

STATE OF SMA



**cure
SMA**

2023 REPORT

Publication Date: May 6, 2024

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Cure SMA is thankful to all individuals with spinal muscular atrophy (SMA) and their families who have generously shared their data. Their willingness to share 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 Real World Evidence Collaboration (RWEC) and the Cure SMA Industry Collaboration (SMA-IC) for research initiatives.

Cure SMA is also grateful to the SMA Care Center Network (CCN) for their commitment to improving care for people with SMA and contributing consented patient data.

The Cure SMA Real World Evidence Collaboration

The Cure SMA Real World Evidence Collaboration (RWEC) was established in 2021 to leverage the experience, expertise and resources of pharmaceutical and biotechnology companies and nonprofit organizations involved in the development of SMA therapeutics to guide the future direction of real world evidence collection and use in SMA. Funding for the development of the State of SMA was provided by the Cure SMA RWEC. Members of the RWEC include Biogen, Novartis, Genentech/Roche, and SMA Europe.

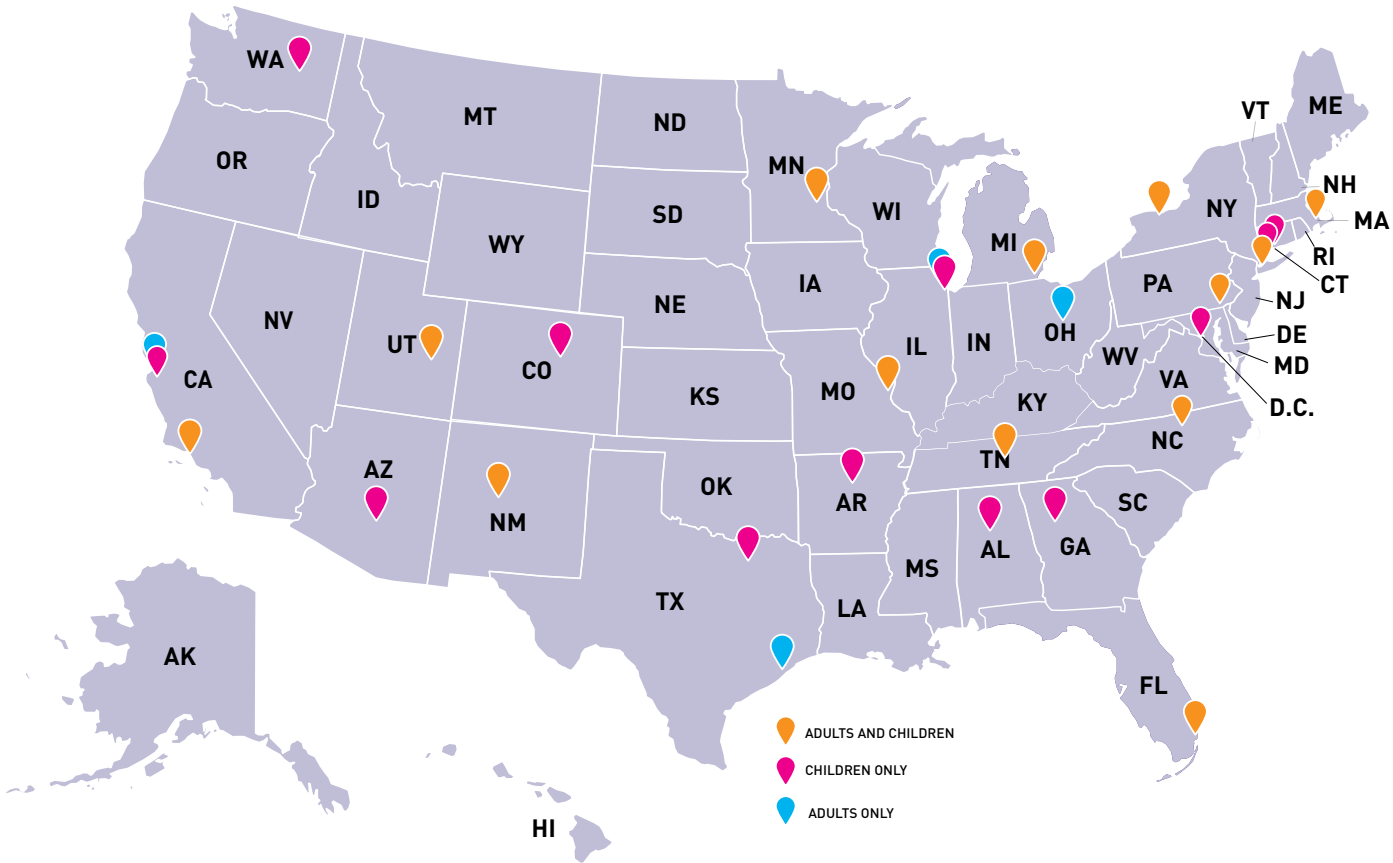
The Cure SMA Industry Collaboration

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. Current members include Cure SMA, Scholar Rock, Biogen, Novartis, Biohaven Pharmaceuticals, Genentech/Roche, Alcyone Therapeutics, NMD Pharma, and SMA Europe. Funding for the research included within the State of SMA Report was provided by the 2023 SMA-IC; members included Cure SMA, Biogen, Genentech/Roche, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, and SMA Europe.

ACKNOWLEDGMENTS

Cure SMA Care Center Network

Since 2018, Cure SMA has partnered with hospitals across the U.S. with the goal to improve healthcare for people with SMA. Every Care Center Network site submits consented patient information and data to the Cure SMA Clinical Data Registry. This data is then analyzed to drive healthcare improvements. The SMA Care Center Network includes the following sites:



ADULT & PEDIATRIC CENTERS

Boston Children's Hospital, Boston, MA
 Columbia University, New York, NY
 Connecticut Children's Medical Center, Hartford, CT
 Duke University Medical Center, Durham, NC
 Gillette Children's Specialty Healthcare, St. Paul, MN
 The Children's Hospital of Philadelphia, Philadelphia, PA
 The University of Michigan, Ann Arbor, MI
 University of California, Los Angeles (UCLA), Los Angeles, CA
 University of Miami, Miami, FL
 University of New Mexico, Albuquerque, NM
