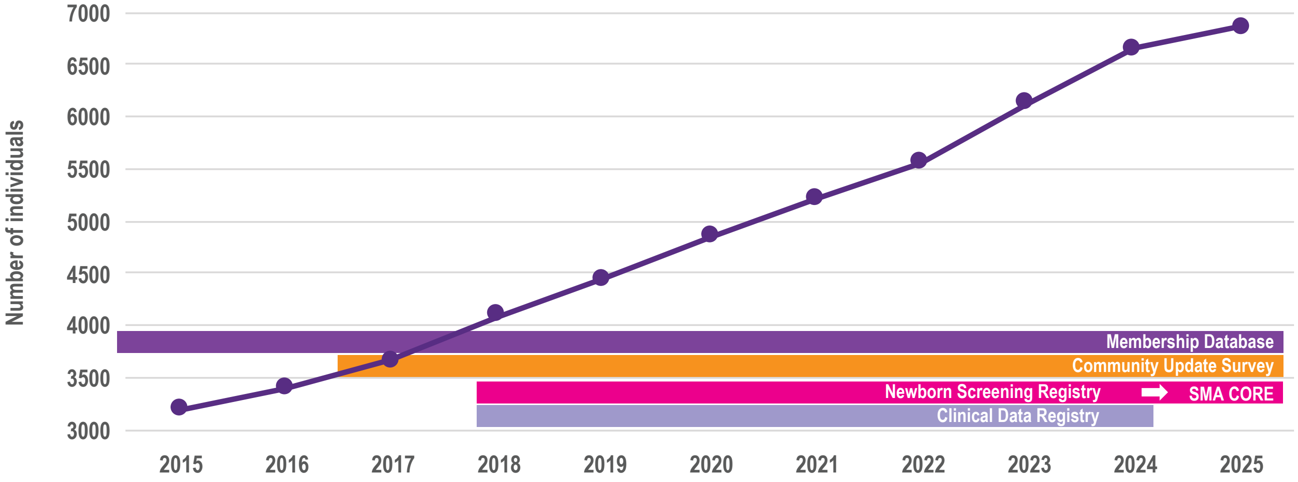
78% of adults report that living with SMA has impacted their mental or emotional well-being

CURE SMA DATA USED IN THIS REPORT

Cure SMA collects data from a variety of sources and surveys to understand the experiences of individuals with SMA, caregivers of individuals with SMA, and clinicians who care for individuals with SMA. The following data sources are used in this report:

	STATUS	PERSPECTIVES	SAMPLE SIZE
Membership Database (1996 – Present)			
The largest patient- and caregiver-reported **database** of individuals with SMA worldwide.	Active	Individuals with SMA and/or their caregivers	~12,000
Community Update Survey (CUS) (2017 – 2025)			
An **annual online survey** to capture longitudinal data from the patient's perspective and develop data that can support assessment of SMA disease impact.	Active	Individuals with SMA and/or their caregivers	~3,200
SMA Care, Outcomes, and Reported Experiences (CORE) Survey (2025 – Present)			
An **online survey** to capture the diagnostic and treatment journey as well as unmet needs for individuals newly diagnosed with SMA (previously the Newborn Screening Registry, NBSR).	Active	Individuals with SMA and/or their caregivers	~70
Unmet Needs Among Adults Across the Healthcare Spectrum (2025)			
An **online survey** to evaluate unmet needs and aspects of care for adult patients with SMA.	Complete	Adults with SMA	~270
The Clinical Data Registry (CDR) (2018 – 2024)			
A **registry** containing data sourced from electronic medical records and clinician-entered data from 25 U.S. care centers.	Complete	Clinicians caring for individuals with SMA	~1,200
Clinician Perspectives on SMA Treatment Decision Making and Access Barriers (2025)			
An **online survey** to understand the SMA treatment decision making process, challenges, barriers to accessing and administering treatment, and readiness for upcoming treatments.	Complete	Clinicians who prescribe SMA treatment	~90

U.S. Individuals with SMA Reflected in Cure SMA Data Sources[1]



Unless otherwise noted, the data presented in this report is pulled from the Membership database, CUS, CORE, and CDR. Individuals in multiple data sources were de-duplicated prior to analyses.

1. If data for an individual was missing date of first contact with Cure SMA, the date that a contact record was created was used, which may not always reflect the date of first contact.

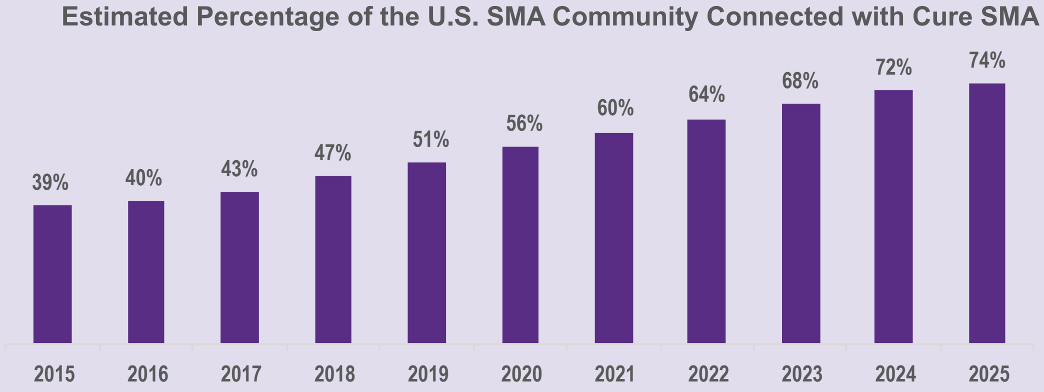
Analysis Notes:

- Graphics include individuals that were alive for any portion of the year specified.
- If an individual was deceased and deceased date was missing from data, they were not included in the graph.

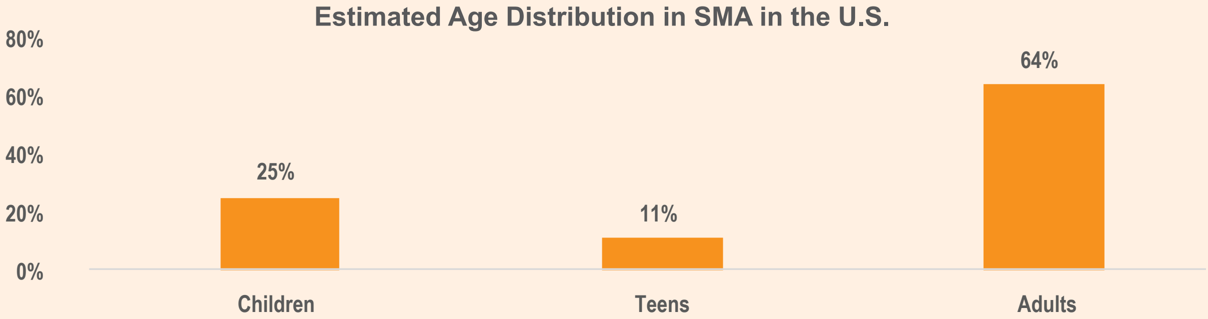
SMA MODEL

The "SMA Model" is not a database but a model that was created by Cure SMA to estimate the prevalence, survival, and characteristics of the population with SMA in the U.S. to gauge the coverage of the Cure SMA databases.

Cure SMA estimates there are currently 9,000-9,500 individuals with SMA currently living in the U.S.
Cure SMA has connected with the majority of these individuals



Cure SMA estimates the majority of individuals living with SMA are adults



Results from the model are based on the following inputs:

- Annual births from the National Vital Statistics System (NVSS)[1]
- Historical ("pre-SMA NBS") incidence rate of 1 in 11,000 decreasing to a current incidence rate of 1 in 17,267 from U.S. Public Health State labs[2]
- A decreasing mortality rate as reported by the CDC WONDER database[3] and the Cure SMA data, see page **50**.
- SMA subtype incidence of 50% for Type 1, 35% for Type 2, and 15% for Type 3/4[4]
- Race and ethnicity SMA carrier rates described by Sugarman et al.[5] (see page **14** for these estimates)

1. NVSS – Birth Data. Centers for Disease Control and Prevention (2026, January 27). https://www.cdc.gov/nchs/nvss/births.htm.
2. Belter L, et al. Newborn Screening and Birth Prevalence for Spinal Muscular Atrophy in the US. JAMA Pediatr. 2024;178(9):946–949.
3. CDC WONDER. Centers for Disease Control and Prevention. https://wonder.cdc.gov/.
4. Verhaart IEC, et al. Prevalence, incidence and carrier frequency of 5q-linked spinal muscular atrophy - a literature review. Orphanet J Rare Dis. 2017 Jul 4;12(1):124.
5. Sugarman EA, et al. Pan-ethnic carrier screening and prenatal diagnosis for spinal muscular atrophy: clinical laboratory analysis of >72,400 specimens. Eur J Hum Genet. 2012 Jan;20(1):27-32.

SMA BIRTH PREVALENCE

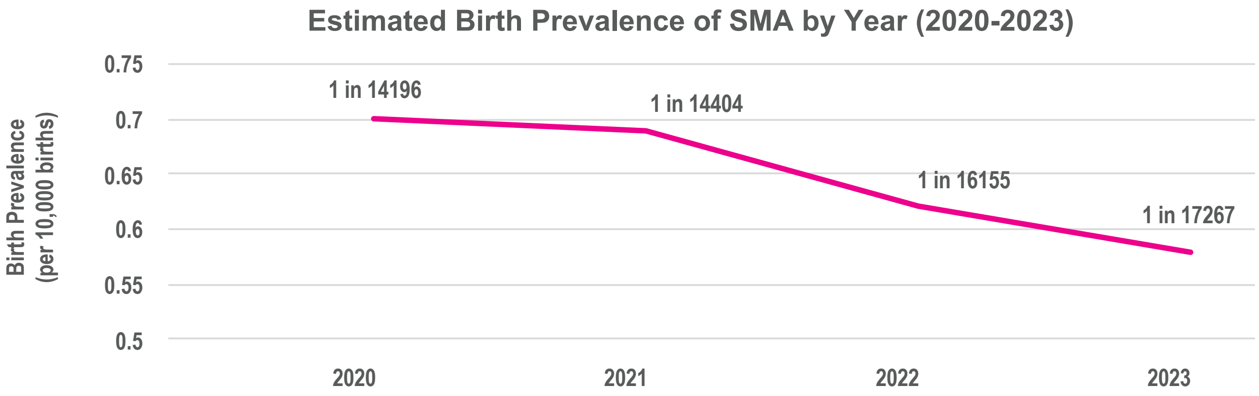
Cure SMA collects data to understand how many babies are born every year with SMA.

DATA FROM U.S. PUBLIC HEALTH STATE LABS

Cure SMA collects data from U.S. public health state labs on the number of individuals screened and the number of individuals that screened positive for SMA through statewide newborn screening (NBS). This data helps us understand the number of individuals diagnosed with SMA in the U.S. each year (birth prevalence).
Between January 2020 – December 2023, more than 6 million infants were screened for SMA, with an estimated 405 infants screened positive with SMA diagnosis confirmed. We have also observed a decrease in annual birth prevalence since 2020.

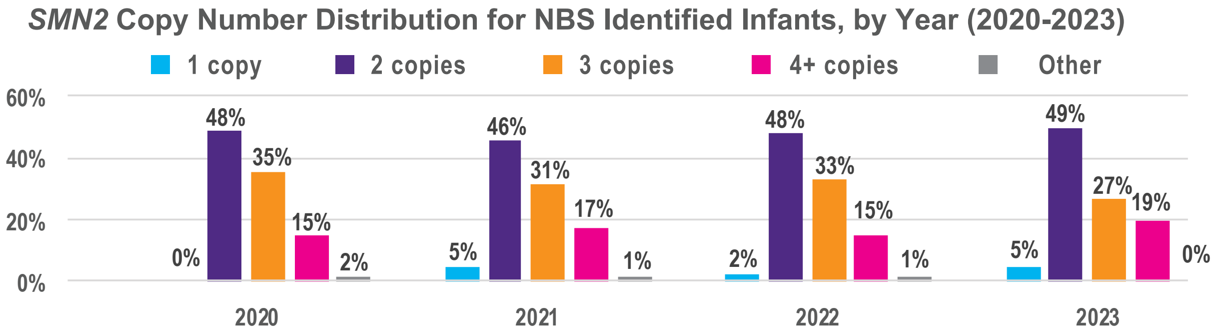
The current overall estimated incidence of SMA from 2020-2023 is approximately **1 in 15,590 births**.

The Association of Public Health Laboratories (APHL) collects data on newborn screening for SMA. For years 2020-2023 APHL has collected data on over 700 positive screenings for SMA.



States (n)	**16**	**18**	**22**	**21**
Infants screened (n)	**1,121,461**	**1,569,982**	**1,809,394**	**1,813,016**
Infants, positive screen (n)	**79**	**109**	**112**	**105**

Trends from 2020-2023 suggest a stabilization in the proportion of NBS infants born with 2 *SMN2* copies (48% vs 49%) and a slight decrease in 3 *SMN2* copies in 2023.



States (n)	**8**	**11**	**13**	**16**
Infants (n)	**62**	**83**	**81**	**83**

Analysis Notes:

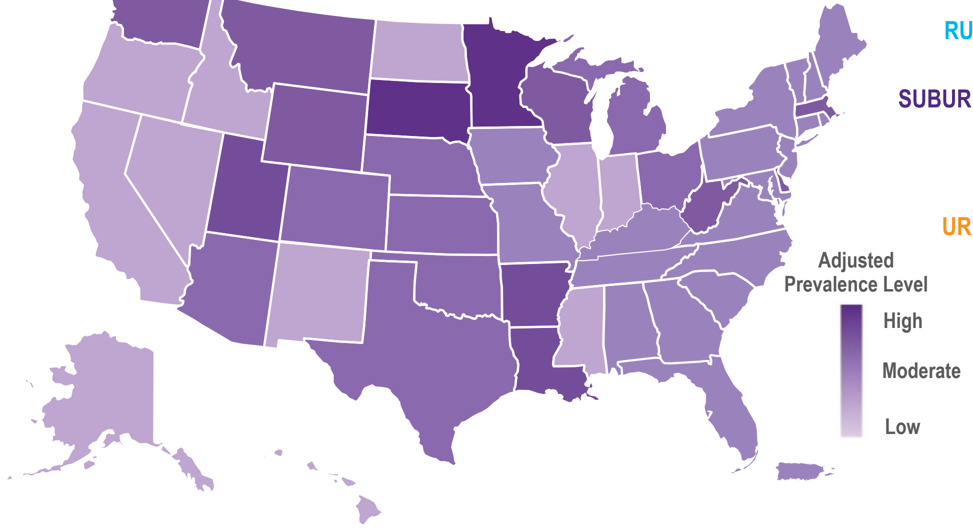
- Data from U.S. Public Health State Labs provided to Cure SMA.
- Birth prevalence is estimated and calculated only using data from states that provided data on number of infants screened per year for SMA.

U.S. DISTRIBUTION

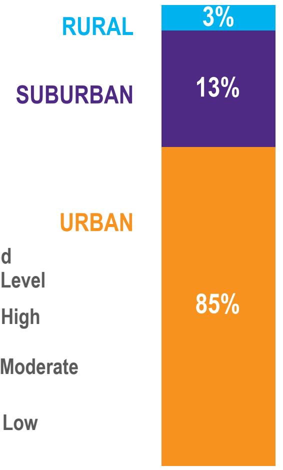
Cure SMA currently has 37 chapters throughout the U.S., and Puerto Rico, providing community and support for families and individuals living with SMA.

South Dakota, Minnesota, Wisconsin, Massachusetts, and Utah had the highest proportion of individuals living with SMA when adjusted for the state population. Additionally, majority of the population resides in an urban area (85%), similar to the general U.S. population (80.7%)

U.S. Distribution of Individuals Living with SMA, Adjusted for State Population (n=6,521)

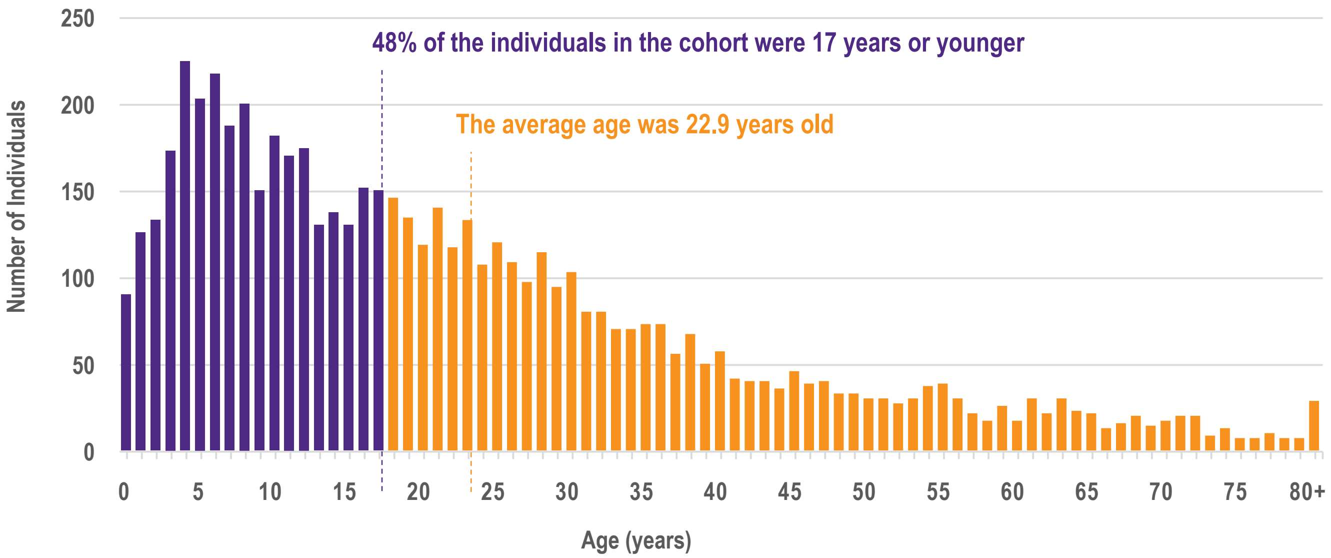


Urban/Rural Residence[1] (n=6,413)



AGE

Age Distribution of Individuals Living with SMA in Cure SMA Data in 2025 (n=6,153)



Average Age: 22.9 years | Standard Deviation: 18.2 years | Median Age: 18 years

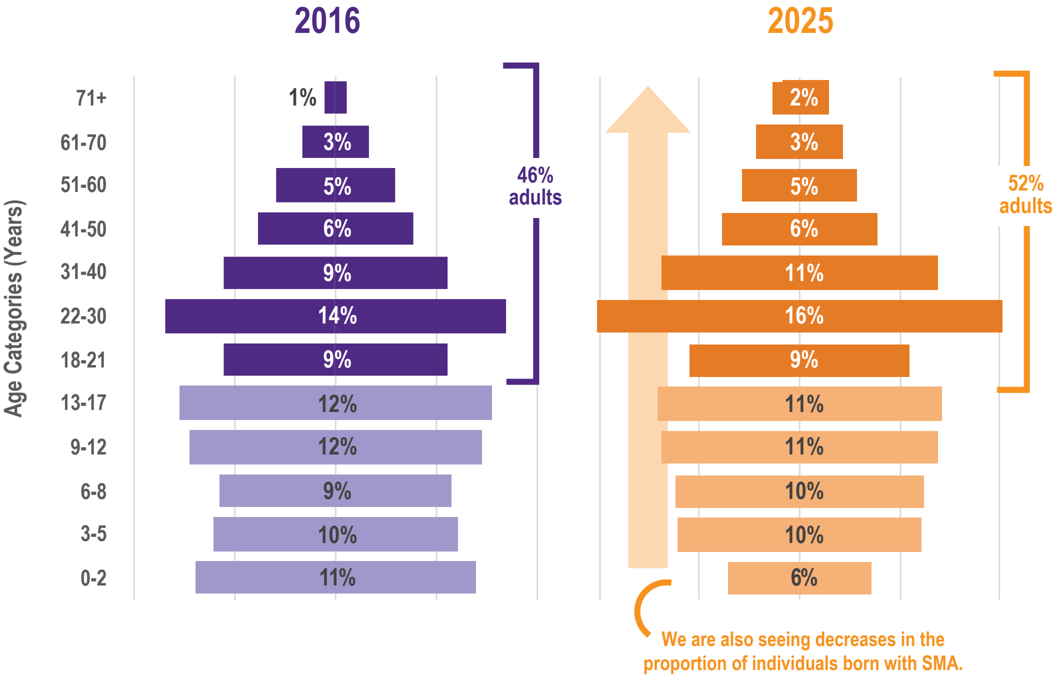
1. Urban/rural residence was determined using zip-code information (if available) and classifications determined by the U.S. Department of Agriculture (USDA). https://www.ers.usda.gov/data-products/rural-urban-commuting-area-codes/.

Analysis Notes:

- If state of residence was reported in multiple databases, CDR listing was prioritized.
- All graphics include individuals that were alive as of December 31, 2025.

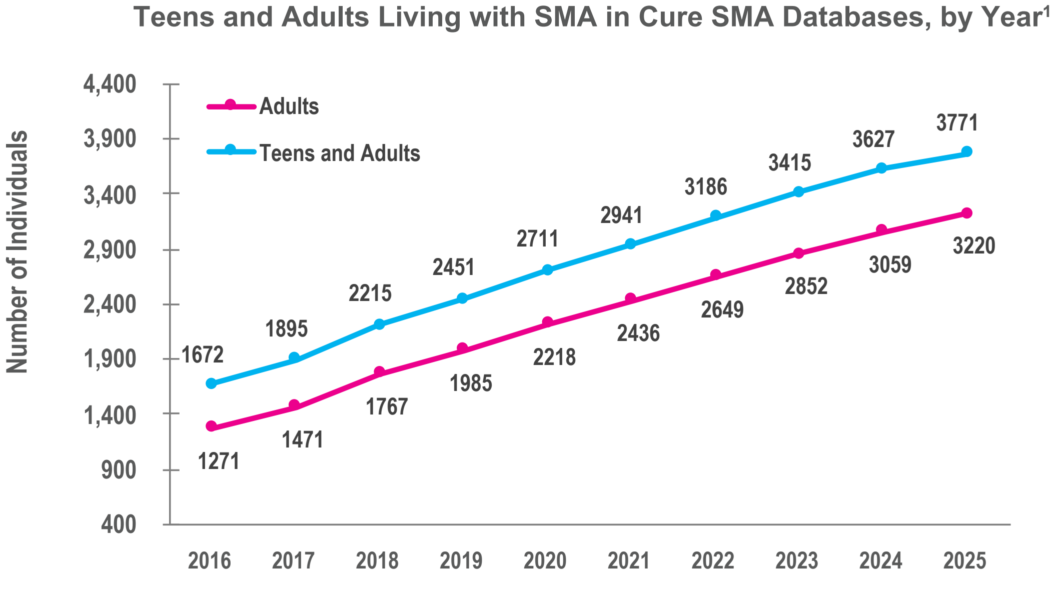
AGE TRENDS

The SMA landscape is changing, and we continue to see an increase in the proportion of individuals with SMA who are living into adulthood in our databases.



We are also seeing decreases in the proportion of individuals born with SMA.

The total number of adults and teens living within SMA in our databases has also steadily increased each year.



We strive to ensure our databases are reflective the SMA community.

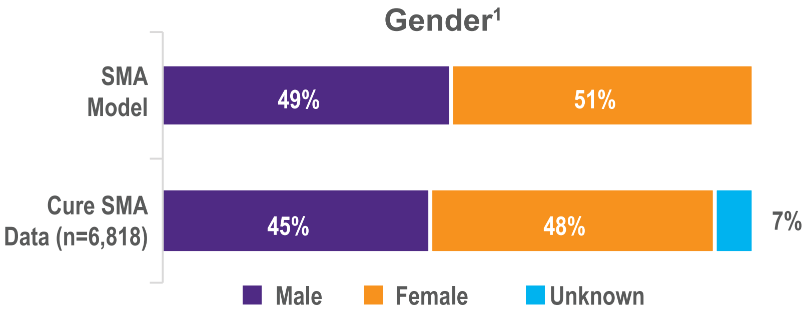
1. Individuals without a reported birthdate, and death date (if applicable) were not included. If an individual was missing date of first contact to Cure SMA, the date that a contact record was created was used, which may not always reflect the date of first contact.

Analysis Notes:

- Graphics include individuals that were alive as of 12/31 of the year specified.

GENDER / GENDER IDENTITY

In both the SMA model and Cure SMA data we observe a nearly even distribution of males and females with SMA.

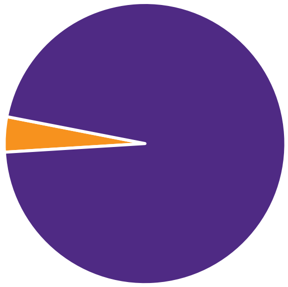


1.3% of individuals living with SMA report identifying with a gender that is different than their birth sex

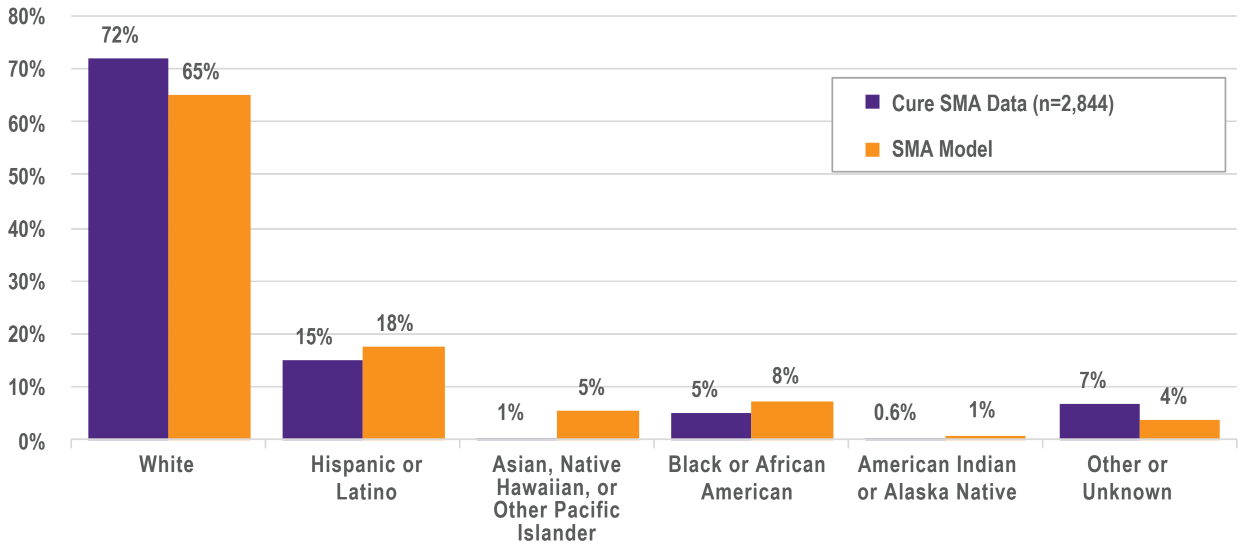
LGBTQIA+ POPULATION[2]

4% of individuals living with SMA identify as LGBTQIA+

Transgender Female Transgender Male Non-binary Gay
Genderqueer Fictoromantic Asexual Biromantic Queer Pansexual
Asexual Bisexual Lesbian Demisexual Homosexual



RACE/ETHNICITY CATEGORIES



1. The CDR collects gender used for administrative purposes (ie. Insurance) that may or may not align with a person's gender in all context.
2. LGBTQIA+ population was determined by those that had a gender listed as "Non-binary or genderqueer", a gender identity that was different from their sex assigned at birth, or a sexual orientation outside of straight or heterosexual.

Analysis Notes:

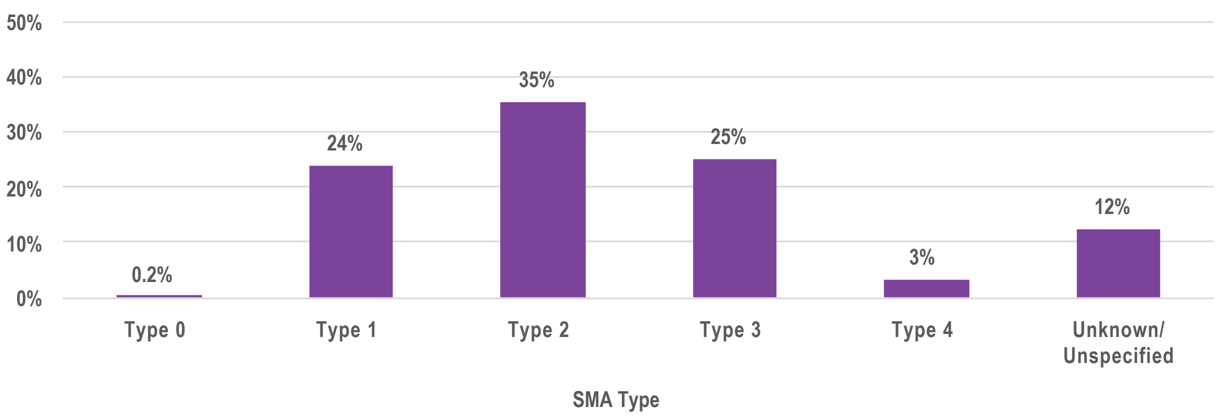
- All graphics include individuals that were alive as of December 31, 2025.
- If gender or race/ethnicity was provided for the same individual in multiple databases, the self-reported data was prioritized.
- Race and ethnicity categories are mutually exclusive. If an individual reported to be Hispanic or Latino and another race, they were categorized as Hispanic or Latino.

PREVALENCE OF SMA TYPE AND *SMN2* COPY NUMBER

SMA Type:

Historically, SMA has been characterized by a classification system for describing age of symptom onset and maximum motor function achieved. This classification divides SMA into five types: Types 0, 1, 2, 3, and 4.

Distribution of SMA Type (n=6,614)

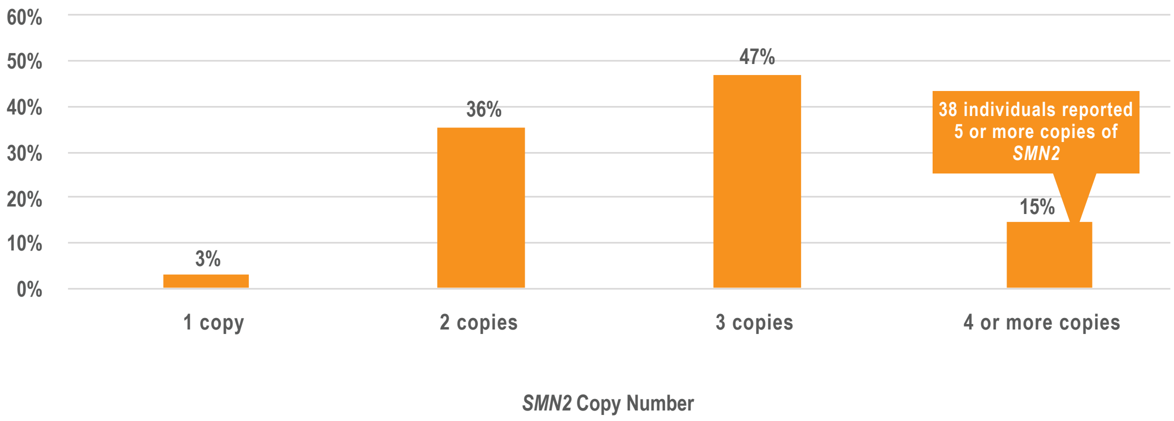


The largest proportion of individuals living with SMA in Cure SMA data sources were reported to have Type 2 SMA.

SMN2 Copy Number:

***SMN2* is an inefficient variant of the *SMN1* gene. This means that *SMN2* cannot fully make up for the mutated *SMN1* gene. The number of *SMN2* genes can vary from person to person, and individuals with more *SMN2* copies usually have a less severe form of SMA than those with fewer copies. However, there are exceptions.**

Distribution of *SMN2* Copy Number[1] (n=3,048)



38 individuals reported 5 or more copies of *SMN2*

The largest proportion of individuals living with SMA in Cure SMA data sources were reported to have 3 copies of *SMN2*.

1. Individuals with unknown *SMN2* copy number were not included in this graphic.

Analysis Notes:

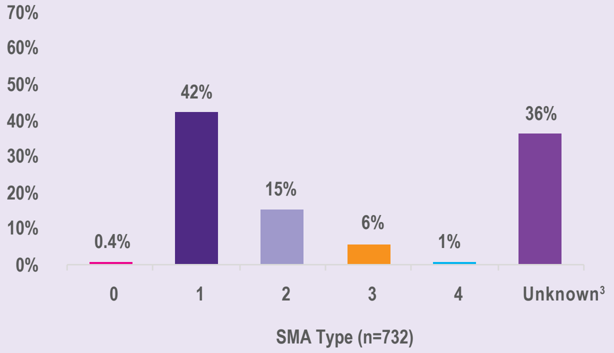
- All graphics include individuals that were alive as of December 31, 2025.

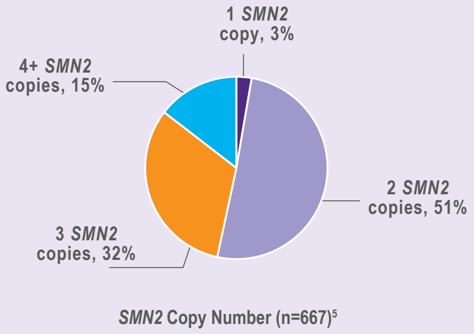
PREVALENCE OF SMA TYPE AND *SMN2* COPY NUMBER

The prevalence of SMA Type and *SMN2* copy number vary by current age and method of diagnosis

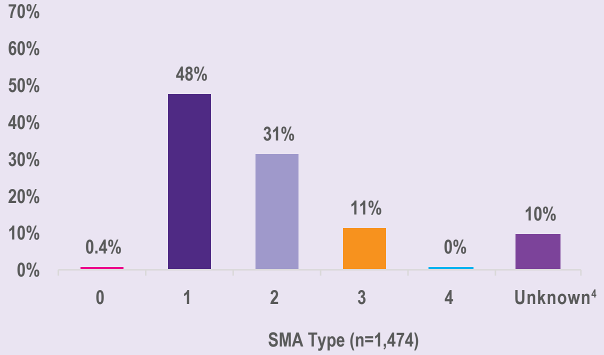
CHILDREN LIVING WITH SMA[1] (0-12 YEARS OLD)

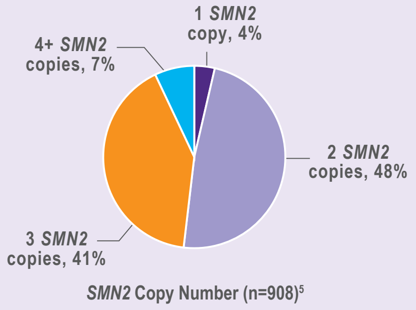
Identified by Newborn or Prenatal Screening[1,2]



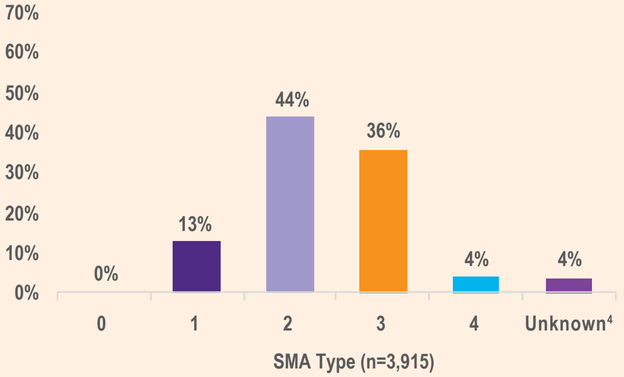


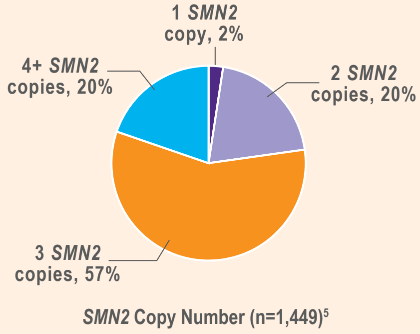
Not Reported as Identified via Newborn or Prenatal Screening





TEENS AND ADULTS LIVING WITH SMA (13+ YEARS OLD)





1. Newborn screening status is caregiver-reported.
2. Graph restricted to individuals diagnosed before 2 years of age.
3. Unknown SMA type includes the following: Unknown, Unspecified, Pre-symptomatic, and Unknown because diagnosed < 6 months of age before symptom onset. If does not include missing values.
4. Unknown SMA type includes the following: Unknown, Unspecified. Individuals with data missing an SMA type were not included in the graph.
5. Individuals with unknown *SMN2* copy number were not included (children via screening, n=70; children not via screening, n=568; teens/adults, n=2,472).

Analysis Notes:

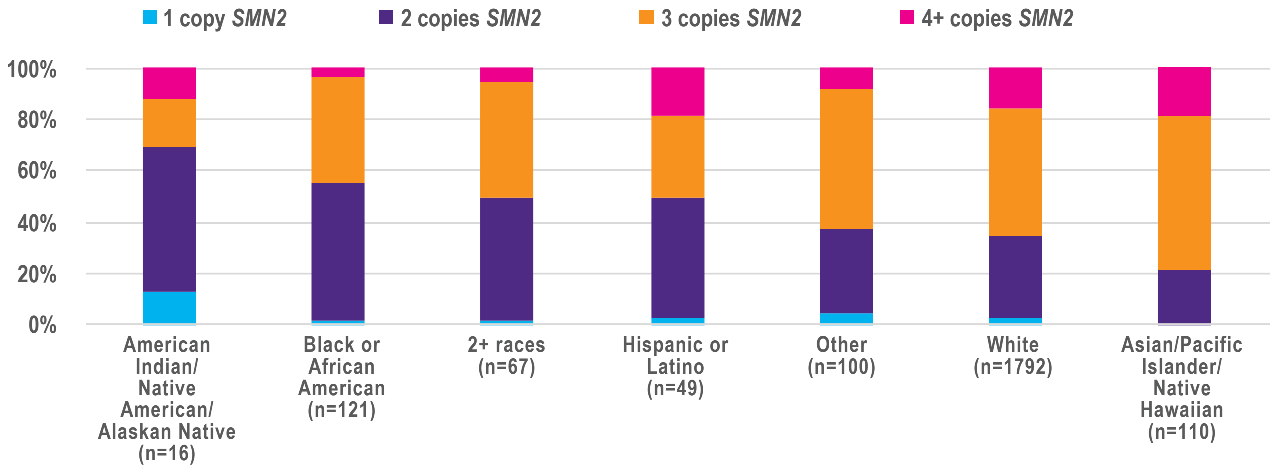
- All graphics include individuals that were alive as of December 31, 2025.

PREVALENCE OF *SMN2* COPY NUMBER

BY RACE / ETHNICITY

In our data, the distribution of *SMN2* copy number among living individuals varied by reported race/ethnicity. Individuals reporting American Indian/Native American/Alaskan Native had the highest proportion of 2 copies of *SMN2* and the lowest proportion of 3 copies of *SMN2*. In contrast, Asian/Pacific Islander/Native Hawaiian had the lowest proportion of 2 copies of *SMN2* and the highest proportion of 3 copies of *SMN2*. Data below shown in order of decreasing proportion of 2 copies of *SMN2*, by race/ethnicity.

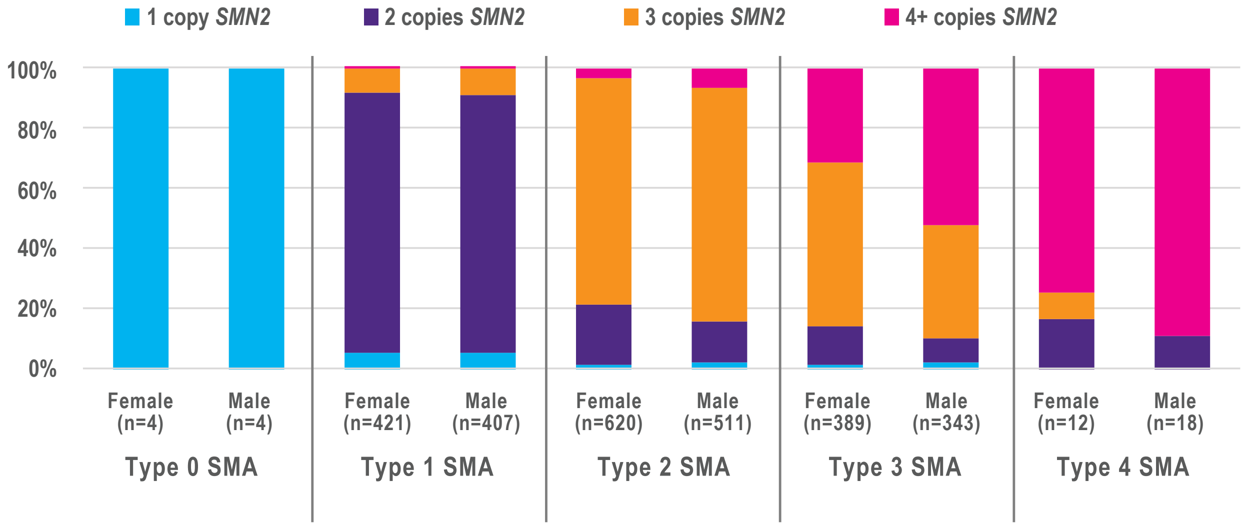
Prevalence of *SMN2* Copy Number, by Race/Ethnicity[1-2]



BY BIOLOGICAL SEX

When stratified by SMA Type, the distribution of *SMN2* copy number was similar between males and females for SMA Type 0, 1, and 2. We observed a slightly higher proportion of males with 4+ copies of *SMN2* for individuals that reported Type 3 or Type 4 SMA.

Distribution of *SMN2* Copy Number, by SMA Type and Biological Sex – All Ages[3]



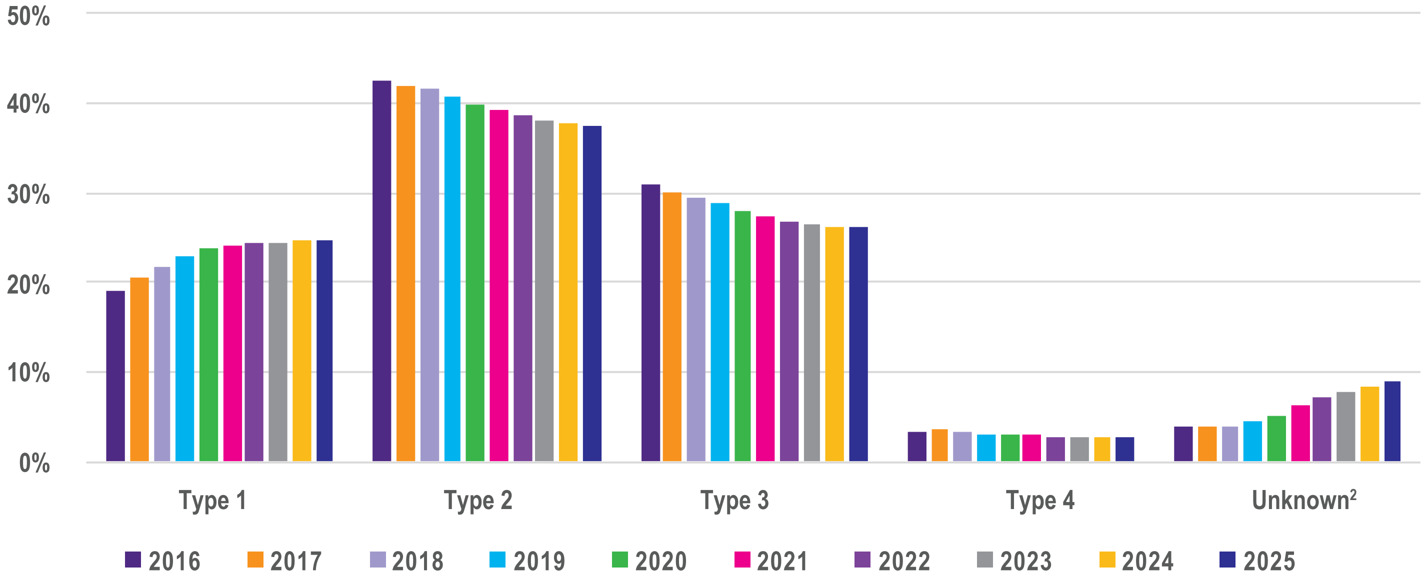
1. Individuals with unknown race/ethnicity or *SMN2* copy number were not included (n=4,477).
2. Race and ethnicity categories are mutually exclusive. If an individual reported to be Hispanic or Latino and another race, they were categorized as Hispanic or Latino.
3. Individuals with unknown SMA Type, biological sex, or *SMN2* copy number were not included (n=3,807).

Analysis Notes:

- All graphics include individuals that were alive as of December 31, 2025.

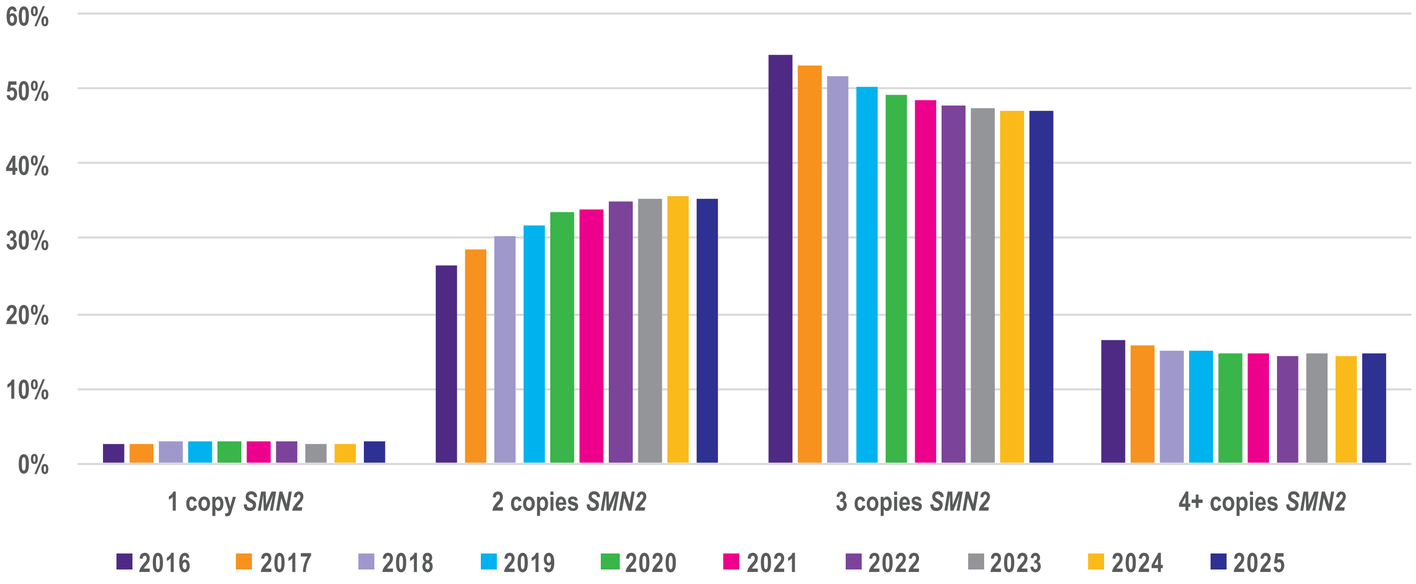
TRENDS IN OUR DATA: SMA TYPE & *SMN2* COPY NUMBER

PREVALENCE OF SMA TYPE OVER TIME[1]



We continued to observe rising proportions of individuals with SMA Type 1 and Unknown SMA Type and individuals with 2 *SMN2* copies. While we didn't analyze what factors might be driving this change, an increase in survival of individuals due to diagnosis before symptom onset and the availability of treatments may be possible factors.

PREVALENCE OF *SMN2* COPY NUMBER OVER TIME[3]



1. SMA Type 0 was not included in the graphic due to small sample size.
2. Unknown SMA type includes the following: Unknown, Unspecified, Pre-symptomatic, and Unknown because diagnosed < 6 months of age before symptom onset. It does not include missing values.
3. Individuals with unknown *SMN2* copy number were not included in this graphic.

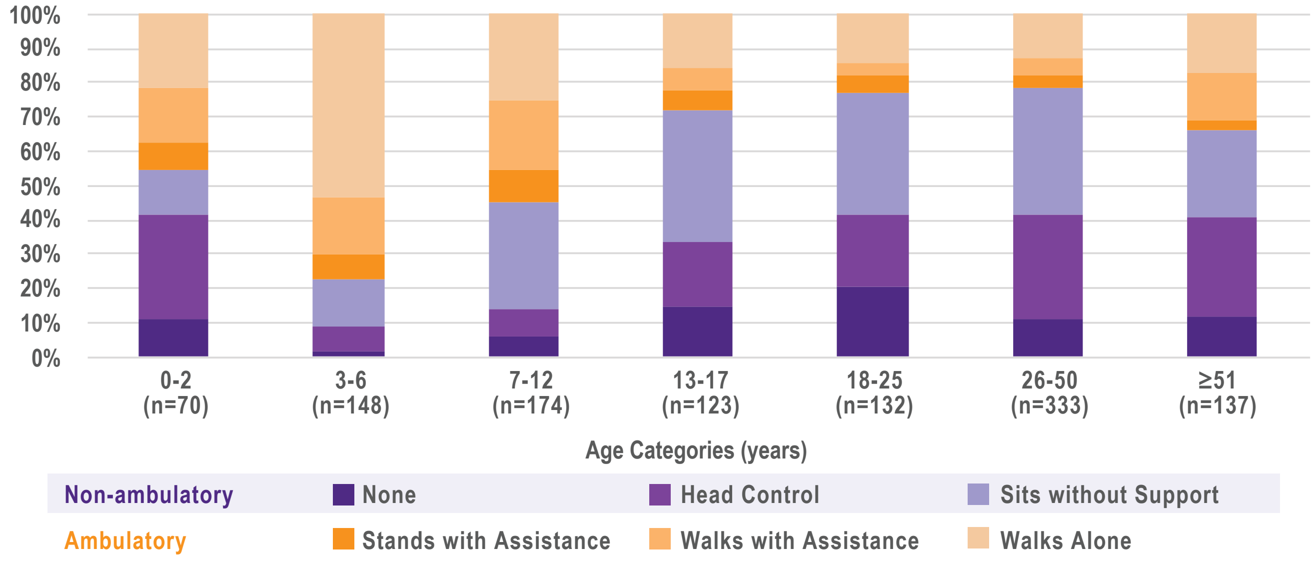
Analysis Notes:

- All graphics include individuals that were alive as of December 31st of each specified year.