 University of Rochester Medical Center, Rochester, NY
 University of Utah, Program for Inherited Neuro Disorders, SLC, UT
 Washington University/ St. Louis Children's Hospital, St. Louis, MO

PEDIATRIC CENTERS

Advocate Children's Hospital, Park Ridge, IL
 Arkansas Children's Hospital, Little Rock, AR
 Children's Healthcare of Atlanta, Atlanta, GA
 Children's Hospital Colorado, Aurora, CO
 Children's National Medical Center, Washington, DC
 Children's of Alabama, Birmingham, AL
 Phoenix Children's Hospital, Phoenix, AZ
 Seattle Children's Hospital, Seattle, WA
 Stanford Children's Health, Palo Alto, CA
 University of Texas Southwestern/Children's Health, Dallas, TX
 Vanderbilt University Medical Center, Nashville, TN
 Yale Pediatric Neuromuscular Clinic, New Haven, CT

ADULT CENTERS

Baylor College of Medicine, Houston, TX
 Northwestern University, Chicago, IL
 Stanford Health, Palo Alto, CA
 The Ohio State University, Wexner Medical Center, Columbus, OH

Additional Acknowledgments

Cure SMA acknowledges the Care Center Network Registry Committee for their review and editing of this work. Funding for the Cure SMA Care Center Network has been provided in part by the Erin Trainor Memorial Fund and the Tyler William Orr Memorial Fund.

DEAR CURE SMA COMMUNITY,

In 2023, the Cure SMA community has continued to make tremendous progress and achieve new milestones. We are so proud to witness the rapid uptake of SMA newborn screening across the U.S. so that we now have 100% of states screening for SMA! Additionally, the SMA treatment pipeline continues to expand to new treatments utilizing new mechanisms of action and we are witnessing the benefits of early treatment on motor function gains in the real world.

Cure SMA proudly hosts three databases: a patient-reported outcomes database with data from over 10,000 affected individuals worldwide that also incorporates longitudinal data from our annual community update survey; an electronic medical record (EMR) sourced registry that compiles clinical data from 29 U.S.-based SMA Care Center Network sites; and a newborn screening registry with data from parents of babies with SMA identified through statewide SMA newborn screening.

We are excited to share with all of you the third annual State of SMA report that highlights all of the exciting trends that people with SMA and their family members have generously contributed over the years. It reflects the current landscape of the SMA community, and we hope it will foster future research, programs, and therapies.