MOTOR FUNCTION STATUS

63% of children, 28% of teens, and 24% of adults living with SMA are currently ambulatory[1,2]

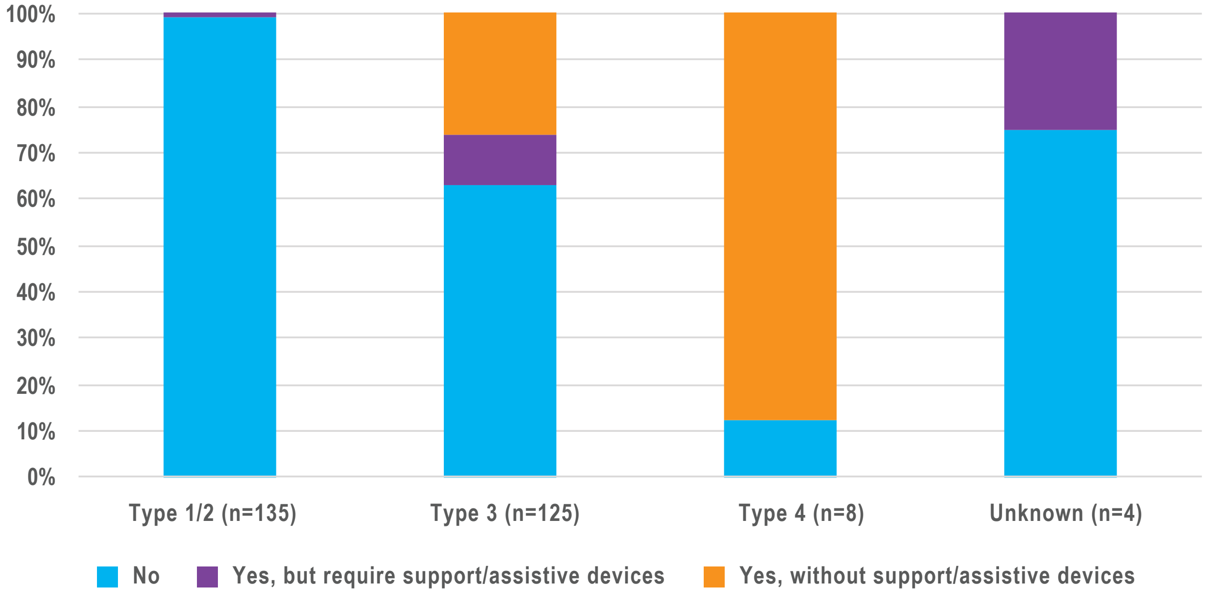
Maximum Current Motor Function of Individuals with SMA by Age



AMBULATION STATUS AMONG ADULTS

20% of adults living with SMA are able to walk 10 or more steps with or without a supportive/assistive device.

Ability to Walk 10 or More Steps by Type of SMA[3]

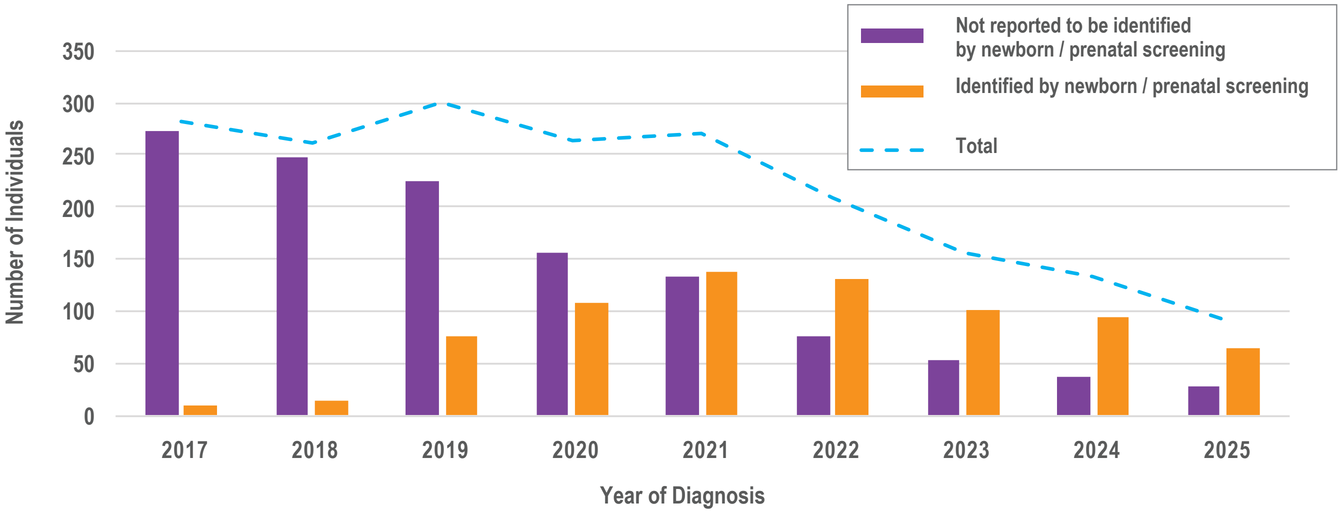


1. Analysis limited to those 2 years of age and older and excludes those with SMA Type 0 and Type 4 due to small sample size.
2. Ambulatory defined as those who stands with assistance, walks with assistance, or walks alone.
3. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

DIAGNOSIS OF SMA

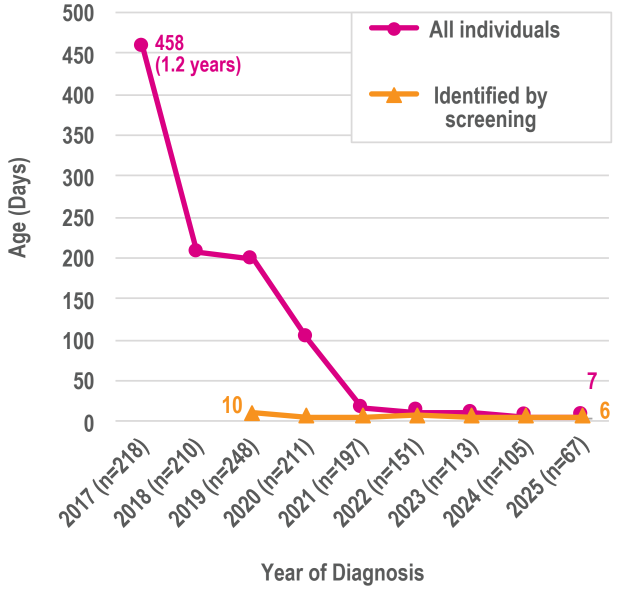
In the Cure SMA databases, we have observed a decline in the number of individuals diagnosed with SMA in the last few years. We continue to see that most diagnoses are identified via newborn or prenatal screening.

Reported Diagnosis Method for Individuals Diagnosed with SMA Between 2017-2025

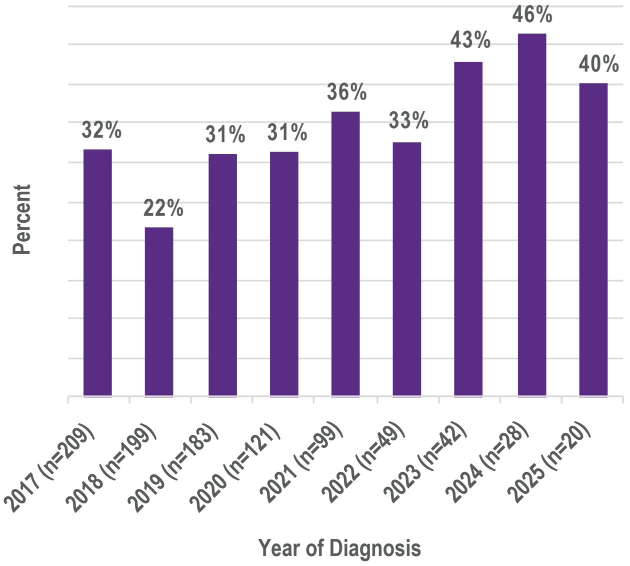


The median age at SMA diagnosis in 2025 continued to remain at 7 days of age. However, we are observing a general increase in the percentage of individuals not identified by screening that are diagnosed after 3 years of age, and we anticipate that percentage may continue to increase as more individuals diagnosed symptomatically are diagnosed at an older ages due to later symptom onset.

Median Age at SMA Diagnosis[1]



Individuals Not Identified by Screening Who Were Diagnosed after Age 3



1. Included individuals where the date of diagnosis was not reported to be estimated.

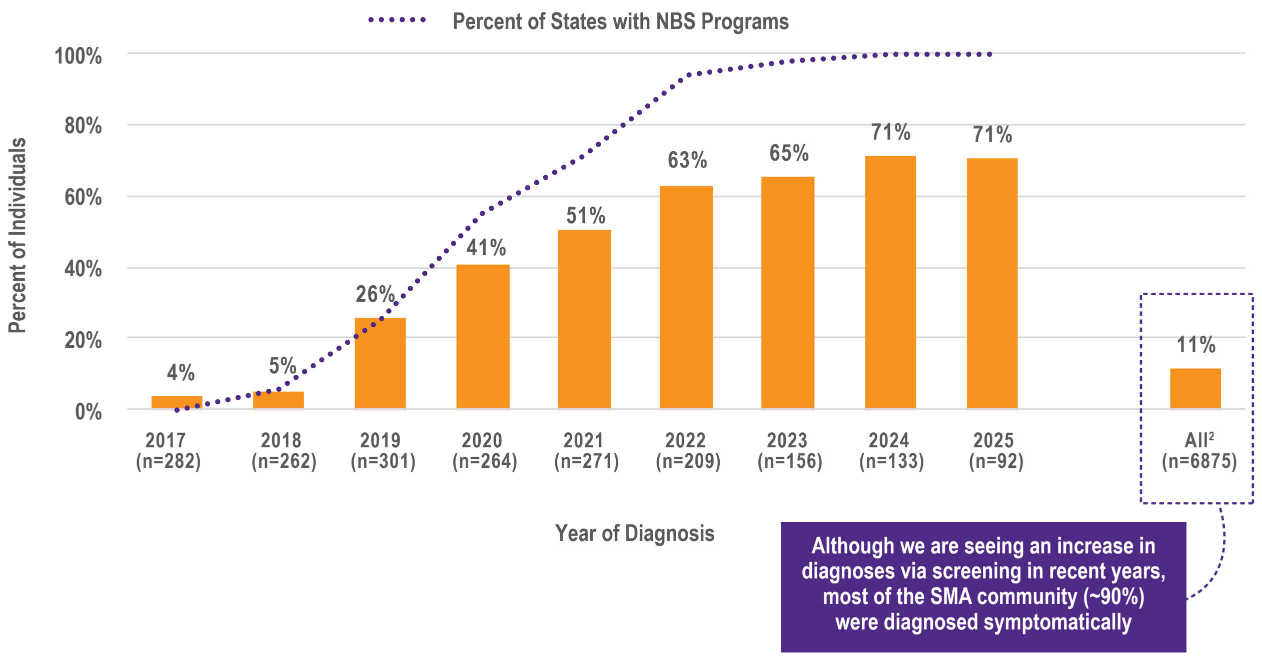
Analysis Notes:

- Newborn and prenatal screening status are patient reported. If status was missing, it was assumed the individual was not identified by screening.
- Diagnosis date could not be more than 1 year prior to birthdate.

DIAGNOSIS OF SMA

The number of U.S. states implementing newborn screening programs reached 100% in 2024, and we continue to see >70% of individuals diagnosed in 2025 were identified by newborn or prenatal screening.

Percent of Individuals Identified by SMA Newborn or Prenatal Screening, by Year of Diagnosis[1]



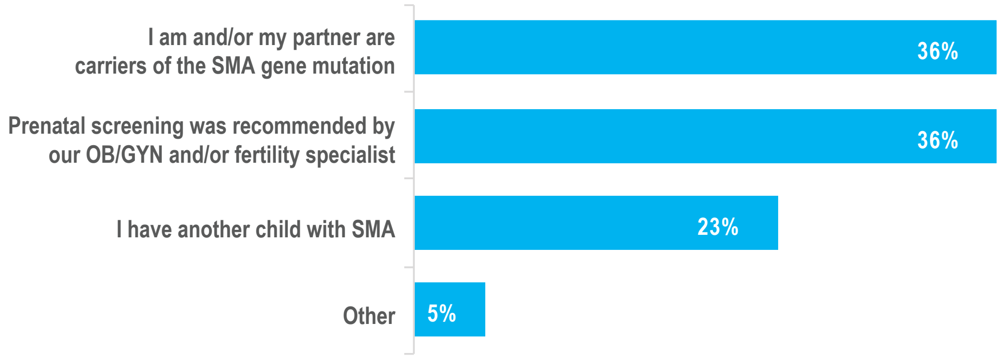
Although we are seeing an increase in diagnoses via screening in recent years, most of the SMA community (~90%) were diagnosed symptomatically

PRENATAL SCREENING

The most common primary reasons for prenatal screening were that one or both biological parents knew they were carriers of the SMA gene mutation (36%) and that prenatal screening was recommended by the OB/GYN and/or fertility specialist (36%).

18% of individuals were identified by prenatal screening in 2025

Reason for Prenatal Screening (n=21)[3]



1. Sample includes anyone reported to be identified via newborn or prenatal screening.
2. Data from the Cure SMA Membership database, CDR, CUS and CORE.
3. Data from the 2025 CUS and SMA CORE.

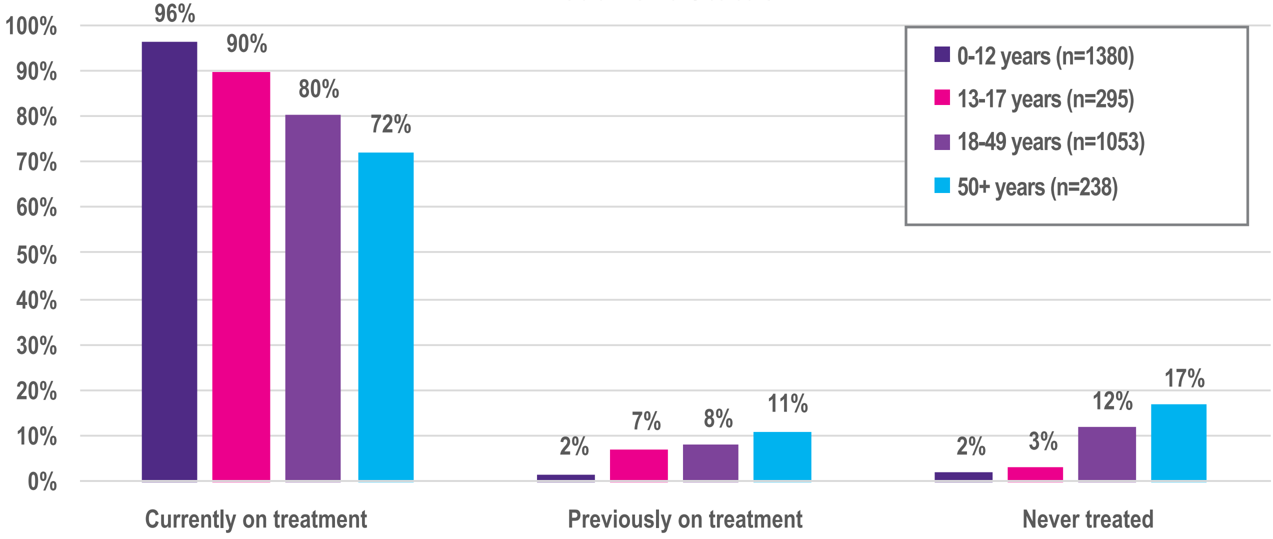
Analysis Notes:

- Newborn and prenatal screening status are patient reported. If status was missing, it was assumed the individual was not identified by screening.

TREATMENT: UTILIZATION

In our data, approximately 93% of individuals have received an SMA treatment. When broken down by age categories and current treatment status, a greater percentage of children and teens are currently receiving treatment[2] (90%-96%) than adults (72%-80%). A higher proportion of adults have discontinued or never initiated SMA treatment.

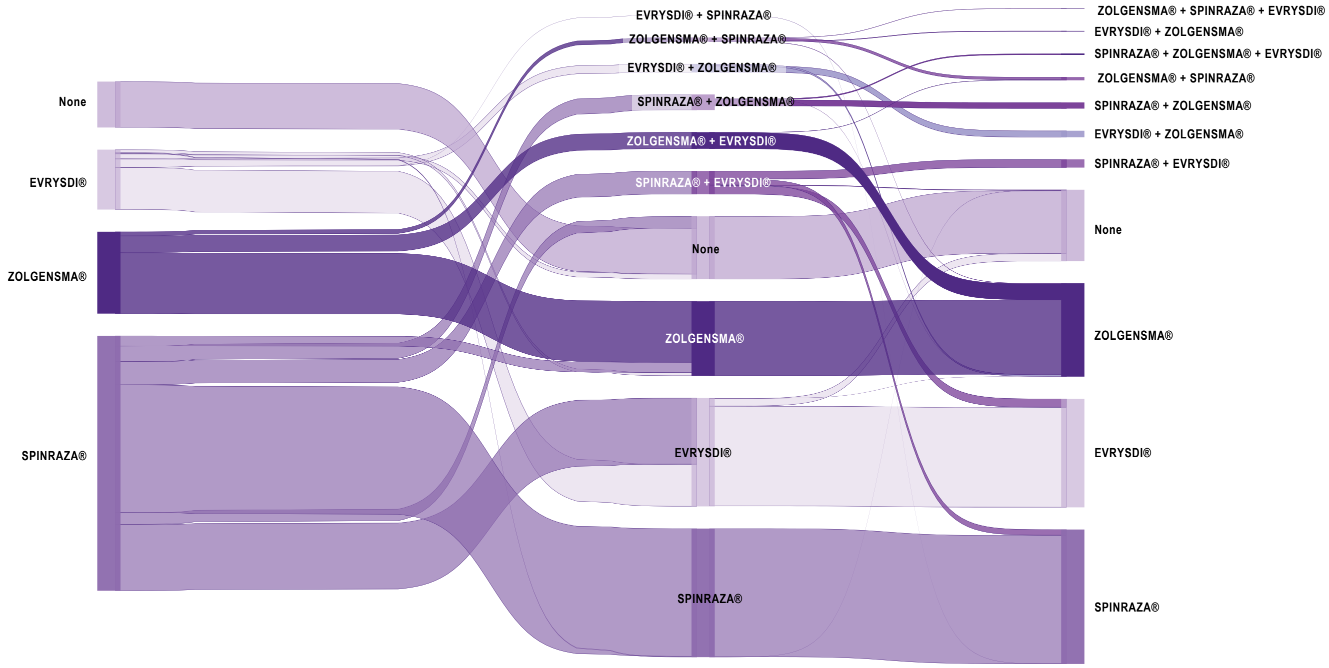
Treatment Status[1,2]



THE COMPLEXITY OF SMA TREATMENT PATTERNS

As the number of treatments available to patients living with SMA continue to increase, so do the different treatment patterns. As seen in the diagram below, the treatment patterns when utilizing more than one treatment are varied and complex.

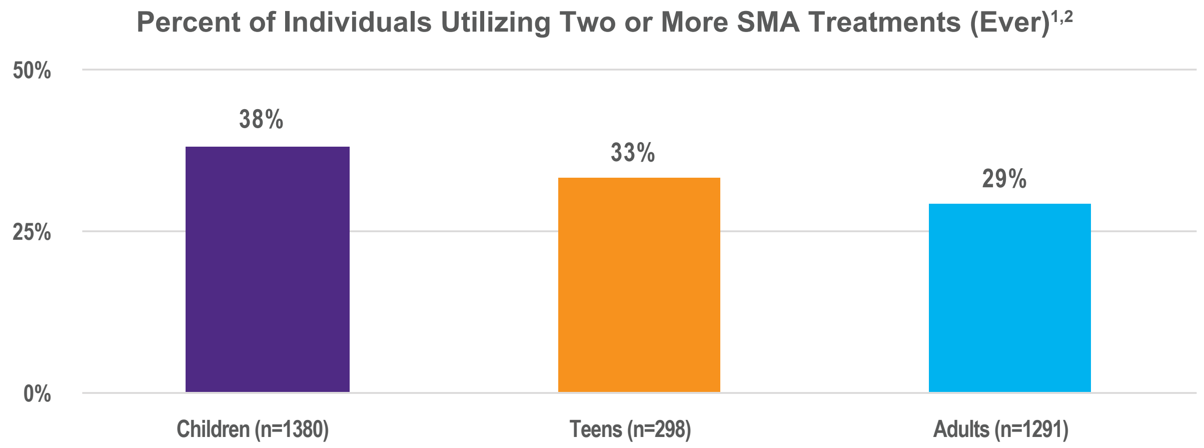
SMA Treatment Patterns[3]



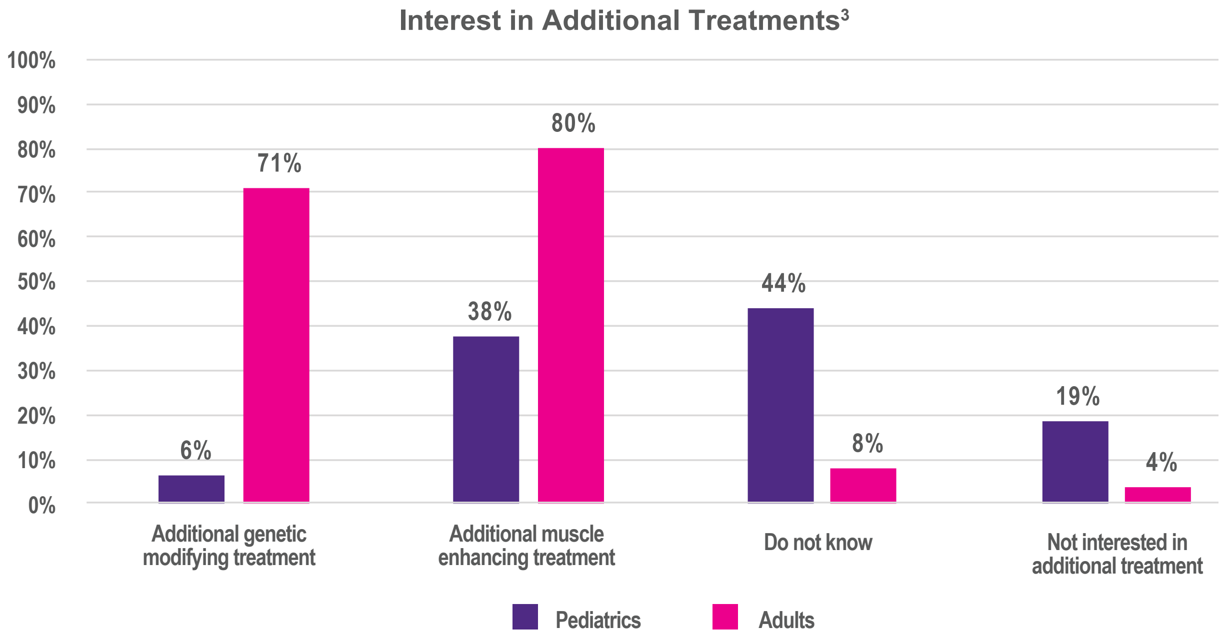
1. Sample does not include any deceased individuals.
2. Current at the time of last data collection. There may be a lag in our data capture and this chart may not reflect the most up to date information for all individuals.
3. Order of treatment names reflect the order at which someone received their treatment.

TREATMENT: USE OF MULTIPLE TREATMENTS

In our data, approximately 34% of individuals living with SMA have received two or more FDA-approved SMA treatments, either sequentially or in combination. A higher percentage of children have received multiple SMA treatments compared to adults.



81.5% of individuals living with SMA that have received one FDA-approved SMA treatment are interested in receiving an additional genetic and/or muscle enhancing SMA treatment.[3]



Many clinicians report that they would like to see more data on the effectiveness (73%) and safety (60%) of combining more than one disease modifying treatment[4]

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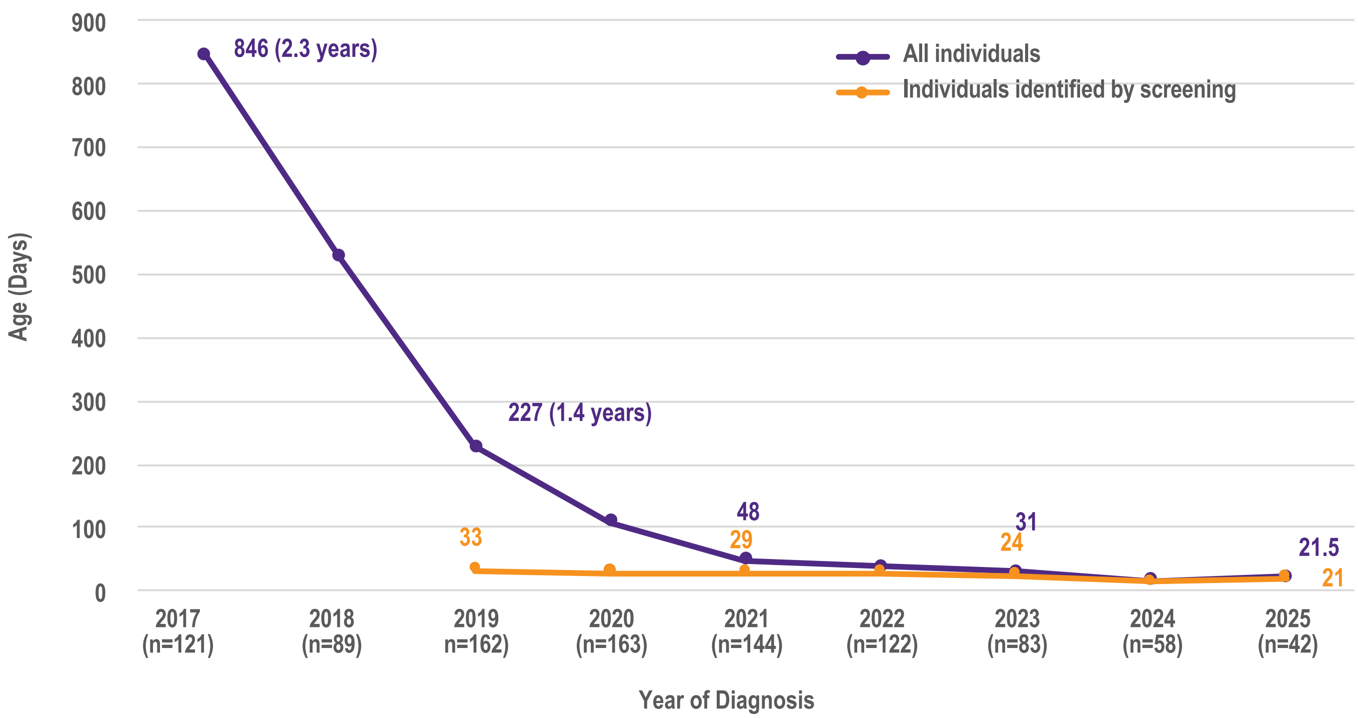
1. Based on data from individuals with treatment status information on FDA approved treatments only. Individuals who have passed away were not included in the graph. Note that Evrysdi is counted as one treatment, regardless of formulation.
2. Data from the Cure SMA Membership database, CDR, CUS and CORE.
3. Data from the 2025 CUS data among individuals who have received one FDA-approved treatment, n=92.
4. Data from the Clinician Perspectives on SMA Treatment Decision Making and Access Barriers survey (2025); n=83.

TREATMENT: AGE AT FIRST TREATMENT

In Cure SMA databases, the average age at first treatment has decreased since 2017. For individuals diagnosed with SMA in 2025, the average age at first treatment remains under 30 days.

Median Age at First SMA Treatment, by Year of Diagnosis[1]



Even though the median age at first treatment has decreased to less than 30 days in 2025, and we are seeing increases in the proportion of individuals receiving treatment before 30 days old, this does not reflect the historical treatment journey for most individuals with SMA in our databases.

16% of individuals with SMA in Cure SMA databases that did not participate in a clinical trial received treatment by 30 days old (n=2017)

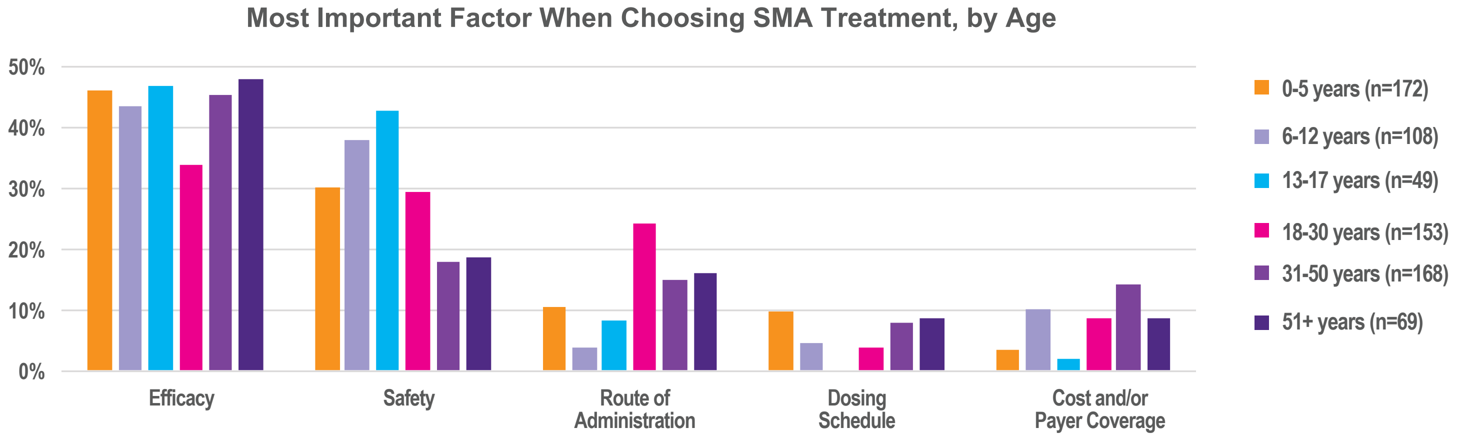
1. Newborn and prenatal screening status are caregiver reported. If status was missing, it was assumed the individual was not identified by screening.

Analysis Notes:

- Includes SMA treatments that were FDA-approved as of 12/31/2025.
- Graphs include individuals who have not participated in clinical trials and where all treatment dates are known.

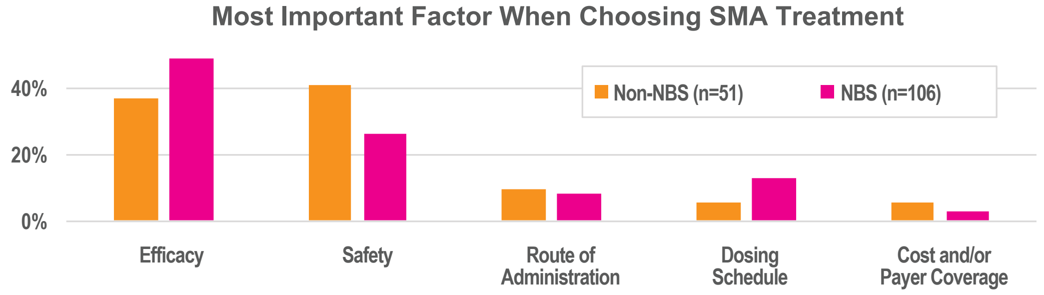
TREATMENT: FACTORS WHEN CHOOSING TREATMENT

Across age categories, individuals with SMA and/or their caregivers rank efficacy and safety as the most important factors when choosing an SMA treatment. However, there was variability across age groups and across subgroups within age categories.

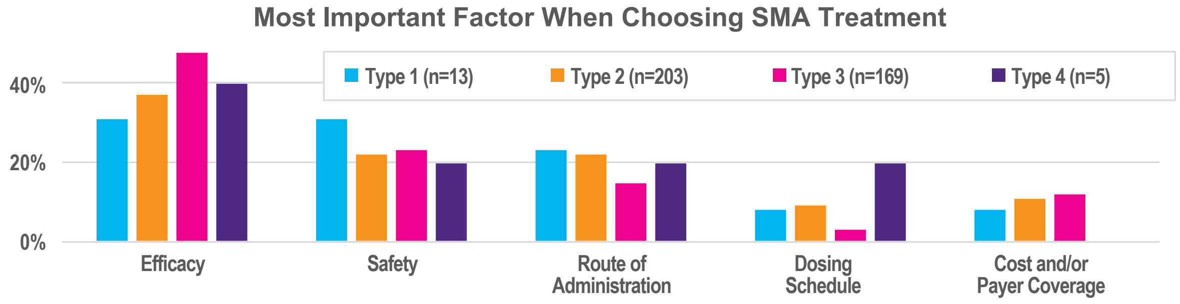


DIFFERENCES BY SUBGROUP

Children 0-5 Years Old, by Newborn Screening (NBS) Status



Adults 18+ Years Old, by SMA Type



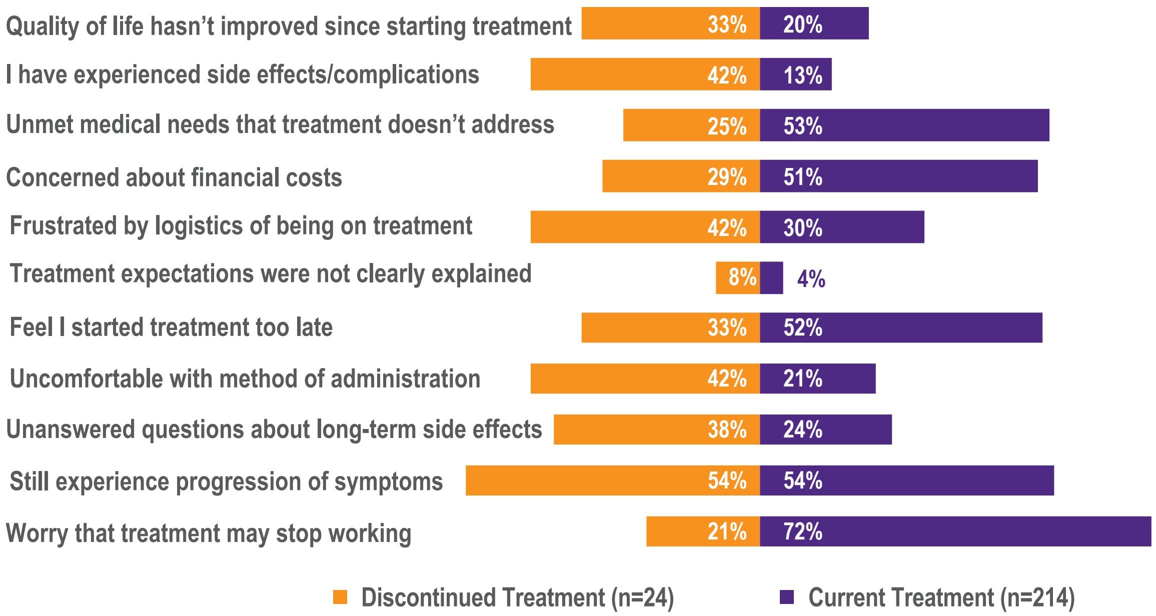
Analysis Notes:

- Data sourced from 2024-2025 CUS. Individuals participating in multiple years were de-duplicated.
- The caregiver perspective is captured for individuals under 18 years of age. For individuals 18 years or older, this data includes the perspectives of individuals with SMA and their caregivers. If both an adult with SMA and their caregiver completed a survey, the more complete survey was used.

TREATMENT: UNMET NEEDS AND GAPS

Adults who reported ever receiving a disease modifying therapy were asked what unmet needs and concerns still remain. "Worry that treatment may stop working" overwhelmingly stood out among adults currently on treatment, while a top concern among those who have discontinued treatment is "still experiencing a progression of symptoms."

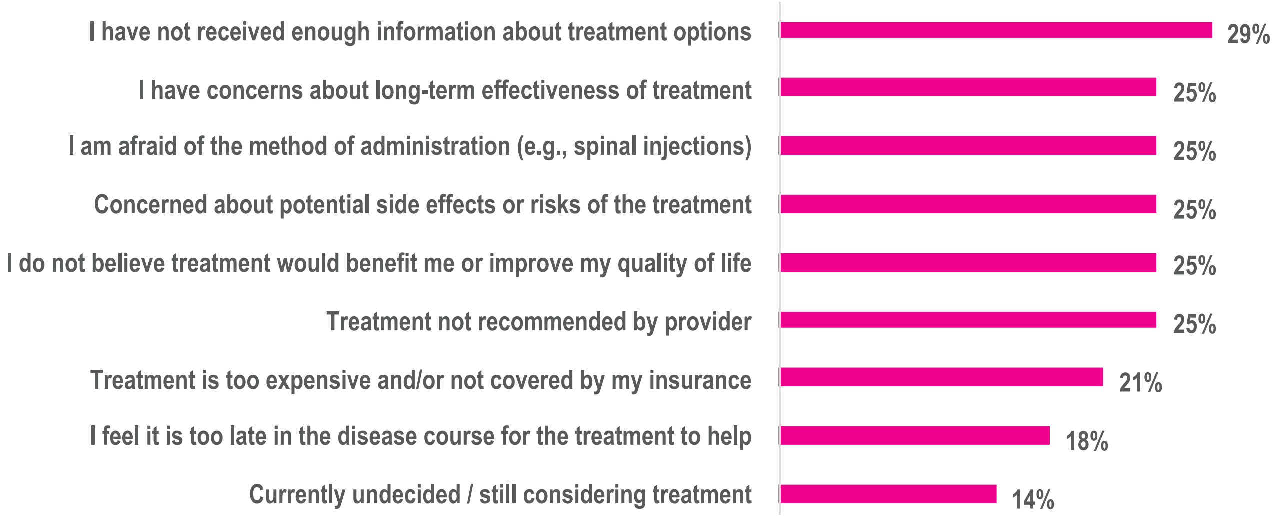
Top Reported Remaining Unmet Needs and Concerns Among Adults Ever Treated for SMA[1,2]



DECLINING TO RECEIVE TREATMENT

While most adults do report being treated, there is a segment of the population who has never received a disease modifying therapy. When asked about reasons for declining treatment, responses varied, suggesting that the decision is highly individualized.

Top Reported Reasons for Declining to Receive Treatment[1] (n=28)



1. Options are not mutually exclusive, and respondents were able to "select all that apply".
2. Current treatment classified as on FDA-approved treatment at time of survey completion, regardless of duration. Discontinued treatment classified as having stopped FDA-approved treatment at time of survey, regardless of duration.

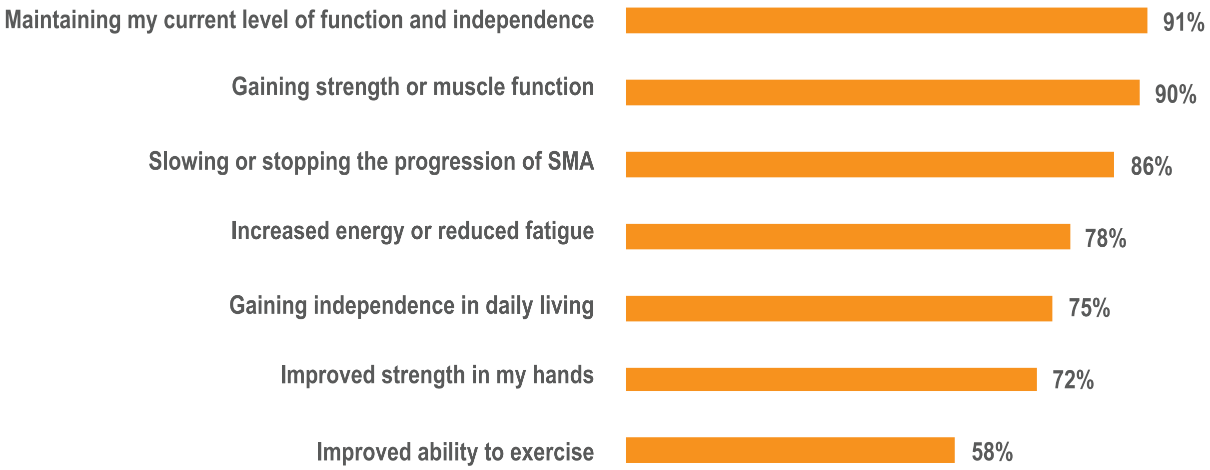
Analysis Notes:

- Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

TREATMENT: MEASURING TREATMENT EFFECTIVENESS

When adults with SMA who were on a current FDA-approved treatment were asked what they consider to be indicators of a successful treatment, "Maintaining my current level of function and independence," "Gaining strength or muscle function," and "Slowing or stopping the progression of SMA" stood out as top indicators.

What do you consider to be indicators of successful treatment?[1] (n=211)



CLINICIAN PERSPECTIVES[2]:

Providing Support of Treatment Effectiveness to Payers:

Metrics that have been used to successfully provide support of treatment effectiveness to payers when motor function gain has not been observed:

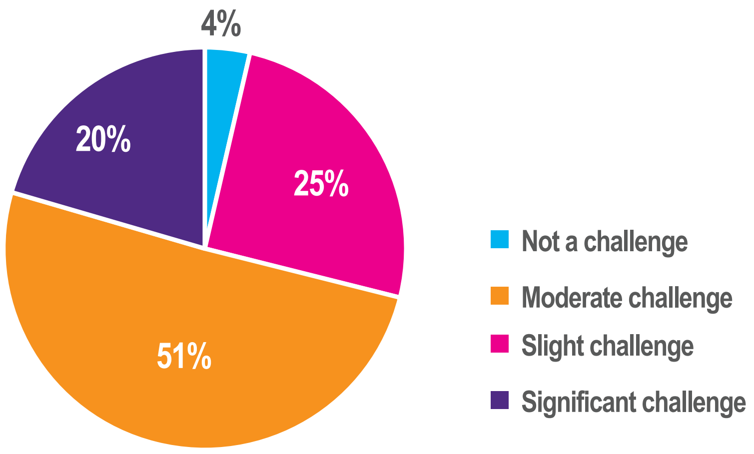
72% reported stability in motor function scales

55% reported reduced need for respiratory support

55% reported decreased frequency of respiratory infections or complications

50% reported improvements in fine motor function that lead to greater autonomy not detected on routine motor function scales

71% of clinicians reported that lack of scales that are sensitive enough to detect small changes in motor function was a moderate or significant challenge with treatment decision making:



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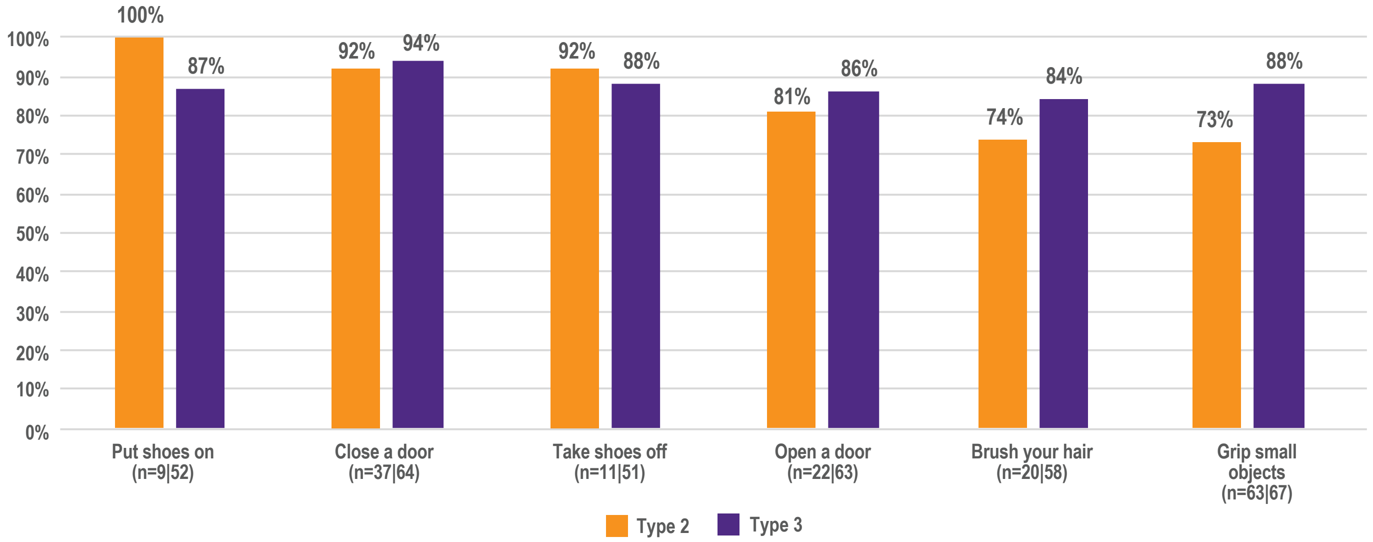
1. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.
2. Data from the Clinician Perspectives on SMA Treatment Decision Making and Access Barriers survey (2025); n=83.

TREATMENT: CHANGES IN ACTIVITIES OF DAILY LIVING

A recent publication by Cure SMA described findings of a study of key expert leaders in the SMA space and those living with SMA in which they found agreement that activities of daily living (ADLs) would be sensitive and impactful outcomes in the assessment of function, perceived fatigue, and perceived fatigability.[1] As a result of this study, Cure SMA began collecting in the annual community update survey the current status and changes in ADLs among adults living with SMA.

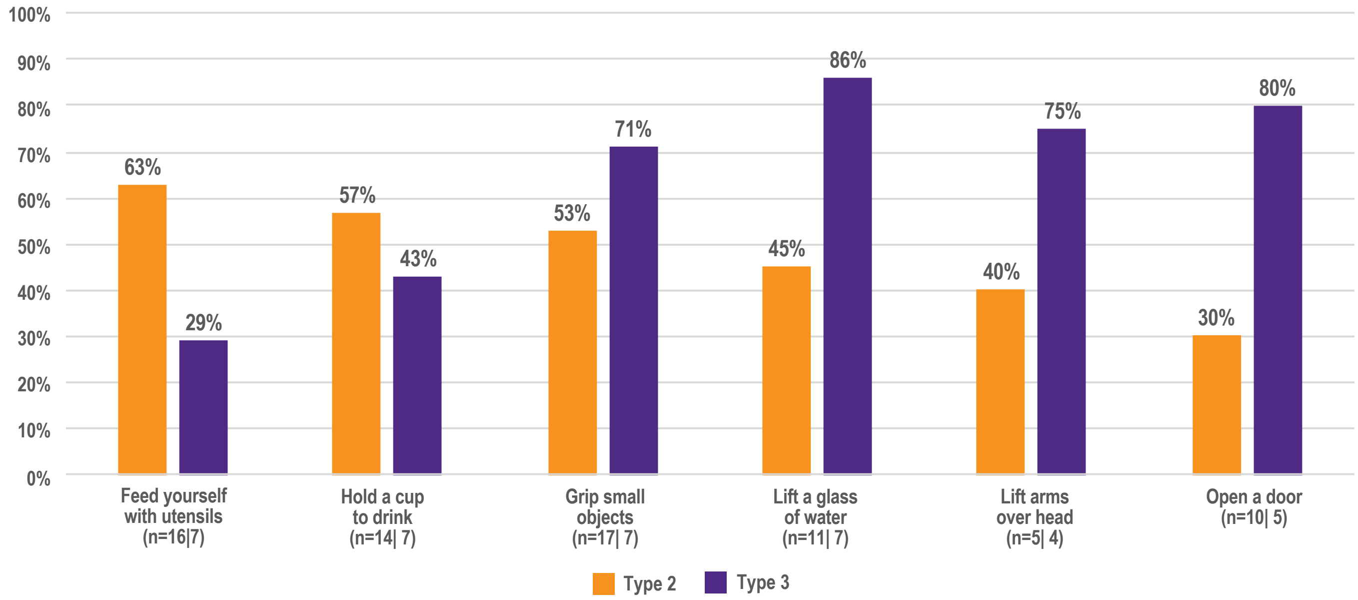
STABILIZATION

Which ADLs have shown stabilization among adults living with SMA on continuous treatment over the past 12 months?



DECLINE

Which ADLs have declined over the past 12 months among those never on treatment?



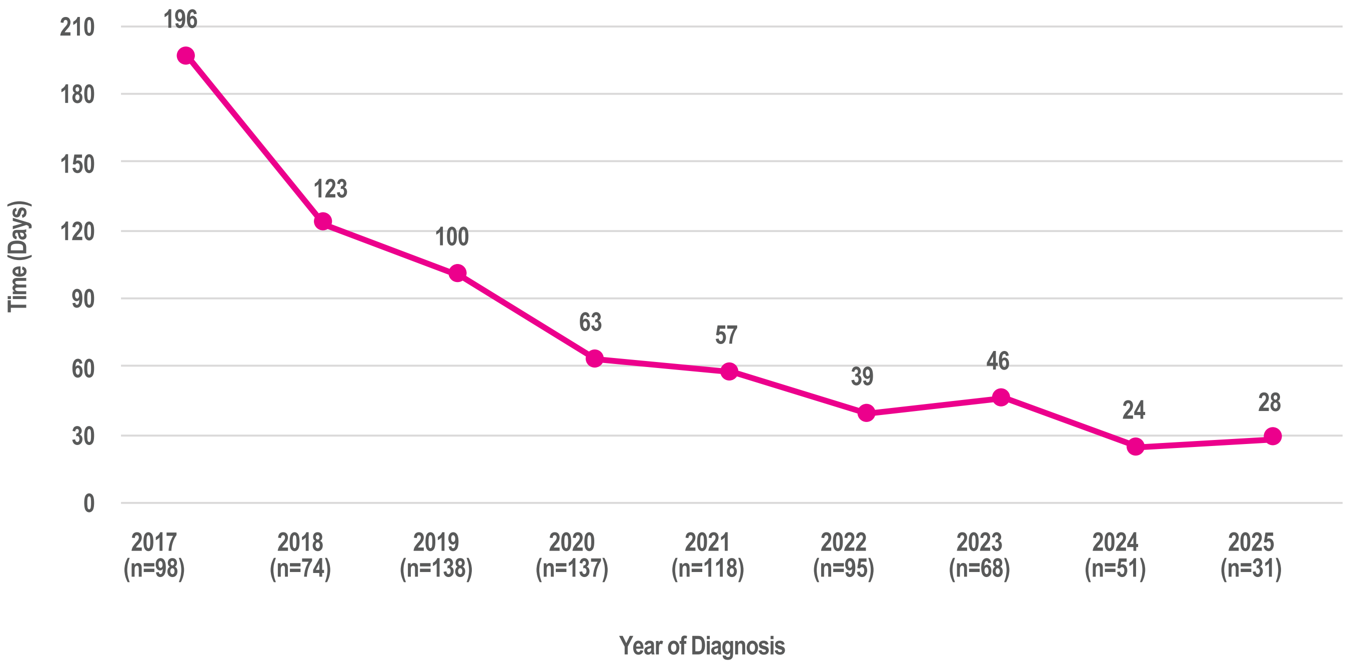
1. Glascock J, et al. A qualitative, mixed-method approach to reaching consensus on function, fatigue, and fatigability outcomes in teens and adults living with spinal muscular atrophy. Orphanet J Rare Dis. 2025 Oct 17;20(1):519.

Analysis Notes:

- Data from the 2025 Community Update Survey.
- Analysis was limited to those that indicated they had been able to perform each ADL at any given time.

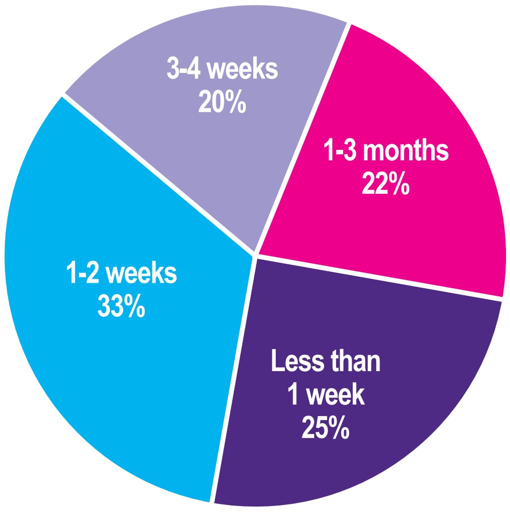
In our data, we observed a decrease in time between diagnosis and first treatment since 2017. For individuals diagnosed with SMA in 2025, the average time between diagnosis and first treatment was 28 days.

Average Time between Diagnosis and First SMA Treatment[1-4]



However, some newly diagnosed families report experiencing massive delays - typically due to insurance denials or review time.