As always, we cannot thank enough those who contribute to the Cure SMA databases. This report would not be possible without you. Participating in our surveys and registries enables us to capture your voice and understand your unique journey. Every participant counts and lends insight into further understanding the changing landscape of SMA.

This work celebrates you. Thank you all for your commitment to Cure SMA.

Sincerely,



Lisa Belter, MPH
Vice President,
Data Analytics



Sarah Whitmire, MS
Director,
Data Analytics



Erin Welsh, MPH
Specialist,
Data Analytics



Mary Schroth, MD,
FAAP, FCCP
Chief Medical Officer

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, 2023
- Residents of the United States

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)

Please note that 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.

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

CLINICAL CHARACTERISTICS:

The description of SMA-specific attributes.

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.

ELECTRONIC CASE REPORT FORM (eCRF):

A digital questionnaire used to collect data.

ELECTRONIC MEDICAL RECORD (EMR):

Digital version of a patient's healthcare chart.²

INSTITUTIONAL REVIEW BOARD (IRB):

A group that has been formally designated to review and monitor biomedical research involving human subjects. The IRB has the authority to approve, modify, or disapprove research.³

MORTALITY RATE PER YEAR:

The frequency of the occurrence of death within a subgroup of individuals within a one-year period.

PATIENT-REPORTED DATA:

Data that is gathered directly from a patient, commonly through online surveys and questionnaires.

SMA BIRTH PREVALENCE:

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

SMA CARE CENTER NETWORK:

SMA Care Centers across the U.S. who partner with Cure SMA to provide patient consented information and data to the Clinical Data Registry with the goal to improve healthcare for people with 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.

SOCIAL DETERMINANTS OF HEALTH (SDOH)

The conditions in which people are born, grow, live, work, and play that affect their health and well-being.

References:

1. Belter L, Whitmire S, Curry M, Schroth M. 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.
2. www.forbes.com/advisor/business/software/what-is-an-emr/
3. [www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/institutional-review-boards-irbs-and-protection-human-subjects-clinical-trials#:~:text=Under%20FDA%20regulations%2C%20an%20Institutional,approval\)%2C%20or%20disapprove%20research](https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/institutional-review-boards-irbs-and-protection-human-subjects-clinical-trials#:~:text=Under%20FDA%20regulations%2C%20an%20Institutional,approval)%2C%20or%20disapprove%20research)

2023 KEY FINDINGS

37%

of the population reported having Type 2 SMA

48%

of the population reported having 3 copies of the SMN2 gene

50%

of individuals in Cure SMA databases are 18 years of age or older

>1,000

adults have connected with Cure SMA since 2017

65%

of the SMA population is included in Cure SMA databases

The incidence rate was

1 in 15,000

based on information received from U.S. public health state labs

The mortality rate was

0.75 per 100

individuals with SMA

60%-70%

of individuals with SMA had received an FDA approved treatment as of Q4 of 2023

68%

of individuals in our databases that were diagnosed with SMA in 2023 were diagnosed via newborn screening

8.1

years was the average of diagnosis in 2023 among individuals diagnosed not through newborn screening

97%

of adults affected with SMA reported that they hope new therapies will help them gain muscle strength