Reported Time from Diagnosis to First Treatment[5] (n=60)



Primary reason for delay:
- **42% insurance denial**
- **42% insurance review process**

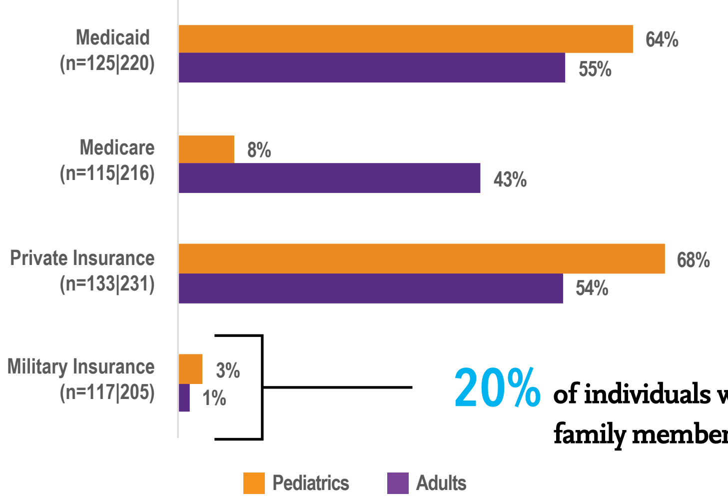
> “Waiting on insurance drastically changed [child]’s future and our lives, and I hope other children won’t have to go through this in the future.”
>
> - Parent of a 9 month old child with SMA

1. The minimum and maximum values were removed from each year.
2. Includes SMA treatments that were FDA approved as of 12/31/2025.
3. Graphs includes individuals who have not participated in clinical trials and where both diagnosis and treatment dates are known.
4. Analyses include individuals where the date of diagnosis occurred on or before the date of first treatment.
5. Data from SMA CORE.

ACCESS

INSURANCE TYPES

Types of Insurance Among Pediatrics and Adults with SMA[1,3,4]



20% of individuals with SMA have an immediate family member who has served in the military

> **“The struggle of insurance is a part-time job.”**
>
> - Adult with Type 2 SMA

98% of individuals with SMA reported having health insurance in 2025.

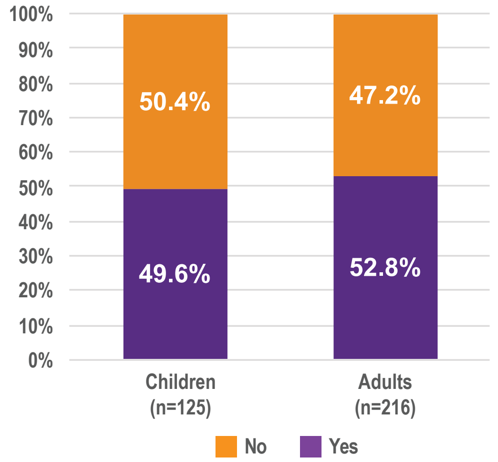
43% of individuals had more than one form of insurance.

INSURANCE DENIALS AND APPEALS

About half of children and adults have reported ever receiving an insurance denial for a prescribed SMA treatment. Prescribing physicians have also expressed both positive and negative experiences when dealing with coverage for their patients.

Patient Experience with Insurance[1]

Ever received an insurance denial for an SMA treatment?



Physician Experience with Insurance[2]

“[Insurance companies] only make the process more frustrating”

- Primary care provider from private practice

“Problems, especially only getting one dose of nusinersen approved at a time, which leads to repeated delayed doses.”

- Pediatric neurologist from an academic medical center

“Over the years, approvals have become more routine and there are fewer barriers”

- Pediatric neurologist from an academic medical center

“We’ve been successful - so far. Not for lack of providers trying!”

- Pediatric neurologist from academic medical center

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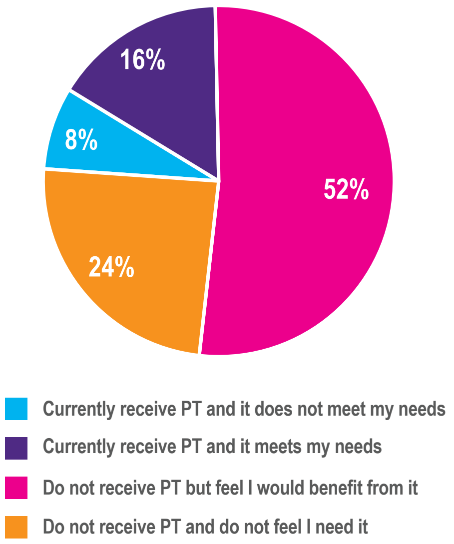
1. Data from the 2025 CUS.
2. Data from the HCP Treatment Decision Making Survey.
3. Insurance categories are not mutually exclusive.
4. Categories in insurance coverage of “Don’t exclusive” and “Not applicable” were excluded from the graph, but were used in calculating proportions.

PHYSICAL THERAPY (PT)

In the Adult Unmet Needs Survey, over half (52%) of adult respondents reported they do not currently receive PT but feel that they would benefit from it. When asked what challenges they've experienced when trying to receive PT, 60% reported their insurance only covers a limited number of sessions, and 55% reported having trouble finding a PT knowledgeable about SMA.

What is your current experience with physical therapy (PT)?[1] (n=263)



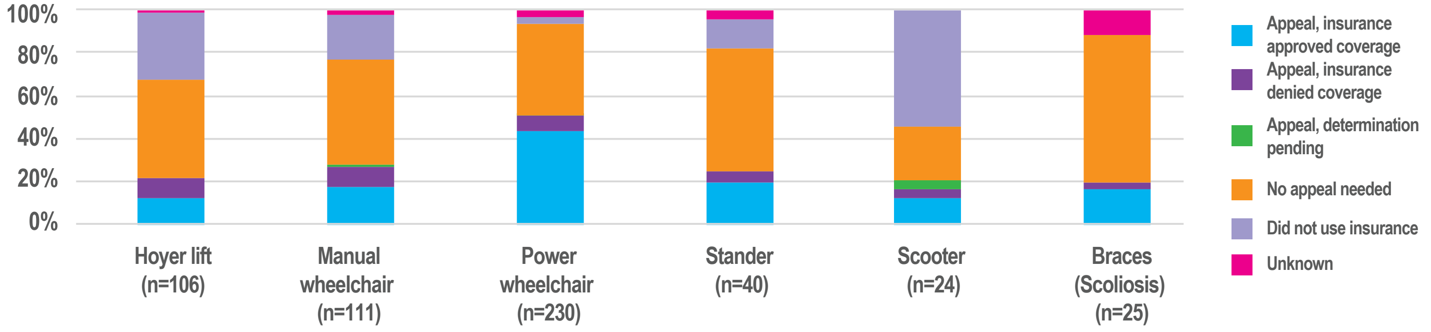
Adults who do not currently receive PT but feel that they would benefit from it (n=137) reported the following challenges[1,3]:

- Insurance only covers a limited number of sessions (60%)
- Trouble finding therapists who are knowledgeable about SMA (55%)
- Need in-home PT, but haven't found a provider (28%)
- Want to try other types of PT, but they are not available near me (27%)
- PT is too far or difficult to get to (26%)
- Insurance denied coverage for PT (23%)

DURABLE MEDICAL EQUIPMENT (DME)

78% of individuals reported using one or more forms of DME for their SMA[4]. Among those, 49% reported receiving an insurance denial. Insurance denials were most frequently reported for power wheelchairs.

Outcomes for Durable Medical Equipment[2]



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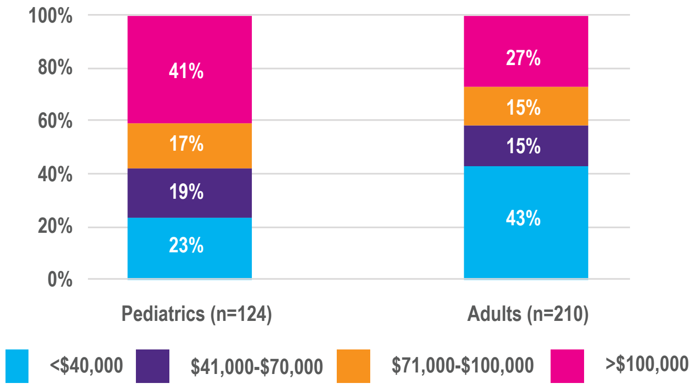
1. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.
2. Data from the 2025 CUS.
3. Options are not mutually exclusive; respondents were able to "select all that apply".
4. Restricted to individuals who indicated usage for Hoyer lift, manual wheelchair, power wheelchair, stander, scooter, and braces for scoliosis only.

SOCIAL DETERMINANTS OF HEALTH

HOUSEHOLD INCOME

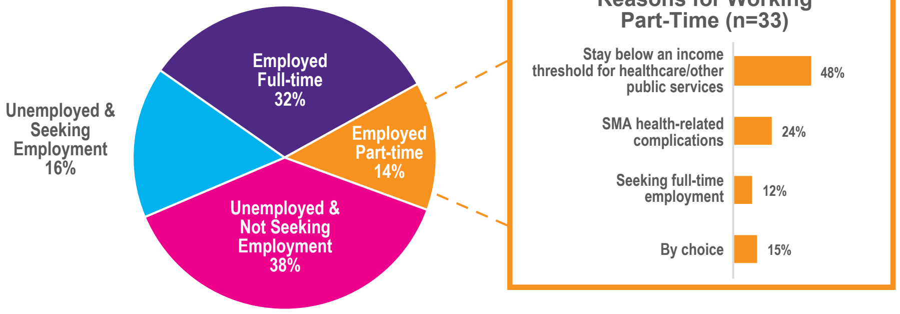
Household Income Among Pediatrics and Adults



Household income significantly influences access to resources and overall living conditions, with lower income associated with worse health outcomes. A greater proportion of adults are living in household making < $40,000 annually compared to pediatrics.

EMPLOYMENT

Employment Status Among Adults 18 Years and Older (n=242)

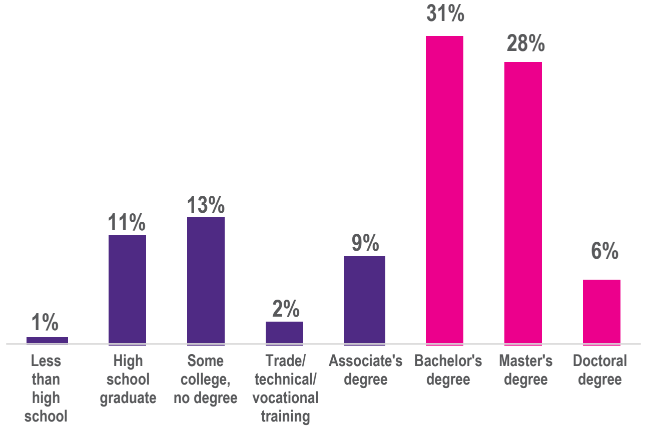


The proportion of adults with SMA that report working part-time in order to remain below income thresholds for healthcare or other public services increased from 31% in 2023, to 43% in 2024, and reached 48% in 2025.

EDUCATION

65% of adults with SMA aged 25 years and older have gone on to complete a bachelor's degree or higher. This is higher than the U.S. Census estimate of 36.2% for the general population.[1]

Highest Level of Education Completed in Adults 25 Years and Older (n=215)



1. Education attainment levels from U.S. Census data. https://data.census.gov/table/ACSST1Y2023.S1501?q=Educational%20Attainment.

Analysis Notes:

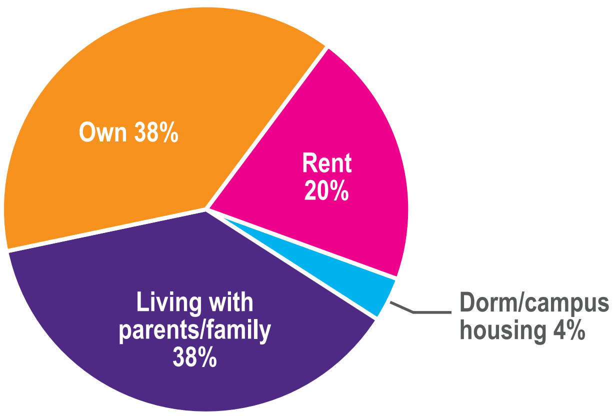
- Data from the 2025 CUS.

SOCIAL DETERMINANTS OF HEALTH

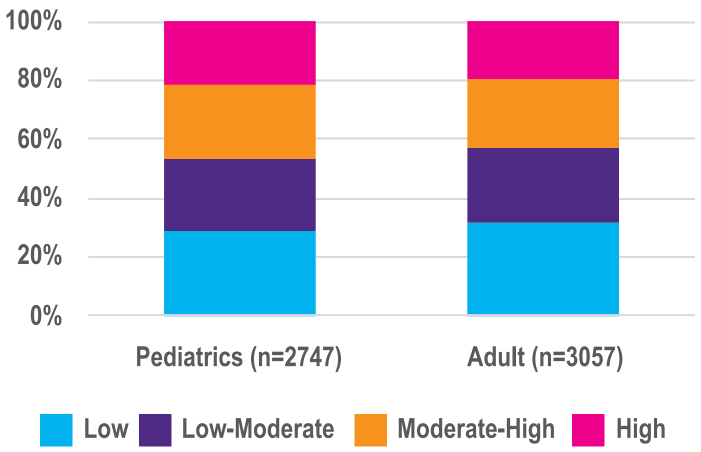
HOUSING

Most adults report either living with parents or family or in a home that they own. Additionally, roughly 20% of both children and adults with SMA are living in a zip-code area with high poverty levels.

Housing Among Adults[1] (n=226)



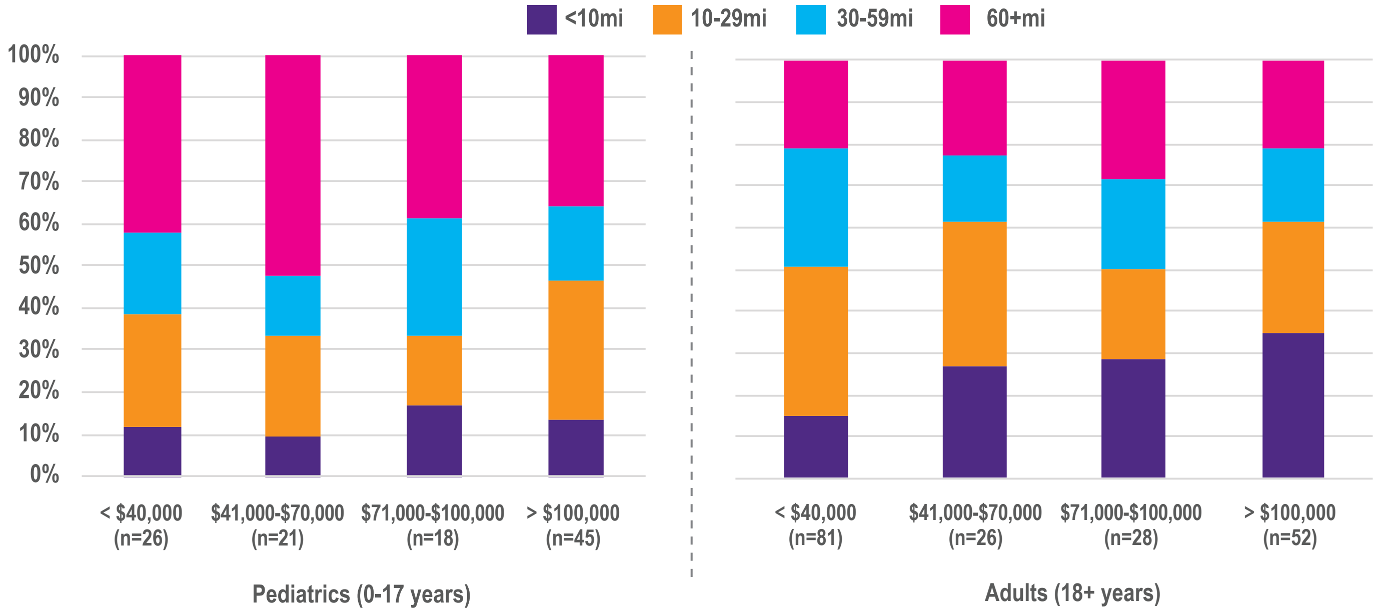
Zip-code Area Poverty Levels by Age[2,3]



DISTANCE TO SMA CARE

Overall, children under 18 and their families were found to travel further distances to SMA-related care compared to adults. When this was examined by household income, children were still shown to travel further to their care across household income levels when compared to adults.

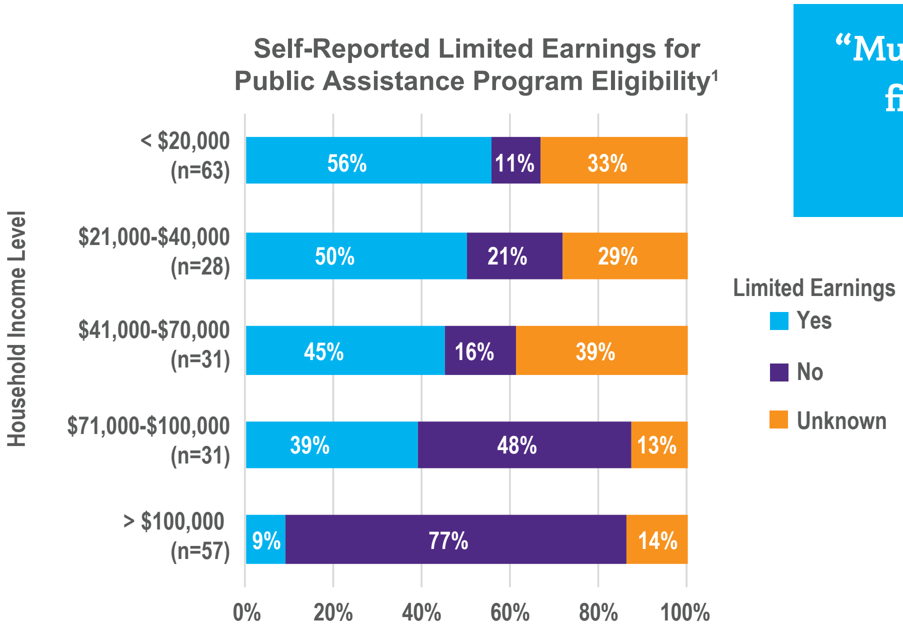
Distance Traveled (One-way) to SMA Care by Age and Household Income[1]



1. Data from the 2025 CUS.
2. Data from the membership data, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.
3. Zip-code level poverty level determined by social deprivation index (SDI) data provided the Robert Graham Institute. https://www.graham-center.org/maps-data-tools/social-deprivation-index.html.

FINANCIAL STRESS

PERSONAL EARNINGS


Self-Reported Limited Earnings for Public Assistance Program Eligibility[1]

> “Much of my stress revolves around the financial aspect of the disability.”
>
> - Adult with Type 2 SMA

46% of adults reported their earnings are limited to remain eligible for public assistance programs. Most adults who report their earnings are limited have a household income < $20,000 annually.

COST OF TREATMENT

50% of affected adults and children who have never received an SMA treatment (n=14) report the cost of medication being too expensive as a reason for never receiving treatment.[1]

51% of affected adults on current treatment (n=214) report feeling concerned about the financial costs of treatment and/or their insurance not covering the cost of treatment.[2]

CAREGIVER ASSESMENT OF FINANCIAL IMPACT[1]

47%

of caregivers report the cost of doctor's visits to have a moderate to extreme financial impact.[1,3]

46%

of caregivers report child and dependant care to have a moderate to extreme financial impact.[1,3]

46%

of caregivers report lost income due to child's diagnosis to have a moderate to extreme financial impact.[1,3]

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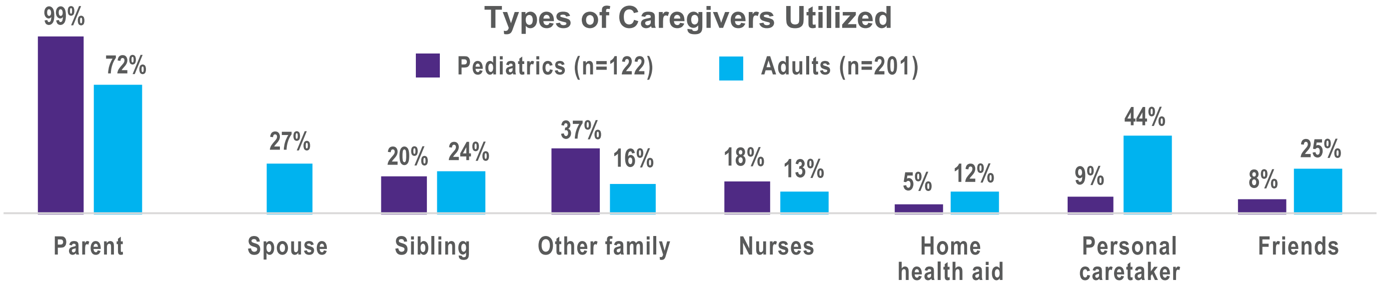
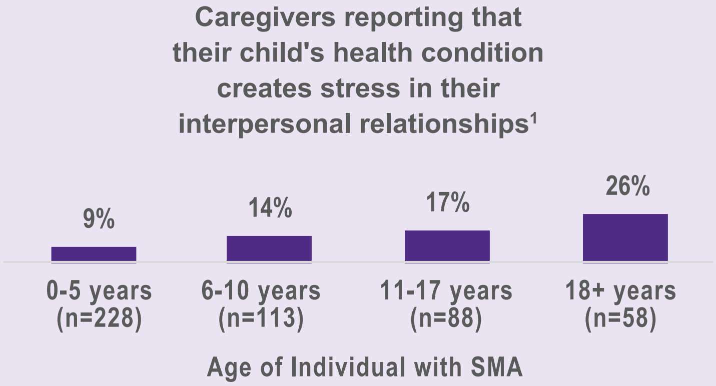
1. Data from the 2025 CUS.
2. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.
3. Data from the Assessment of Caregiver Experience with Neuromuscular Disease (ACEND).

87% of individuals with SMA report having one or more caregiver. Caregiving teams are diverse, with many individuals supporting those with SMA.



IMPACT ON INTERPERSONAL RELATIONSHIPS

While every journey is different, caregiving may have impacts on family dynamics and interpersonal relationships.



For caregivers of individuals 0-5 years, the percentage was lower for those who cared for an individual identified by newborn screening (5%).

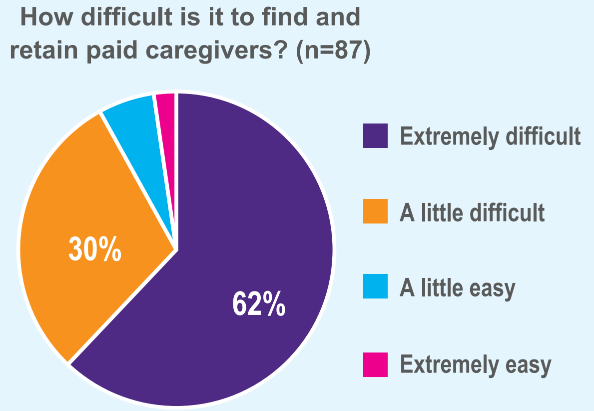
Regardless of what your journey has looked like, having a community to lean on can make a meaningful difference.

> "[in response to resources needed] More local SMA community, but the connections from conference have been life giving."
>
> -Parent of a 6 year old child with SMA

A few ways to get connected can be found on the last page of this report!

DIFFICULTY RETAINING PAID CAREGIVERS

Adults reported greater utilization of caregivers outside of the immediate family compared to pediatrics (60% vs. 32%)



Many adults living with SMA have expressed difficulty and frustration with finding, keeping, and affording qualified caregivers to help support their care. This difficulty in finding and retaining paid caregivers has been reported regardless of weekly caregiving needs.

> "The ability to find and maintain caregivers is a tremendous burden…. I have grave concerns as my spouse ages about continuing to receive adequate care in my home."
>
> - Adult with Type 2 SMA

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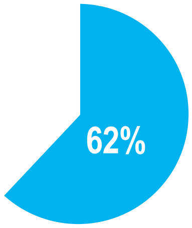
1. Percent of caregivers that responded "Often" or "Always".

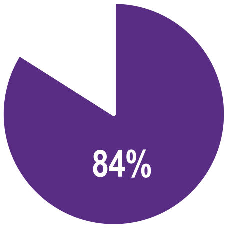
Analysis notes:

- Data from the 2025 CUS.

UNMET NEEDS OF THE CAREGIVER



of parents reported the emotional stress and anxiety about their child's health and future is the most challenging aspect of caregiving for a child with SMA.[1]



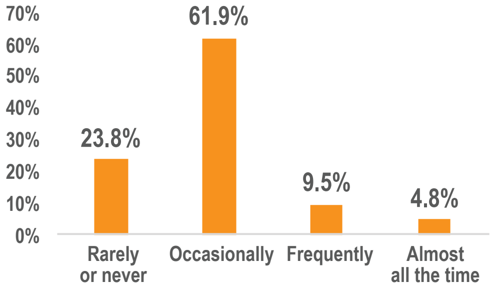
of parents of children with SMA reported the lack of adaptability of public places for disability is the most significant unmet need they currently face when caring for a child with SMA.[2]

> "We aren't superheroes... we are parents taking care of their kids."
>
> -Parent of a 6-year-old child with SMA.

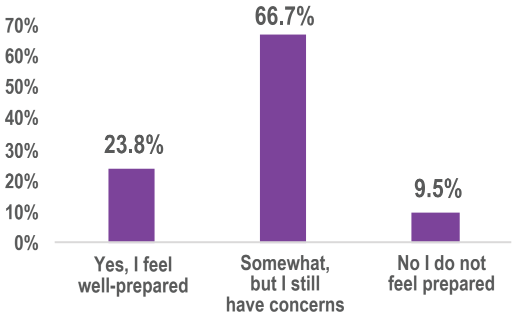
CARING FOR AN ASYMPTOMATIC CHILD

35% of children with SMA under the age of 7 years are reported to be currently asymptomatic by their parents. However, despite their children not showing any symptoms of SMA, parents report worrying about their child developing symptoms related to SMA.[2]

How often do you think about or worry about your child developing symptoms?[3]



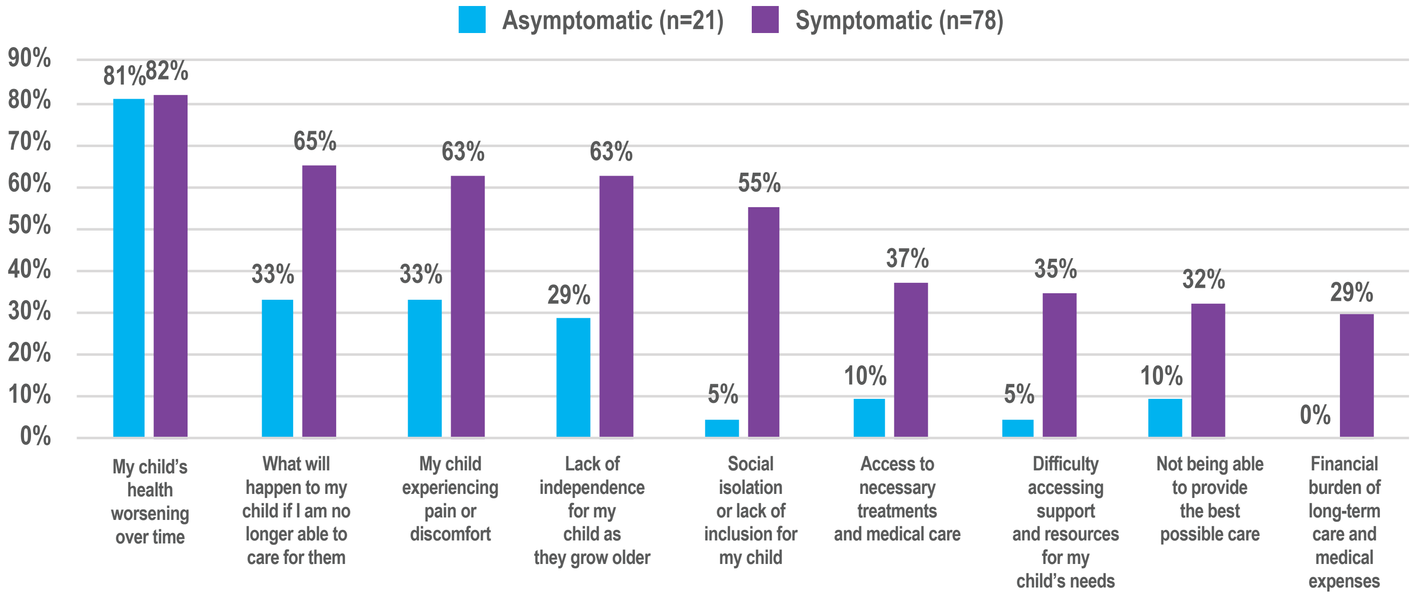
Do you feel adequately prepared for the possibility of your child developing symptoms in the future?[3]



BIGGEST FEARS FOR CHILD'S FUTURE

Regardless of a child showing symptoms related to SMA, parents cite that their biggest fear is their child's health worsening over time.

Fears Faced by Parents of Children Living with SMA by Symptom Status of the Affected Child



1. n=129, parent of a child less than 18 years of age.
2. n=95, parent of a child ages 3-17.
3. n=21, parent of a child ages 3-17.

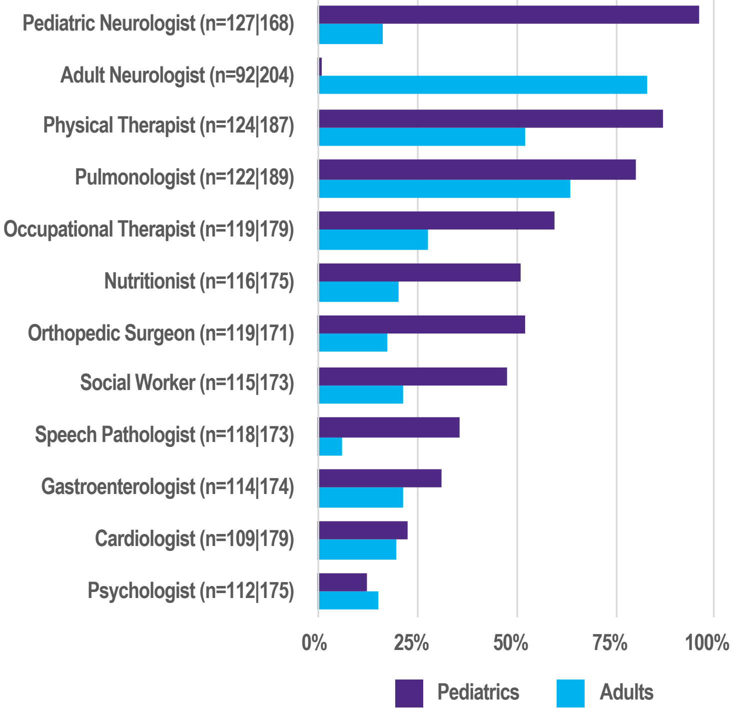
Analysis Notes:

- Data from the 2025 CUS.

SMA CARE TEAM

In 2025, 93% of pediatrics with SMA and 86% of adults with SMA reported having an in-person appointment with a physician or specialist for SMA-related care.

Types of Specialists Seen Among Pediatrics and Adults Living with SMA[1]



The average number of specialists within a pediatric care team was 4.5, while the average number of specialists in an adult care team was 2.8.

? Is your neurologist able to spend enough time with you during appointments?[2]

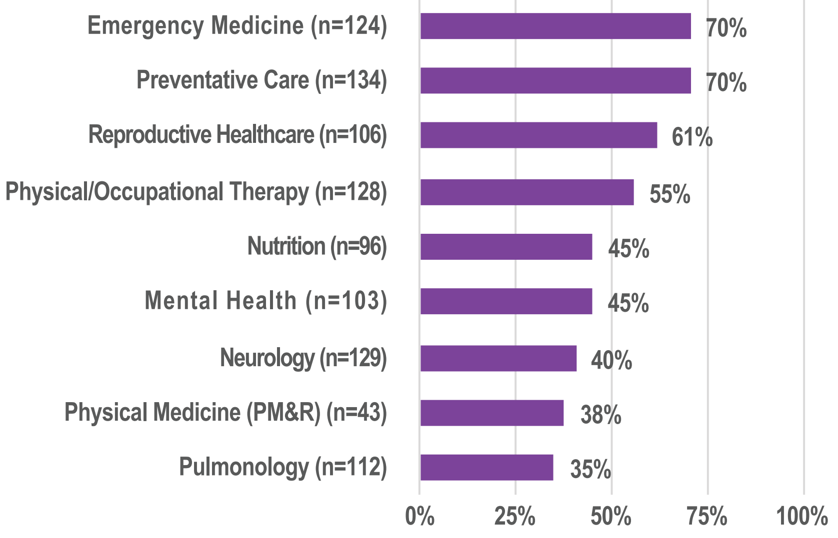
When surveyed, 74% of adults reported that their neurologist was often or always able to spend enough time with them during appointments and address their concerns.

UNMET HEALTHCARE NEEDS

> “It is exhausting to have to advocate and educate the people who are in charge of our healthcare.”
>
> -Adult with Type 3 SMA.

54% of adults living with SMA report that they currently experience incomplete or inadequate healthcare services due to their SMA. The most frequently reported specialties in which this occurs include Emergency Medicine, Preventative Care, and Reproductive healthcare.

Specialties Where Adults Reported Experiencing Incomplete or Inadequate Care[2]



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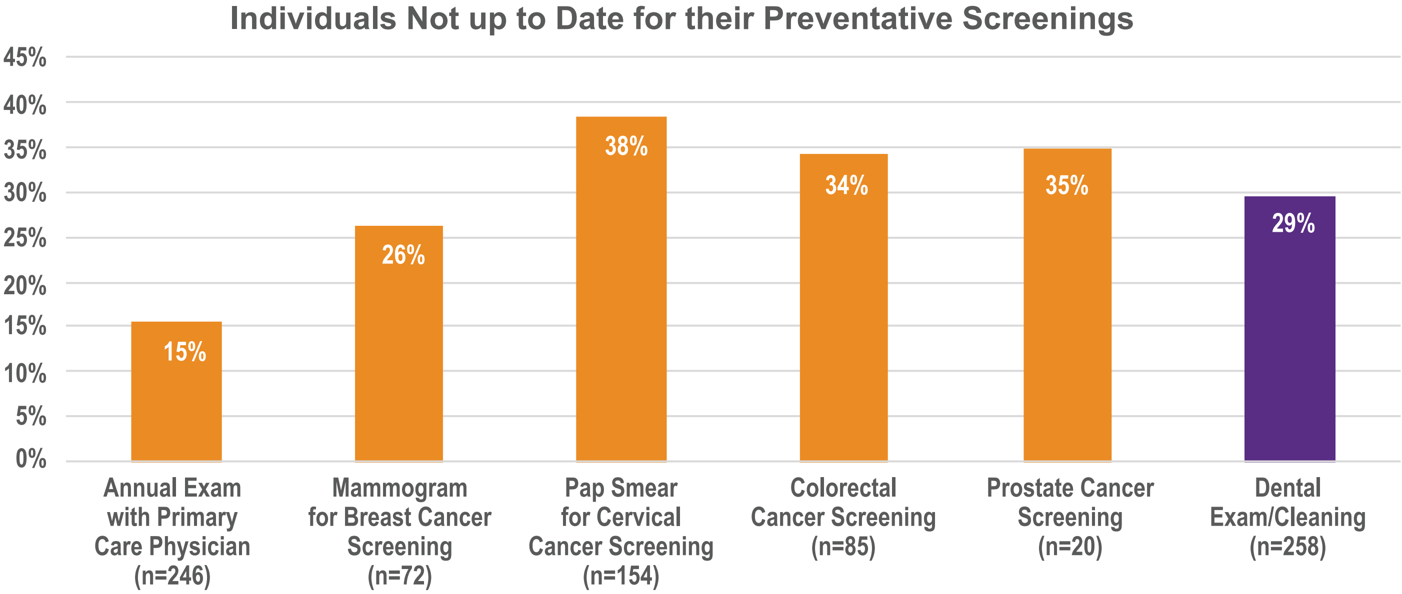
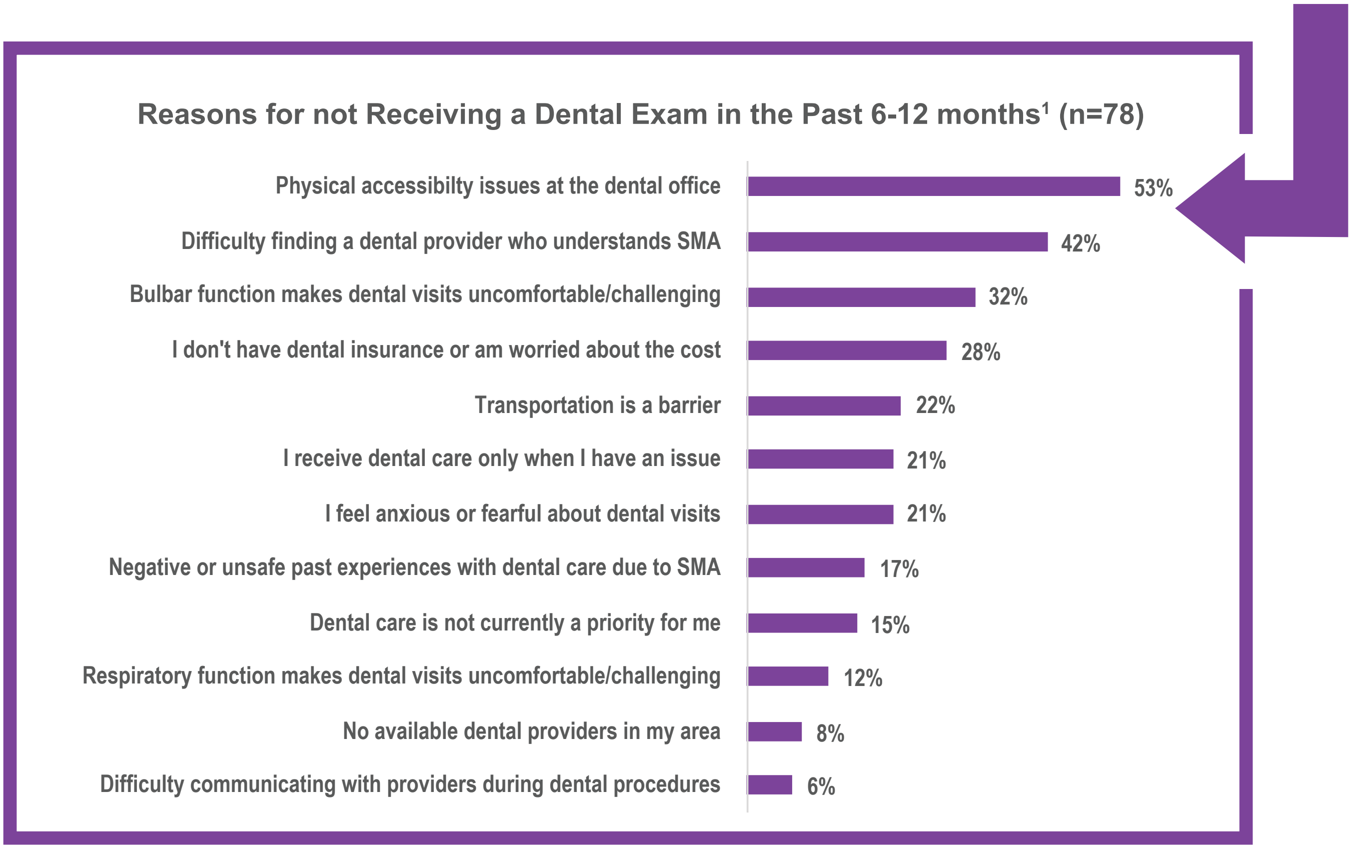
1. Data from the 2025 CUS.
2. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

SMA CARE TEAM: PREVENTATIVE HEALTHCARE

The majority of adults with SMA report they are up-to-date on appropriate preventative healthcare appointments and screenings. Pap smears and colorectal cancer screenings had the greatest proportions of those not up to date. The top reported reasons why individuals may not be up-to-date on their appointments and screenings were related to having not felt the need to get the screening and physical accessibility issues due to SMA.





1. Options are not mutually exclusive, respondents able to “select all that apply”.

Analysis Notes:

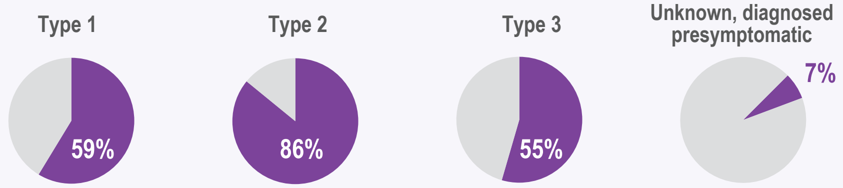
- Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

MUSCULOSKELETAL CONDITIONS

In the Community Update Survey, a high proportion of individuals with SMA report musculoskeletal conditions, including scoliosis and hip dysplasia. The highest rates of scoliosis and hip dysplasia were observed in individuals with Type 2 SMA, and age at diagnosis increased as SMA type increased.

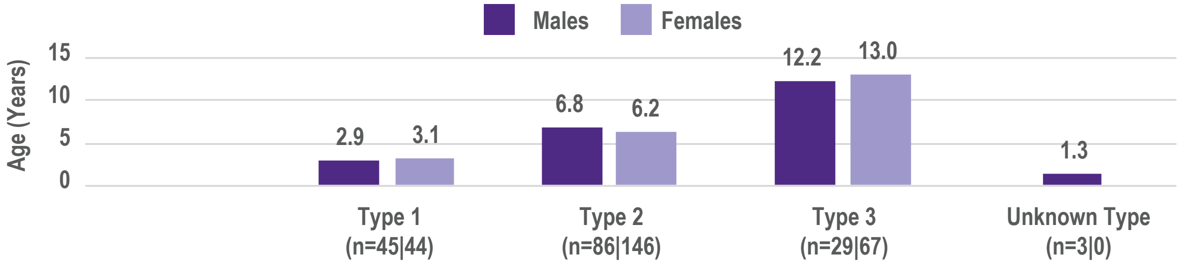
SCOLIOSIS

Percent of Individuals with SMA Reporting a Diagnosis of Scoliosis – All Ages[1]



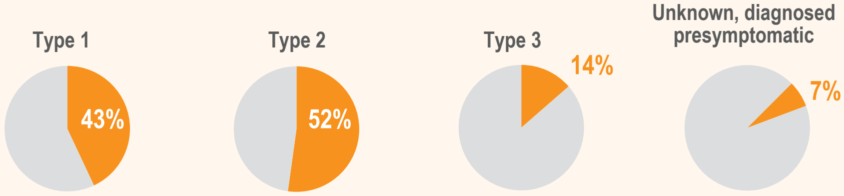
n	179	333	222	44
Mean age of sample	10.3	26	36.4	2.6

Mean Age of Scoliosis Diagnosis, by SMA Type and Gender



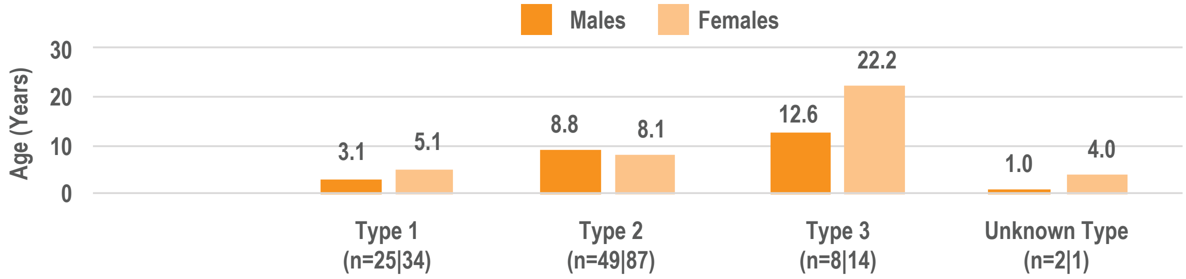
HIP DYSPLASIA

Percent of Individuals with SMA Reporting a Diagnosis of Hip Dysplasia – All Ages[1]



n	172	316	213	44
Mean age of sample	10.3	25.8	36.5	2.6

Mean Age of Hip Dysplasia Diagnosis, by SMA Type and Gender



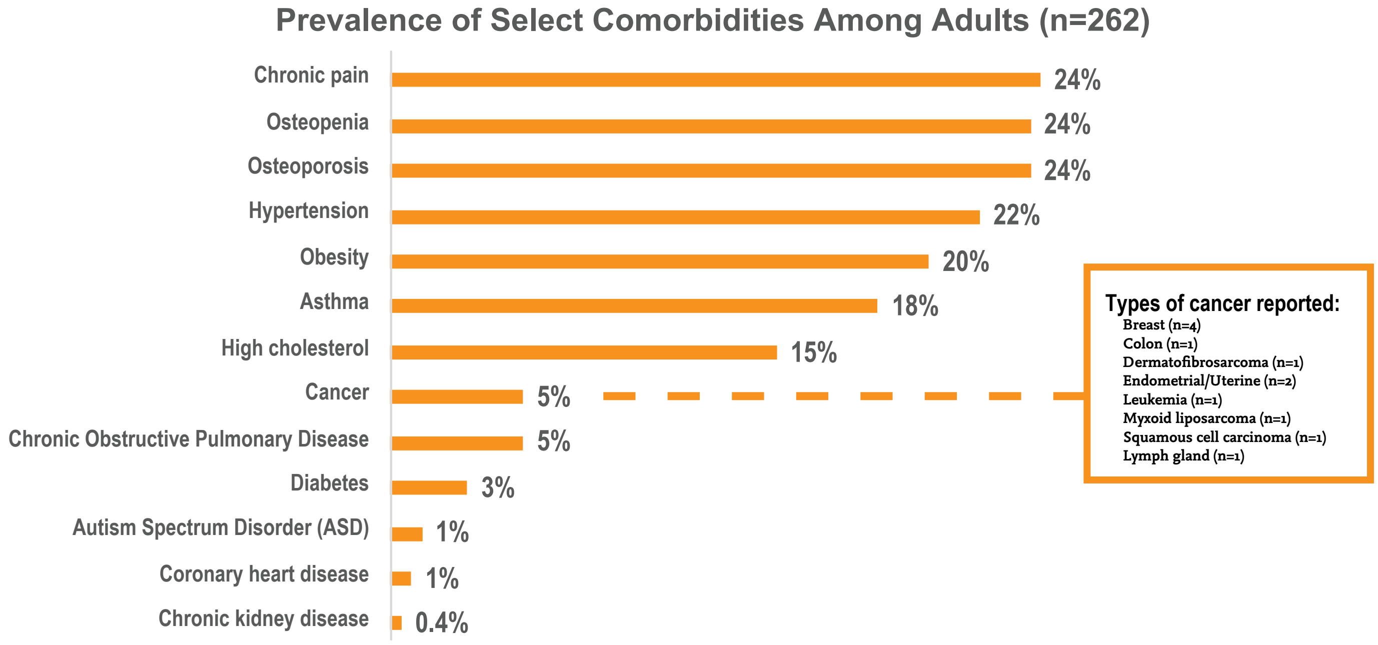
1. Denominator included individuals who answered a question in the comorbidity module; excluded individuals that reported “Don’t know” for scoliosis or hip dysplasia.

Analysis Notes:

- Data sourced from 2024-2025 CUS. Individuals participating in multiple years were de-duplicated.

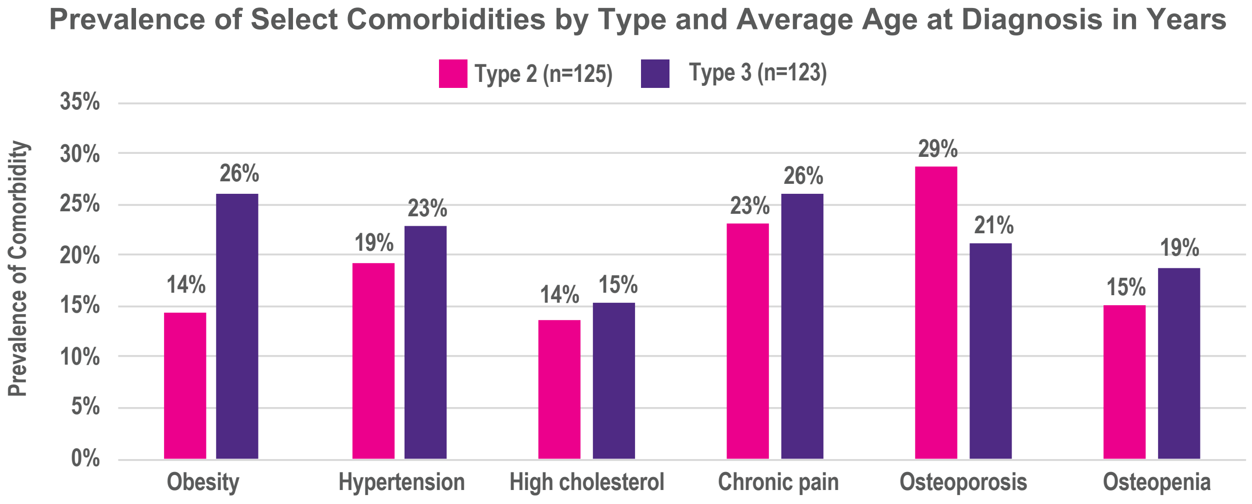
COMORBIDITIES IN ADULTS

In the 2025 Unmet Needs Among Adults Across the Healthcare Spectrum Survey respondents reported a variety of diagnosed comorbidities, with chronic pain (24%), osteopenia (24%), osteoporosis (24%), hypertension (22%), and obesity (20%) having the greatest prevalence rates.



Types of cancer reported:
- Breast (n=4)
- Colon (n=1)
- Dermatofibrosarcoma (n=1)
- Endometrial/Uterine (n=2)
- Leukemia (n=1)
- Myxoid liposarcoma (n=1)
- Squamous cell carcinoma (n=1)
- Lymph gland (n=1)

Select comorbidities were examined by type of SMA and age at diagnosis. Slight differences in prevalence were observed between Type 2 and Type 3 SMA. Additionally, on average those with Type 3 were diagnosed with comorbidities at a later age that those with Type 2 SMA.