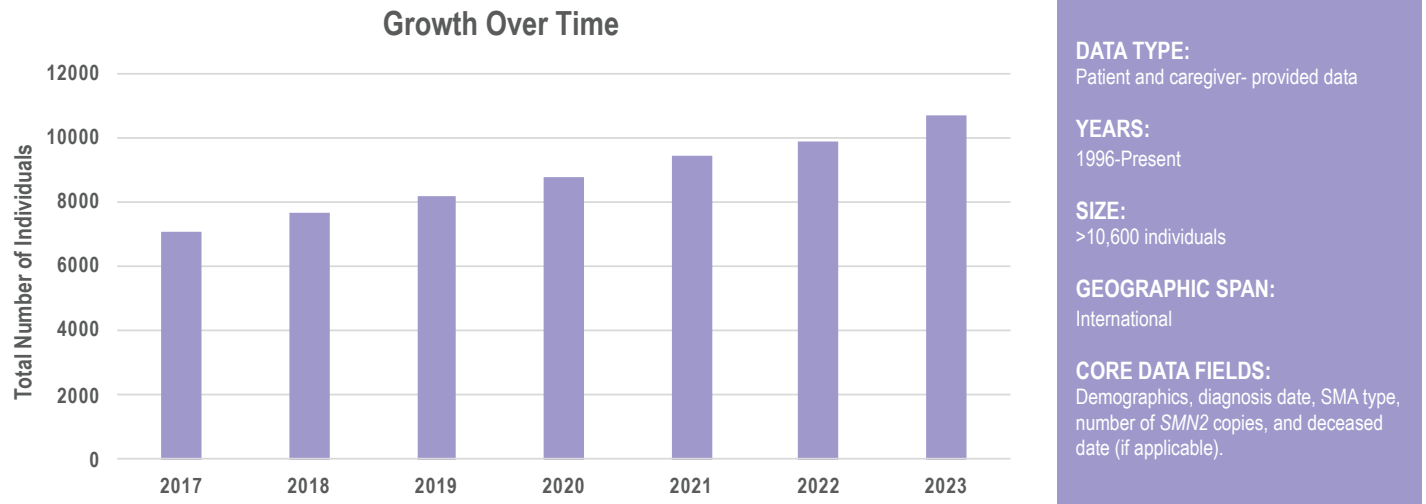
47%

of teens and adults with SMA reported having depression and/or anxiety

CURE SMA DATABASES

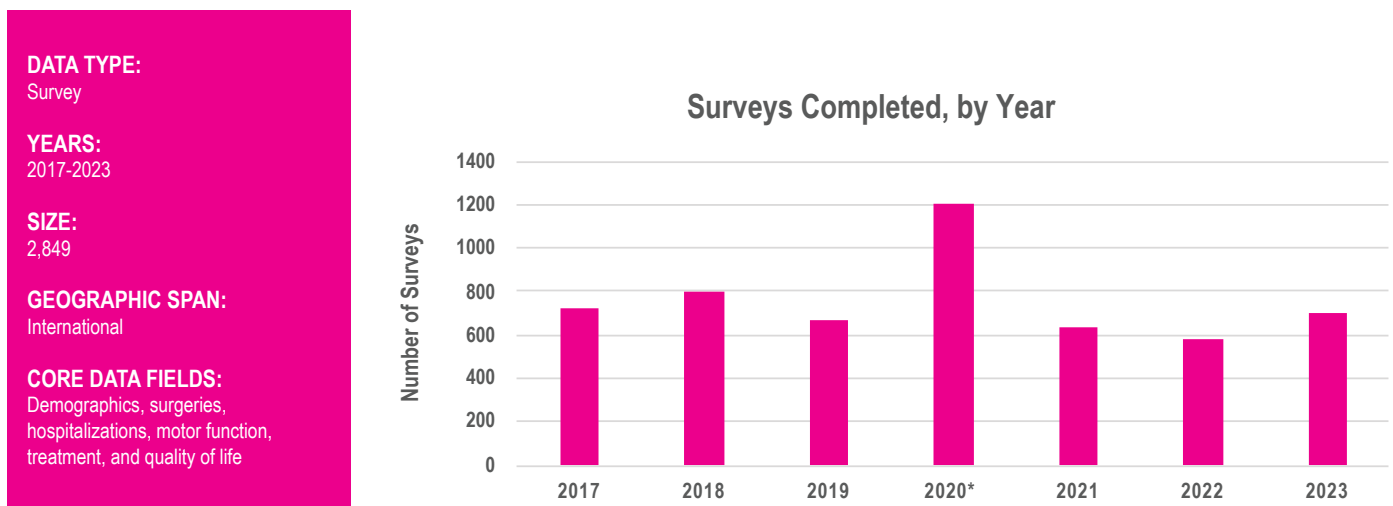
Cure SMA Membership Database

The Cure SMA membership database constitutes one of the largest patient-reported data repositories for people living with SMA worldwide. It was launched in 1996. Since then, an average of 40 newly diagnosed individuals have contacted Cure SMA each month to share information. Patient-reported data captures real world patient experiences and can represent a broad spectrum of patients. Research projects use de-identified patient data from the membership database and receive Institutional Review Board (IRB) approval prior to project start.