Average Age at Diagnosis, in years

Type 2	15.6	30.8	34.4	14.0	19.1	11.0
Type 3	27.3	36.1	36.1	58.0	39.8	17.9

Analysis Notes:

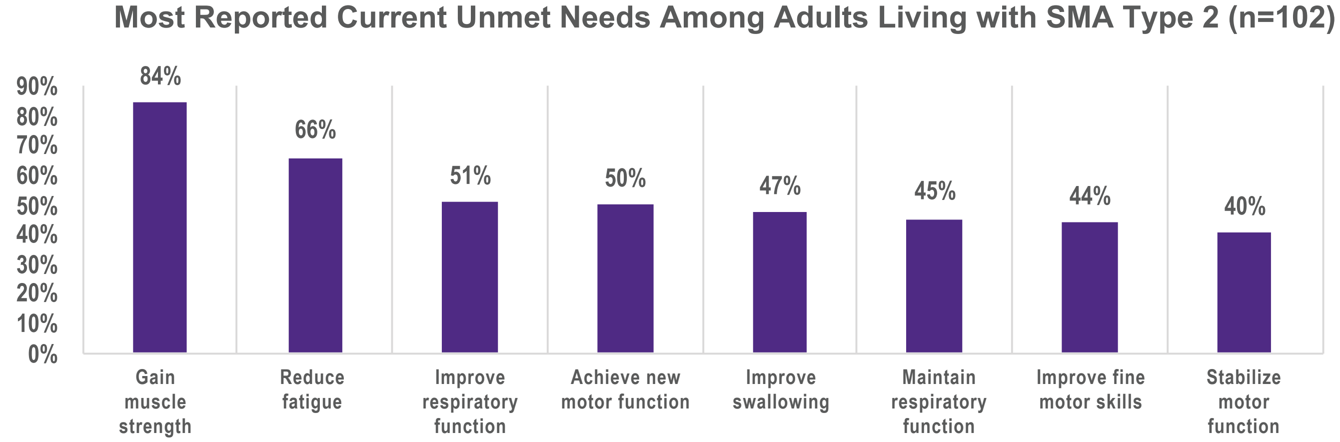
- Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

UNMET NEEDS OF ADULTS

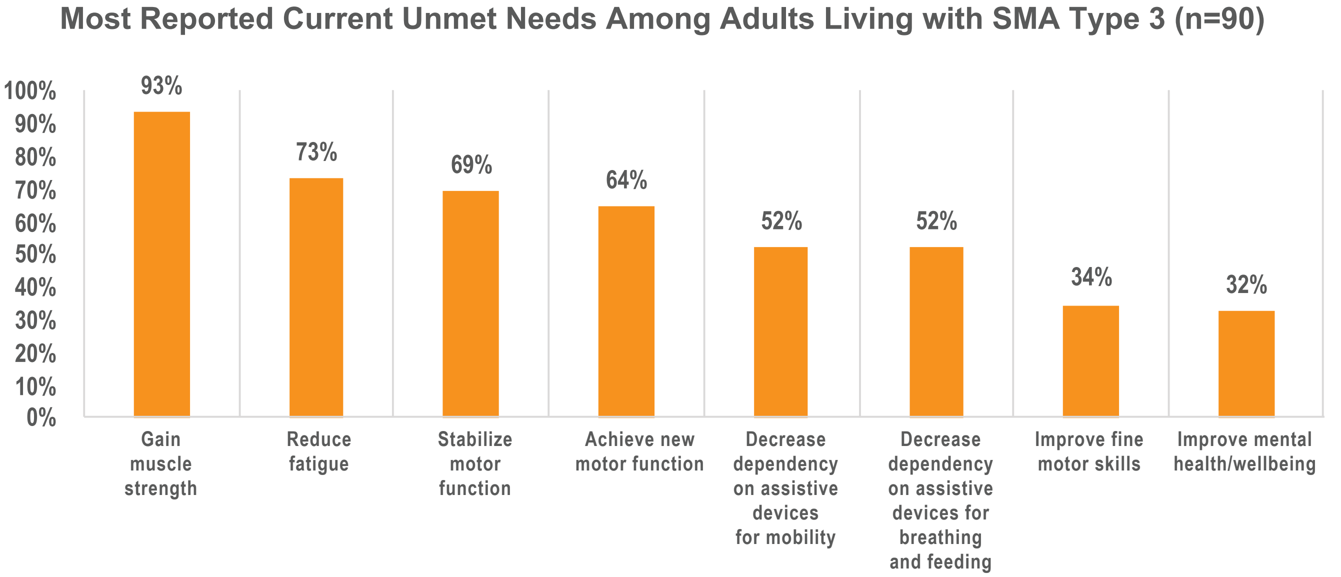
> **“I feel like I have to put in two times the effort in order to produce a result that is the equivalent to the regular output of non-disabled people.”**
>
> - Adult with Type 2 SMA

Each year adults living with SMA are asked in the annual Community Update Survey, what are your most significant unmet needs you hope future therapies will address. “Gain muscle strength” and “reduce fatigue” were the most commonly reported unmet needs among both SMA Type 2 and Type 3.

TYPE 2



TYPE 3



Analysis Notes:

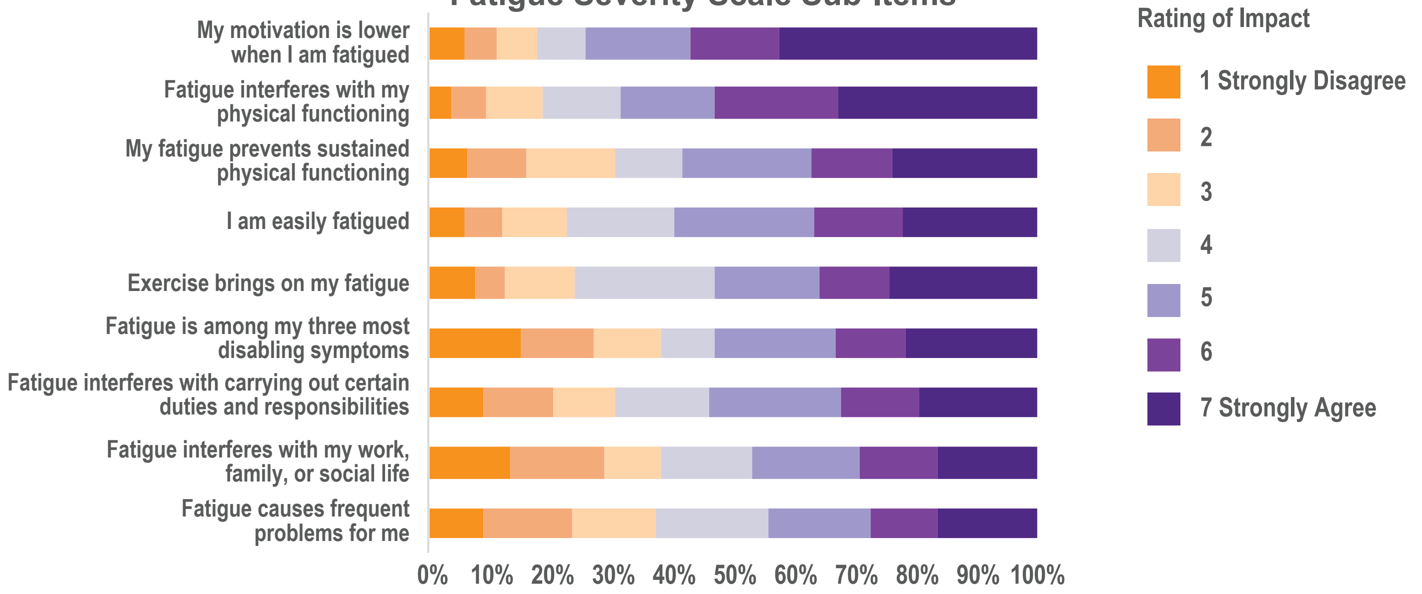
- Data from the 2025 CUS.
- Categories are not mutually exclusive.
- SMA Type 1 & Type 4 excluded due to small sample size.

FATIGUE

Fatigue is a commonly reported symptom of SMA. The Fatigue Severity Scale (FSS) is a short, self-report questionnaire that measures how severe fatigue is and how much it has impacted daily life (work, family, social) over the past week.[1] Higher scores indicate greater fatigue severity. Cure SMA has utilized the FSS in multiple surveys throughout the year to measure fatigue levels in adults living with SMA.

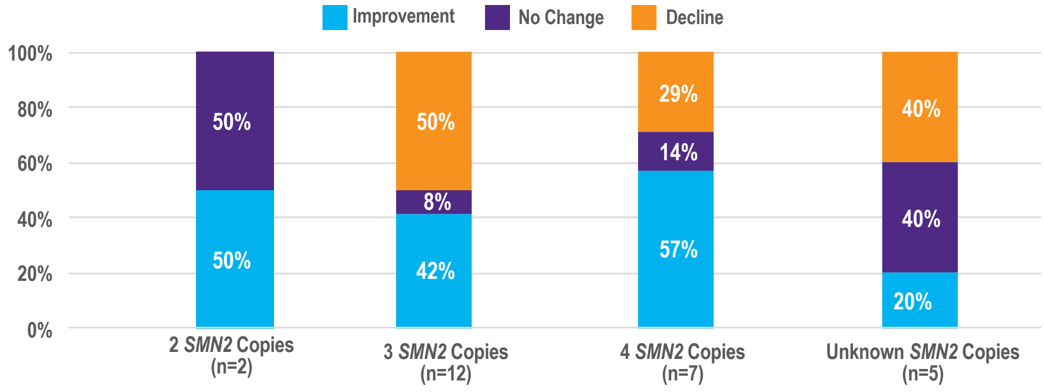
Fatigue levels are in the "severe" category across all SMA types. Highest rated items of fatigue for all types were "My motivation is lower when I am fatigued" and "Fatigue interferes with my physical functioning".

Fatigue Severity Scale Sub-Items[2]



In 2020, Cure SMA hosted a survey evaluating fatigue among adults living with SMA (Fatigue Survey[1]). Changes in fatigue levels were evaluated among adults living with SMA that completed the FSS in both the 2020 Fatigue Survey and again in the 2025 Community Update Survey. Among those that remained on treatment during this period, there was a decrease in fatigue among those with 2, 3 and 4 *SMN2* copies.

Changes in Fatigue Levels Among Those on Treatment[3]

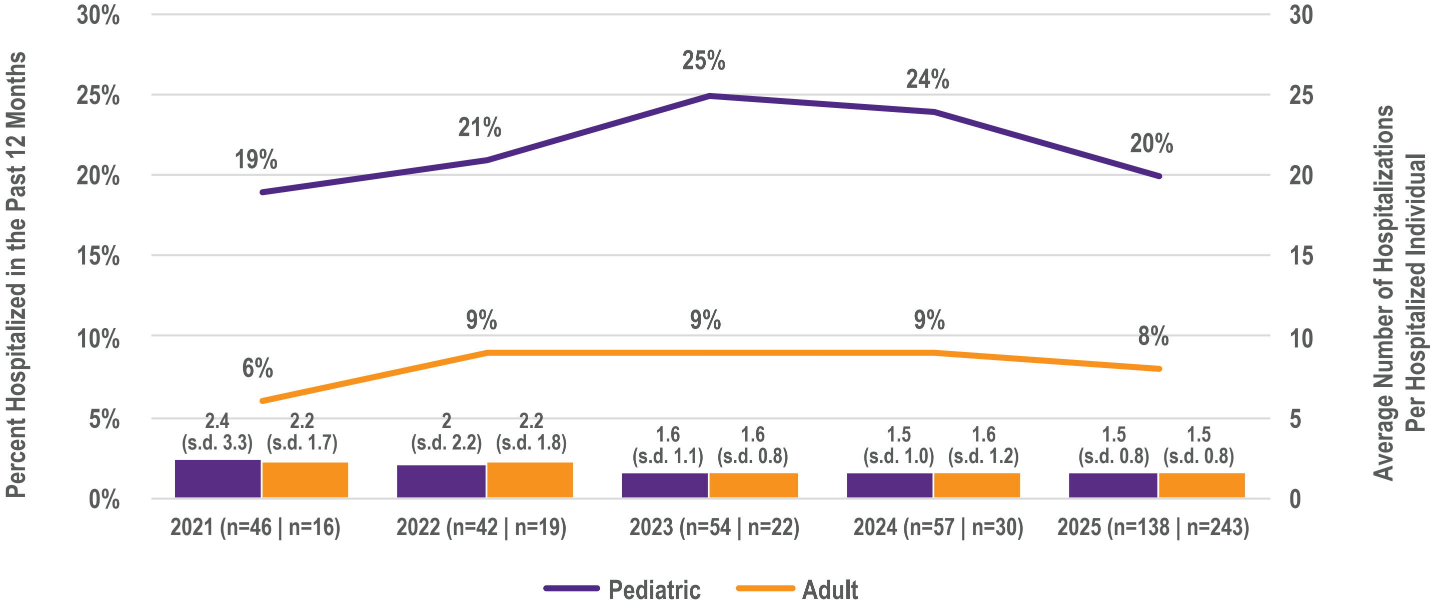


	2 *SMN2* Copies (n=2)	3 *SMN2* Copies (n=12)	4 *SMN2* Copies (n=7)	Unknown *SMN2* Copies (n=5)
Mean change (sd) in FSS total score from baseline to follow-up	-0.56 (0.79)	-0.21 (1.20)	-0.95 (1.54)	0.16 (0.33)

1. Belter et el. Evaluating Perceived Fatigue within an Adult Spinal Muscular Atrophy Population. Neurol Ther. 2023 Dec;12(6):2161-2175.
2. Data from the 2025 CUS.
3. Data from the Cure SMA 2020 Fatigue Survey.

HOSPITALIZATIONS

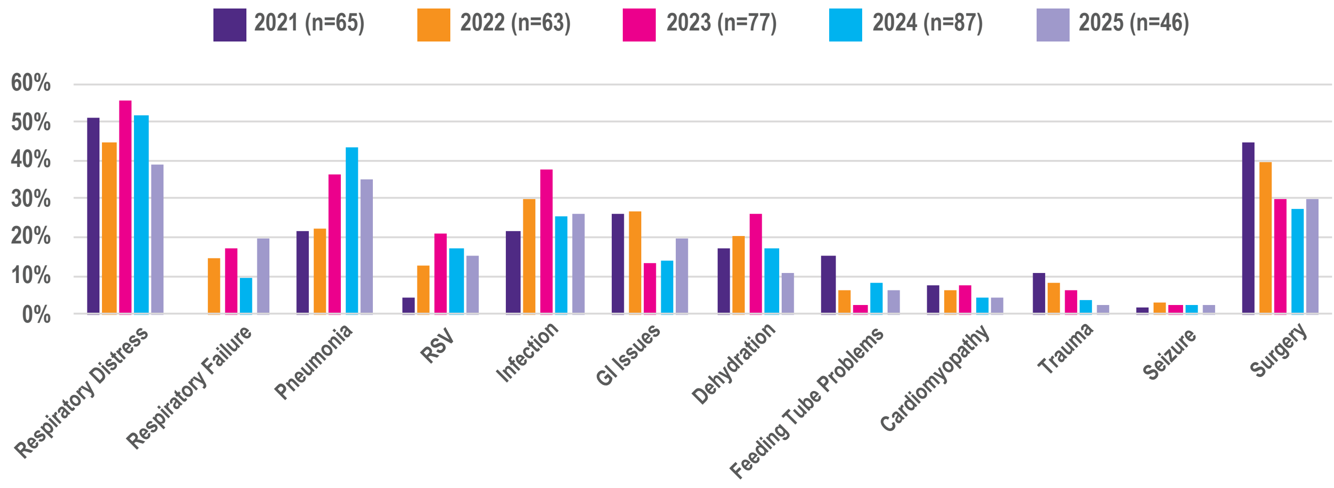
In 2025, 12% of all individuals with SMA reported being hospitalized within the past 12 months. Among the pediatric population, 20% reported a hospitalization, and among adults, 8% reported a hospitalization.



Hospitalization Rate Among Pediatrics and Adults and Average Number of Hospitalizations per Year: 2021-2025[1]

REASONS FOR HOSPITALIZATION

Reasons for Hospitalization 2021-2025



Among individuals who reported being hospitalized within the previous 12 months between years 2021-2025, we have observed a slight increase in the proportion of individuals hospitalized due to pneumonia (22% vs 35%) and RSV (5% vs. 15%), and a decrease in the proportion of individuals hospitalized due to dehydration (17% vs. 11%).

1. This is not truly longitudinal as the same individuals are not included in every time period.

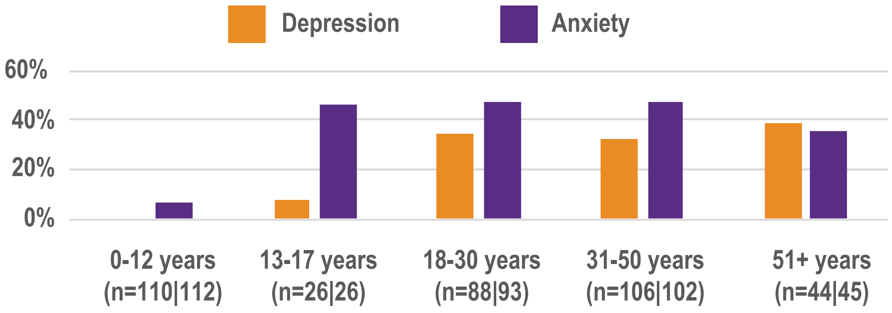
Analysis notes:

- Data from the 2021-2025 CUS.

MENTAL HEALTH

> **“We need more information about mental health specific to the disabled community and people who are familiar with neuromuscular conditions.”**
>
> - Adult with Type 2 SMA

Prevalence of Depression and Anxiety Diagnoses by Age Group[1]



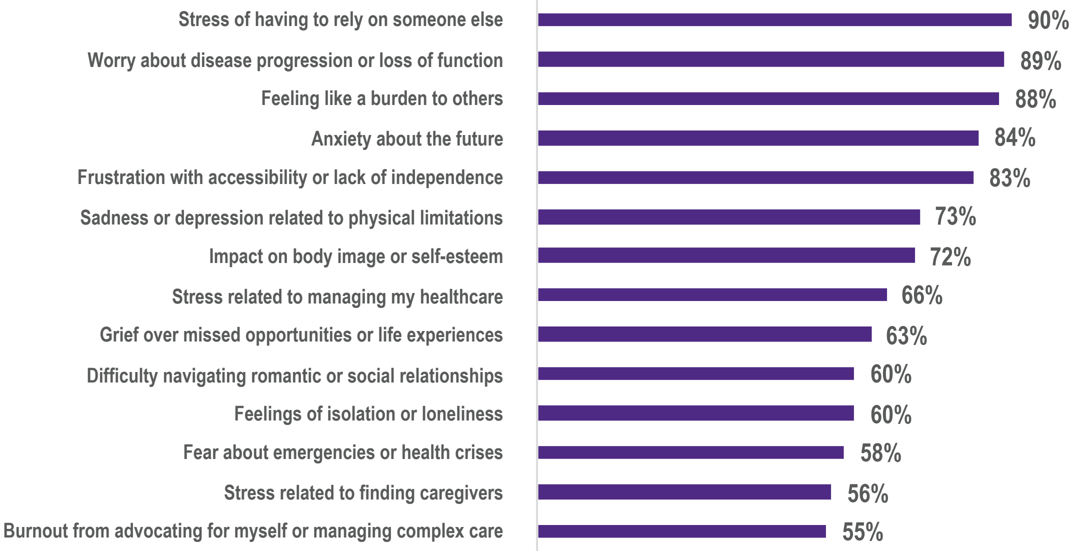
22% of the full population reported ever being diagnosed with depression, and 34% ever diagnosed with anxiety.

The average age at diagnosis of depression was 24.7 years (s.d. 13.1 years), and the average age at diagnosis of anxiety was 23.3 years (s.d. 13.7 years).

UNMET MENTAL HEALTH NEEDS

In the 2025 Unmet Needs Among Adults Across the Healthcare Spectrum Survey, 78% of adult respondents living with SMA reported that living with SMA has impacted their mental or emotional health, with the most reported ways being “stress of having to rely on someone else,” “worrying about disease progression,” and “feeling like a burden to others.”

How has living with SMA impacted your mental or emotional health?[2] (n=204)



In the 2025 CUS, 39% of adult respondents living with SMA (n=218) reported they needed mental health services in the previous 12 months. Most (74%) were able to obtain those services; however, 19% were unable to. The highest reported reason for not obtaining mental health services was not knowing where to go (67%).[1]

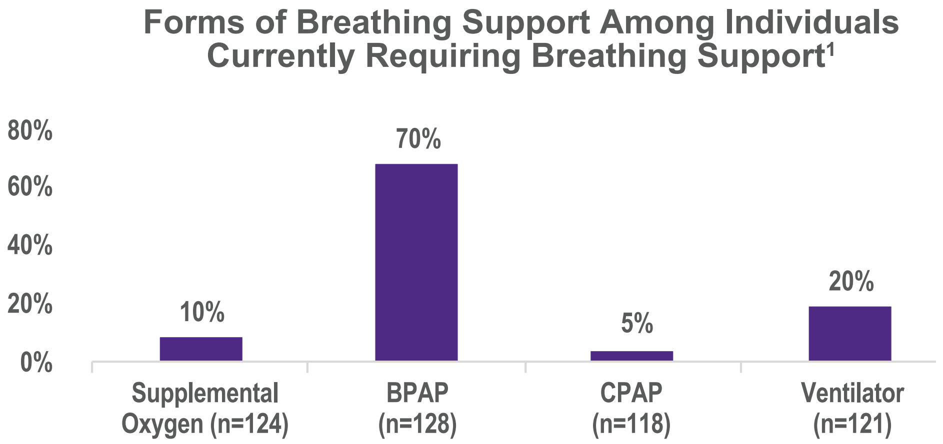
If you are experiencing depression, anxiety, or mental health concerns, please contact your healthcare provider. You are not alone.

---

1. Data from the 2025 CUS.
2. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

RESPIRATORY FUNCTION

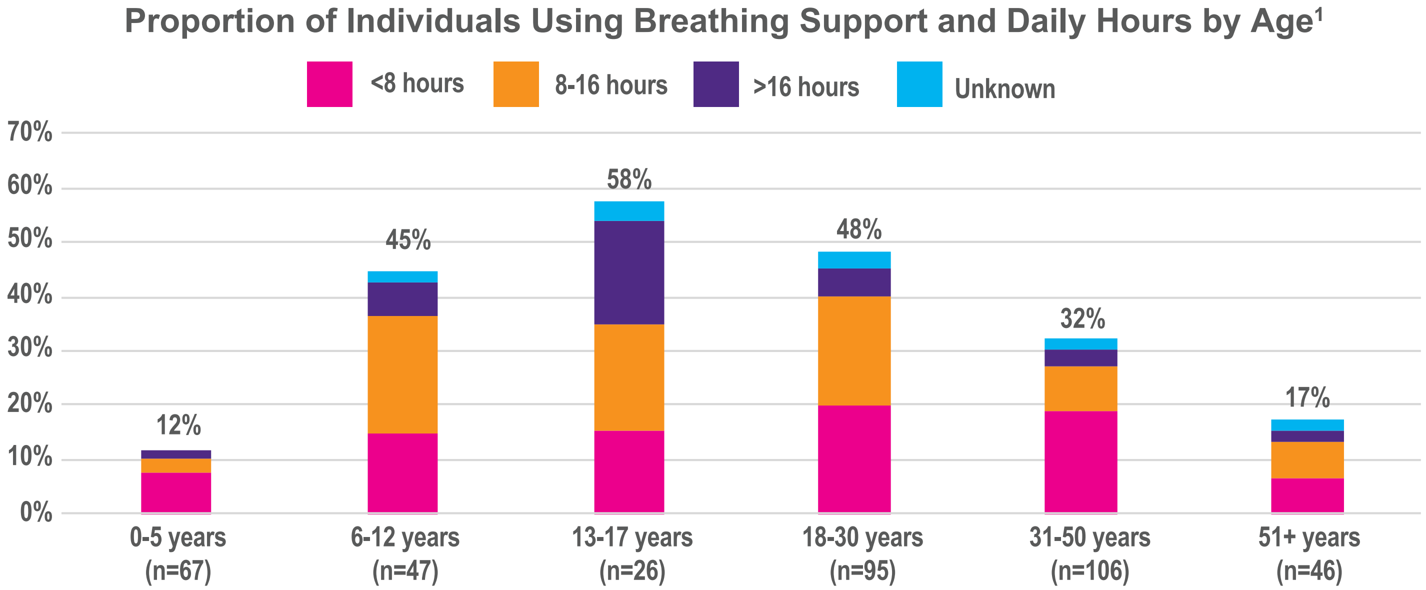
Individuals with SMA often have weakened intercostal muscles between their ribs, making breathing more difficult and requiring additional breathing support.



34%

of individuals with SMA report currently using some form of breathing support. Among those on breathing support, BPAP use is the most prevalent form.

When utilization of breathing support was examined by age, teenagers between the ages of 13-17 years had the highest reported use. These individuals also had the greatest daily usage (> 16 hours per day) compared to all age groups.



UNMET RESPIRATORY NEEDS

54% of adults (n=263) with SMA reported worrying about how their respiratory function may affect their safety during anesthesia.[2]

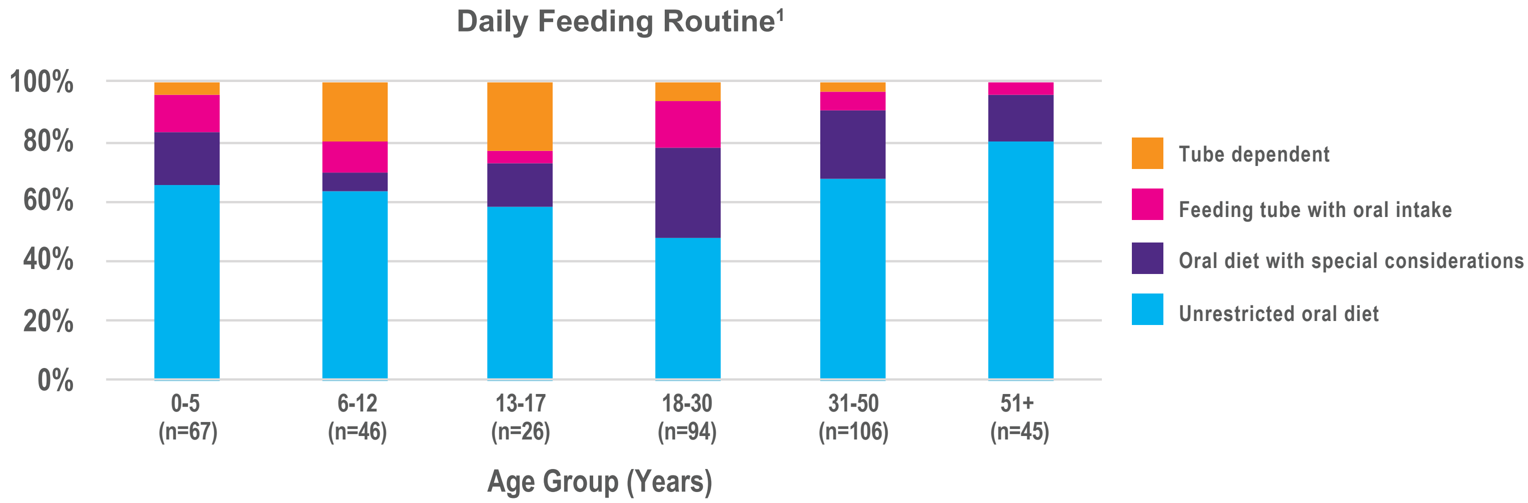
33% of adults (n=221) and 29% of caregivers of children (n=107) reported improving and/or stabilizing respiratory function as a greatest unmet need.[2]

1. Data from the 2025 CUS.
2. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

NUTRITION AND FEEDING

FEEDING TUBE UTILIZATION

Feeding tubes may be necessary for some individuals with SMA to provide partial or total nutritional needs. While the majority of individuals overall reported being on an unrestricted oral diet (63%), greater feeding tube utilization was observed among children (6-17 years) and young adults (18-30 years). Additionally, the proportion of individuals utilizing a feeding tube has decreased over time, from 35% in 2019 to 17% in 2025.



DIET

When adults with SMA (n=238) were asked if they've received enough guidance on nutrition from their provider(s), 50% reported yes, 12% said they have received some guidance but still have unmet needs, 21% said they have not received enough guidance or support, and 17% said they've never discussed nutrition with a provider.[2] Qualitative data and quotes on additional support and guidance related to nutrition and feeding that adults would find helpful can be found below:

What additional support or guidance related to nutrition or feeding would be helpful to you?[2]

- *"What diets are best for people with SMA?"*
- *"What should I eat and not eat to help my SMA?"*
- *"Easy to swallow food suggestions"*
- *"Advice on losing weight without being able to exercise"*
- *"Specific formulas for reduced metabolism"*
- *"Diets to lose weight – being overweight affects my ability to care for myself"*
- *"Meal preparation can be difficult"*
- *"Meal ideas that are easy for caregivers to make"*
- *"There is a lack of SMA-specific diet information"*
- *"Help with digestion issues"*
- *"Diet plans based off my activity level"*
- *"Does my treatment affect my metabolism or hunger? I worry I eat too much or not enough."*
- *"Protein needs"*
- *"I can't get clear information on my protein/diet needs from nutritionist"*

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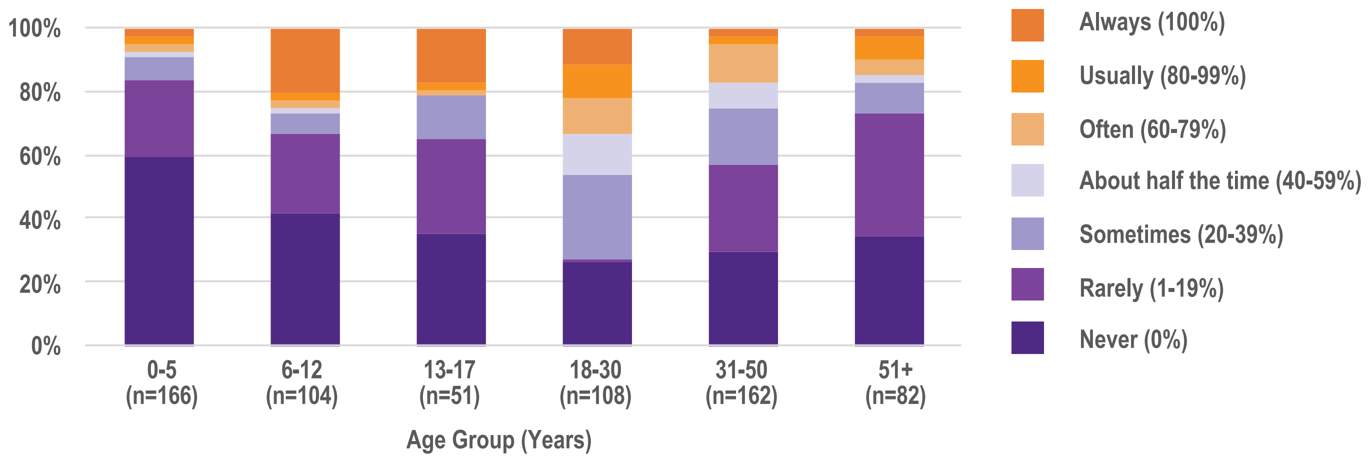
1. Data from the 2025 CUS.
2. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

BULBAR FUNCTION

Bulbar function encompasses activities that are controlled by the brainstem, which includes swallowing, speech, voice, chewing, and airway protection (coughing).

The Cure SMA Annual Community Update Survey collects information on a few bulbar function measures. The following graph shows the distribution of respondents who reported trouble swallowing in the past 12 months, by age group. The highest rates of difficulty swallowing were observed in children and adolescents age 6-17 years.

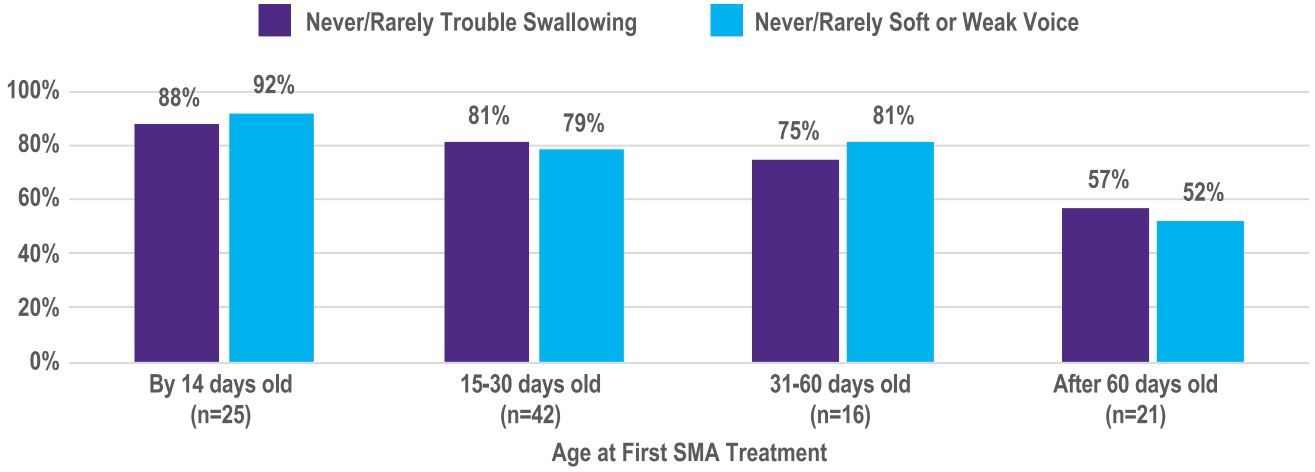
Trouble Swallowing in the Past 12 Months, by Age Group[1]



IMPACT OF TREATMENT TIMING ON BULBAR FUNCTION

In Cure SMA caregiver-reported surveys, young children with 2 copies of *SMN2* that received their first SMA treatment earlier were reported to have less trouble swallowing and soft/weak voice.

Children with 2 *SMN2* Copies Not Reported to Have Trouble Swallowing or Soft/Weak Voice in the Past 12 months[2-4]

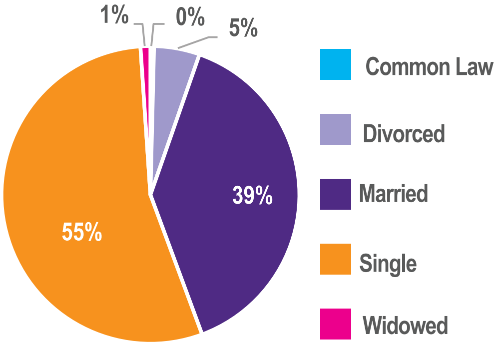


1. Data sourced from 2024-2025 CUS. Individuals participating in multiple years were de-duplicated.
2. Data sourced from 2024-2025 CUS and SMA CORE. Individuals participating in multiple sources were de-duplicated.
3. Includes children age 0-5 years.
4. Caregiver-reported data. Options for responses include “Never (0%)”, “Rarely (1-19%)”, “Sometimes (20-39%)”, “About half the time (40-59%)”, “Often (60-79%)”, “Usually (80-99%)” and “Always (all the time) (100%).”

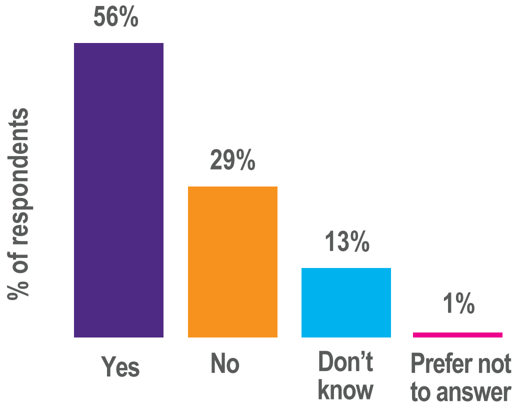
MARITAL STATUS / FAMILY PLANNING

MARITAL STATUS

Marital Status of Adults Living with SMA (n=1,028)[1]

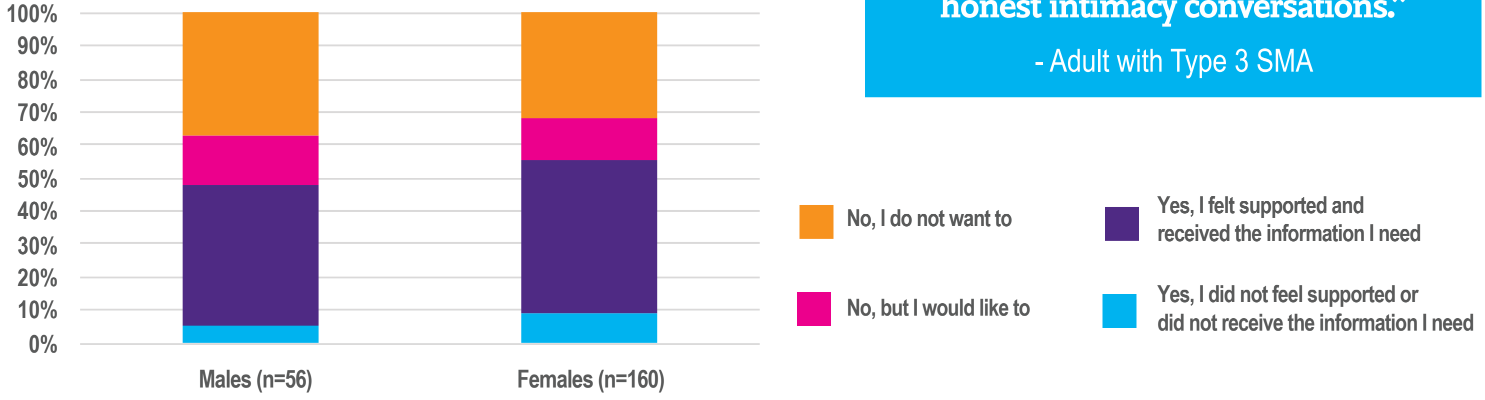


Do you fear or worry about getting married due to its impact on your public benefits (i.e. SSI, caregiving)? (n=149)[2]



REPRODUCTIVE CARE

Have you ever discussed reproductive health/family planning with a healthcare provider?[3]



> **"Adults are not having access to honest intimacy conversations."**
>
> - Adult with Type 3 SMA

HISTORY OF PREGNANCY

22% of women report a history of pregnancy.[2,4,5]

- 92.3% denied use of assisted reproductive technologies (ART) in previous pregnancies.
- 12.1% females indicated that they became pregnant after the initiation of an FDA-approved treatment.

10% of men report a history of pregnancy with a current or previous partner.[2,4,6]

- 85.7% denied use of assisted reproductive technologies (ART) in previous pregnancies.
- No males reported a pregnancy with a partner after the initiation of an FDA-approved treatment.

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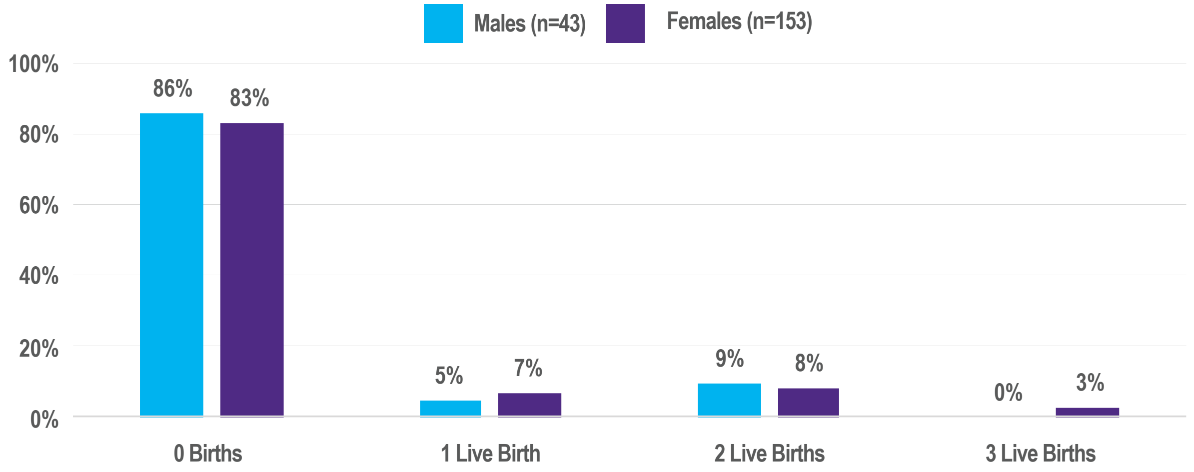
1. Data from the Cure SMA Membership Database.
2. Data from the 2025 CUS.
3. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.
4. Adults living with SMA between the ages of 18 & 74.
5. n=153.
6. n=70.

BIRTH RATE

16.3% of adults living with SMA reported giving birth or fathering[1] at least one child. A similar percentage of females living with SMA (17%) reported giving birth as males living with SMA (14%) reported fathering a child.

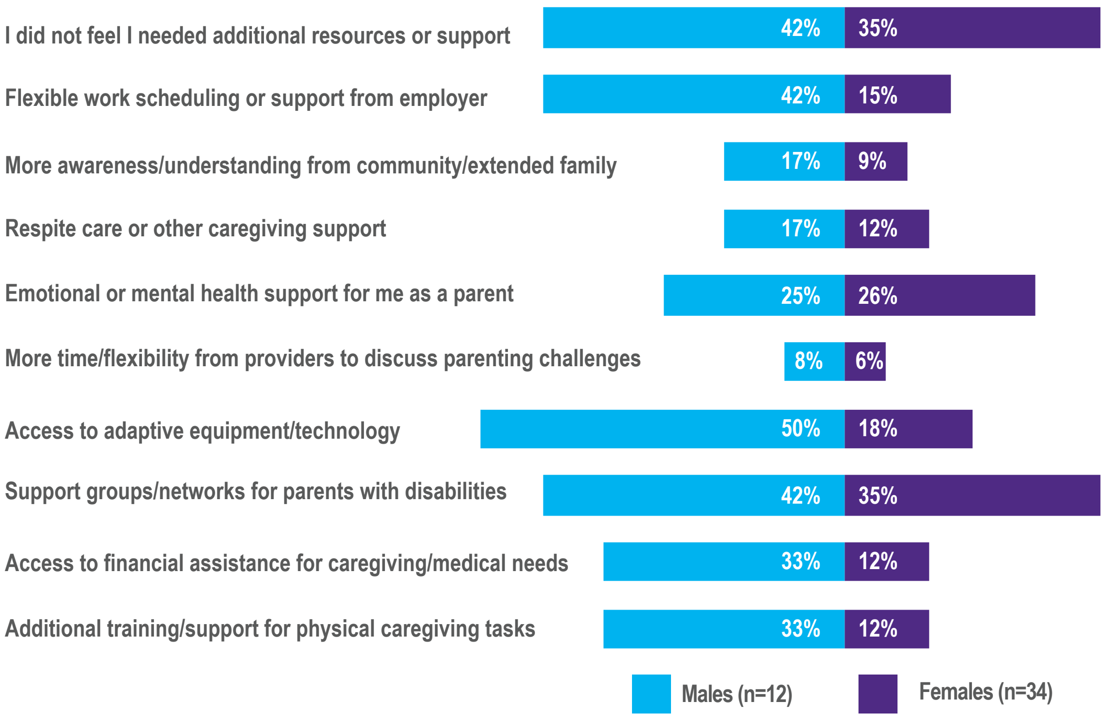
Live Births Among Adults Living with SMA[2]



PARENTING WITH SMA

Data collected from the Unmet Needs Among Adults Across the Healthcare Spectrum survey helps to bridge the gap for what resources are needed to help manage parenting while living with SMA. Female parents most frequently reported they would've benefit from support groups/networks for parents with disabilities, while male parents most frequently reported they would've benefit from access to adaptive equipment/technology.

What resources/support would have helped in managing parenting with SMA?[3,4]



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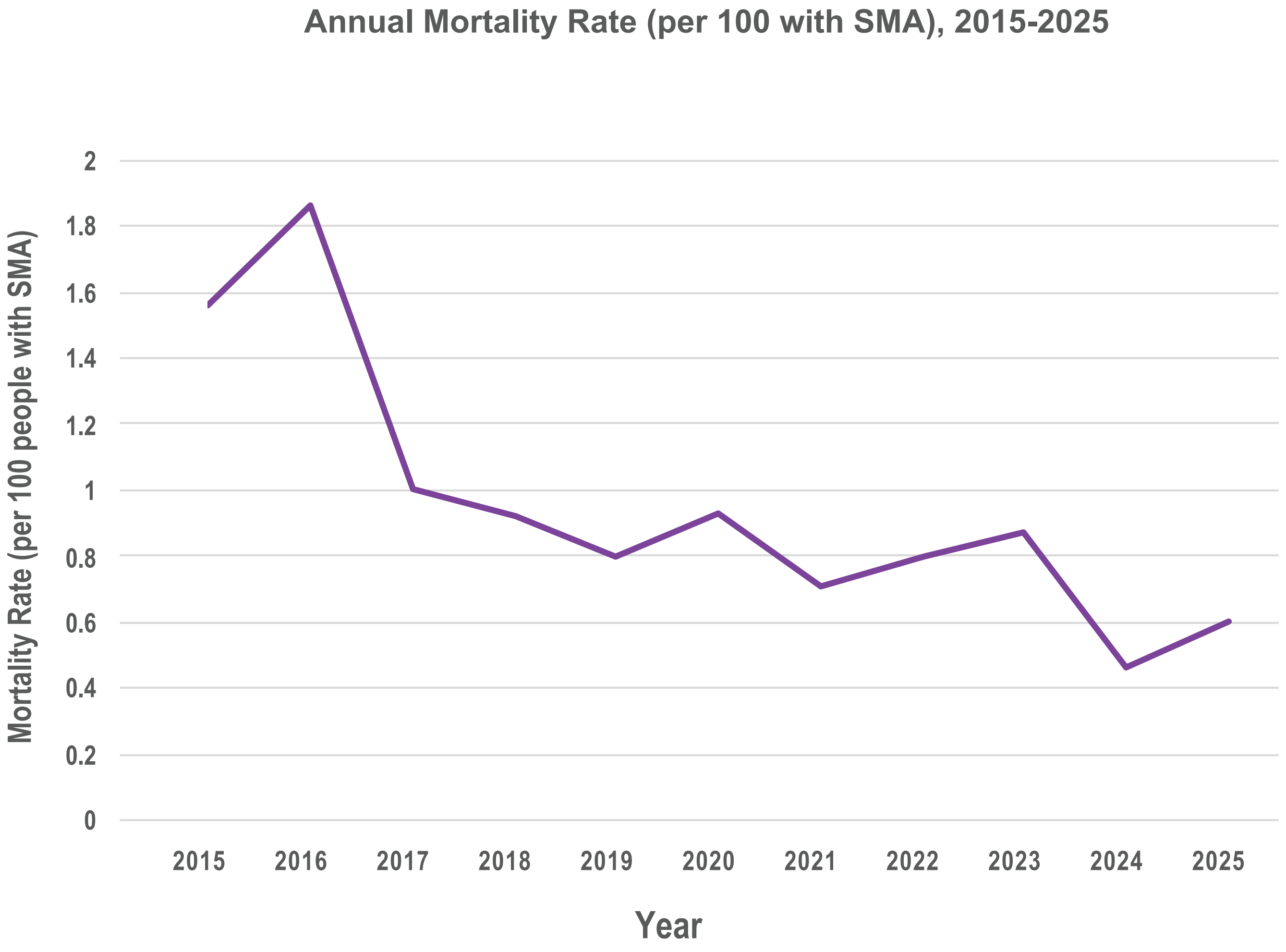
1. Fathering refers to a male living with SMA having a child with a current or previous partner.
2. Data from the 2025 CUS.
3. Options are not mutually exclusive, respondents were able to "select all that apply."
4. Data from the Unmet Needs Among Adults Across the Healthcare Spectrum Survey.

MORTALITY

The mortality rate of SMA in 2025 has dropped nearly 60% since 2015, having decreased from 1.56 per 100 individuals to 0.60 per 100 individuals with SMA.



There has been significant progress made in the SMA community but we will not stop until we have a cure.

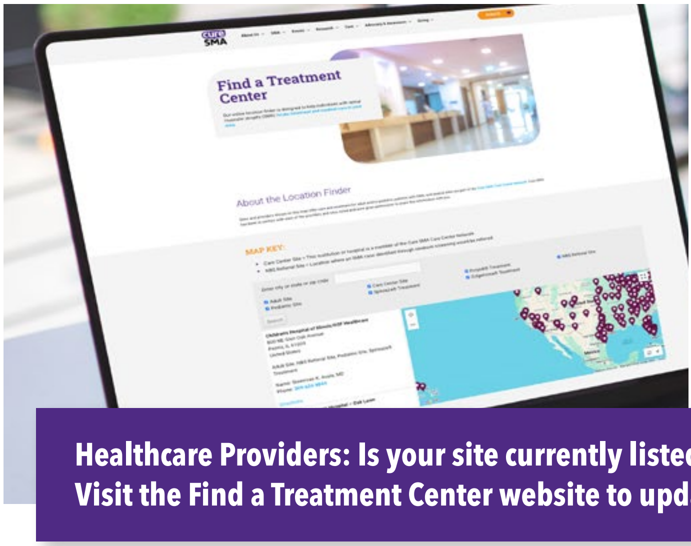
Cure SMA offers support and resources for families that are grieving the loss of a loved one. Please contact community support at CommunitySupport@curesma.org for more information.

Analysis Notes:

- Mortality data is captured from the CDR or family reported in the membership database.

CURE SMA RESOURCES

THE CURE SMA FIND A TREATMENT CENTER WEBSITE



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

Community Treatment Access

- 255 Treatment and Care Sites
  - 178 Pediatric Care Sites
  - 146 Adult Care Sites
- 2,449 Health Care Professionals

Healthcare Providers: Is your site currently listed and is the listing up to date?
Visit the Find a Treatment Center website to update or opt-in today!

INSURANCE RESOURCES

For individuals with SMA:

- Health Insurance Roadmap: Offers clear information about different types of health insurance and helpful tips for understanding what your plan covers and how it works.
- State Resource Guide Request Form: Resource guides to support those with SMA and their families. These resources cover a range of topics to enrich daily living at home and in the community.

For healthcare practitioners:

- Letter of Medical Necessity Checklist : Offers guidance on key areas to include within a letter of medical necessity.
- Insurance Appeal Process Checklist: Offers guidance on key areas to include within an appeal letter, and considerations for physician peer to peer discussions.

SMA CLINICAL TRIAL RESOURCES

By participating in a clinical trial, you can help advance research, contribute to the development of new treatment options, and make a lasting impact for others living with SMA.

Cure SMA offers tools and guidance to empower adults with SMA and caregivers at every stage of the decision-making process.

SMA REGISTRY AND CLINICAL TRIAL FINDER: Explore SMA clinical trials with our easy-to-use search widget. Filter by SMA type, location, trial phase, treatment status, and more to find trials that fit each individuals needs and preferences.

SCREENING VISIT CHECKLIST: Prepare for the screening visit with a thorough checklist that organizes medical history and suggests questions to ask the research team.

DECODING INFORMED CONSENT: Understanding informed consent is crucial! Our handout will guide adults living with SMA and caregivers through the key sections of the informed consent form, ensuring they're confident in their decision to participate in a clinical trial.

TRAVEL PLANNERS (AIR AND GROUND): Manage travel details with ease! Our itineraries will help travel planning, and are tailored to the unique needs of our SMA community.

There are multiple clinical trials that continue to evaluate new therapies. To learn more, please visit:
www.curesma.org/cure-sma-clinical-trials/

CURE SMA RESOURCES

APNCR

The Adult & Pediatric Neuromuscular Clinical Research (APNCR) Network develops and refines outcome measures for spinal muscular atrophy (SMA), conducts clinical trials for new treatment approaches, and conducts research to evaluate the impact of approved treatments to help support broad access. The network was originally established in 2004 as a pediatric SMA network (original name was PNCR) and in 2018 Cure SMA became the sole sponsor of the network. Cure SMA provided $1.2 million in funding to the PNCR in 2020, and in 2025, the PNCR network officially changed its name to APNCR to reflect the new inclusion of adult care centers.

The current sites that comprise the APNCR network are Boston Children's Hospital (Boston, MA), Children's Hospital of Philadelphia (Philadelphia, PA), Columbia University Irving Medical Center (New York, New York), Stanford University (Palo Alto, CA), and Lurie's Children's Hospital of Chicago (Chicago, IL). Data processing and warehousing are carried out by Rochester University (Rochester, NY)

KEY PUBLICATIONS FROM APNCR

Description of the Clinical Features and Natural History of SMA Type 1

Finkel RS, McDermott MP, Kaufmann P, Darras BT, Chung WK, Sproule DM, et al. Observational study of spinal muscular atrophy type I and implications for clinical trials. Neurology. 2014 Aug 26;83(9):810-7.

Observational Study of SMA Types 2 & 3

Kaufmann P, McDermott MP, Darras BT, et al. Observational Study of Spinal Muscular Atrophy Type 2 and 3: Functional Outcomes Over 1 Year. Arch Neurol. 2011;68(6):779–786.

To find out more about the APNCR visit:
www.curesma.org/apncr

GET CONNECTED

Cure SMA has many resources to keep you and your families connected to the SMA community!

Local Chapters

Volunteer-led chapters across the U.S. providing support and opportunities for those impacted by SMA to connect at the local level.

Social Media

Find us on our socials and stay connected on the latest SMA news and updates.

STATE OF SMA FEEDBACK

We want to hear from you! If you have any suggestions on how we can improve our Annual State of SMA or additional outcomes you would like to see in next year's report, please visit https://www.curesma.org/report-feedback to submit your feedback. All responses will be collected anonymously.