Community Update Survey (CUS)

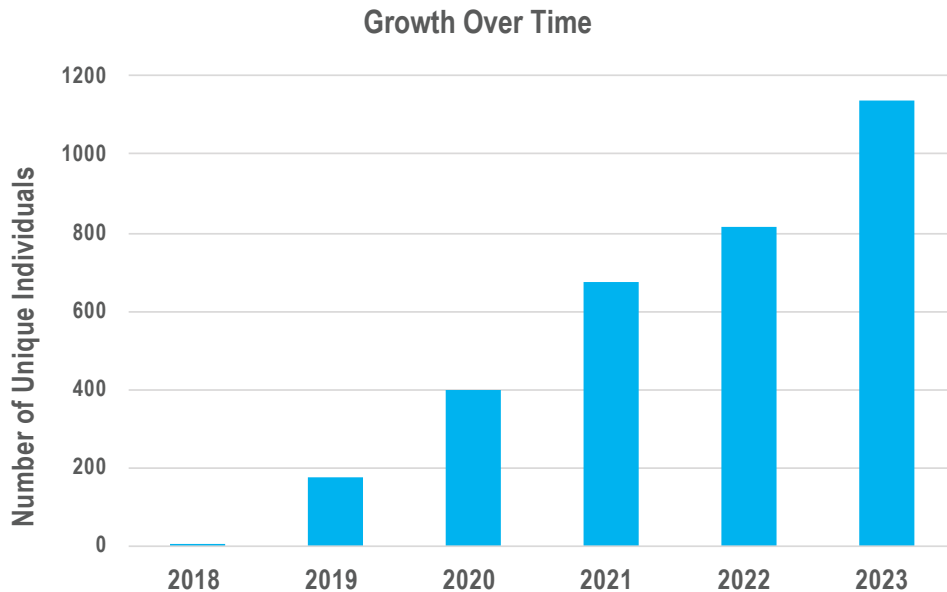
Since 2017, Cure SMA has conducted an annual online Community Update Survey to capture longitudinal data from the patient's perspective and develop additional data that can support assessment of SMA disease impact. Survey participants include both new and existing Cure SMA members.



* The Community Update Survey had the largest response rate in 2020 at the start of the COVID-19 pandemic.

Clinical Data Registry (CDR)

The CDR is an Institutional Review Board (IRB) governed database for individuals with SMA comprised of electronic medical records (EMR) sourced data from Care Center Network sites and clinician-entered electronic case report forms to gather additional information that is not easily found in the EMR. The registry was launched in October 2018. As some of the new Care Center Network sites are being integrated into the CDR, this report contains data from 21 of the 29 sites.



DATA TYPE:

Electronic medical records and electronic case report form

YEARS:

2018-Present

SIZE:

>1100 individuals

GEOGRAPHIC SPAN:

United States*

CORE DATA FIELDS:

Demographics, genetic information, motor function, diagnoses, medications, procedures, laboratory studies, vitals signs, etc.

**Data from a few international patients who receive care in the U.S are included in this registry.*

Newborn Screening Registry Database (NBSR)

The NBSR is a caregiver-reported data repository comprised of individuals with SMA identified via newborn screening. This database was launched in 2019 and allows for the collection of real world data that can be used to track outcomes in this population.

DATA TYPE:

Survey

YEARS:

2019-Present

SIZE:

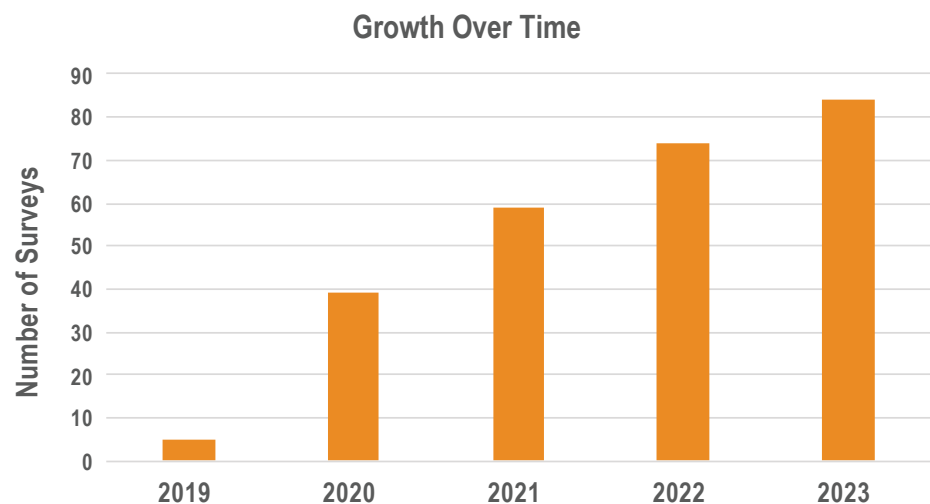
>80 individuals

GEOGRAPHIC SPAN:

United States

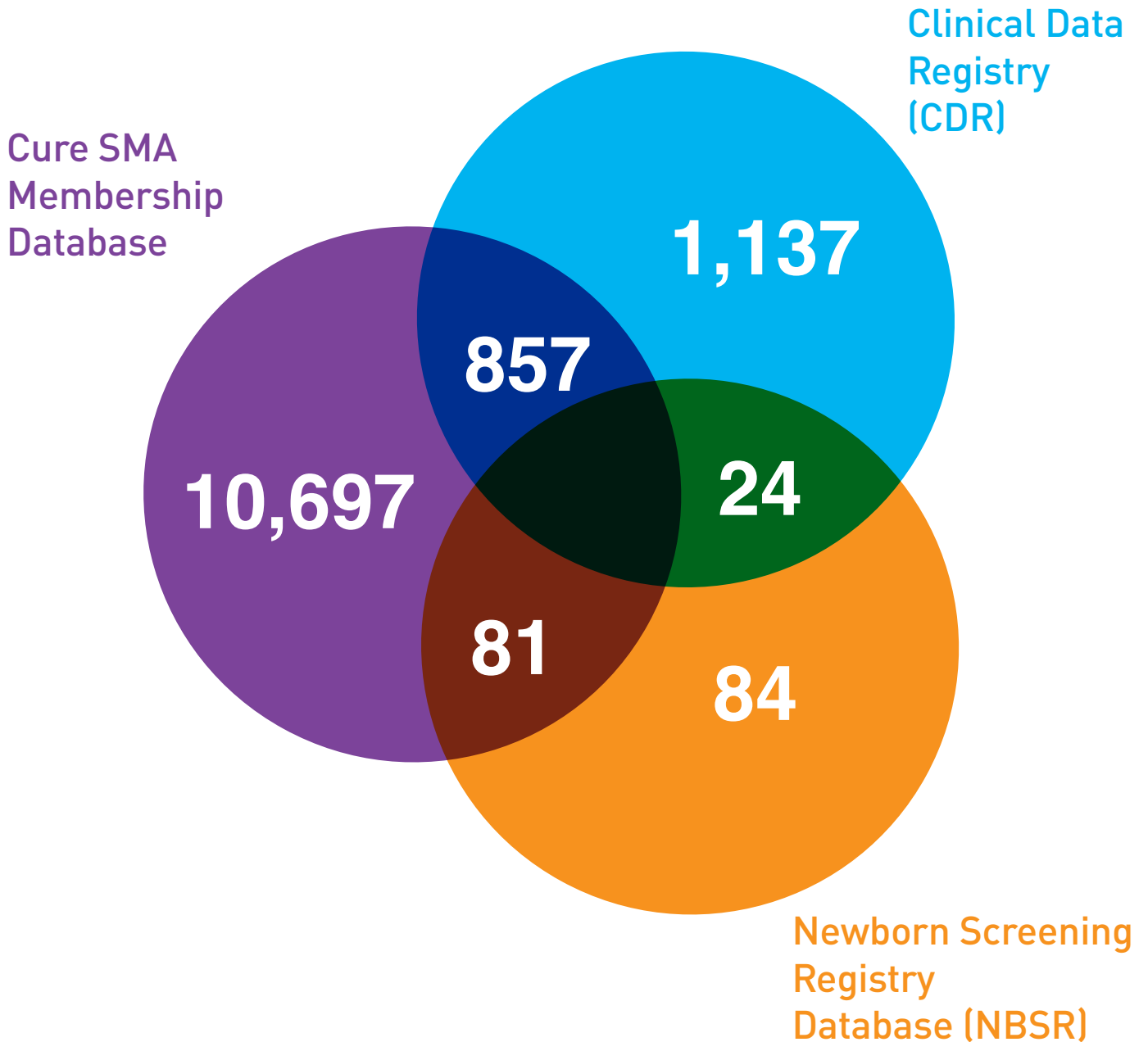
CORE DATA FIELDS:

Demographics, SMN2 copy number, diagnosis date, treatment, treatment date, motor function milestones.



CURE SMA DATABASES

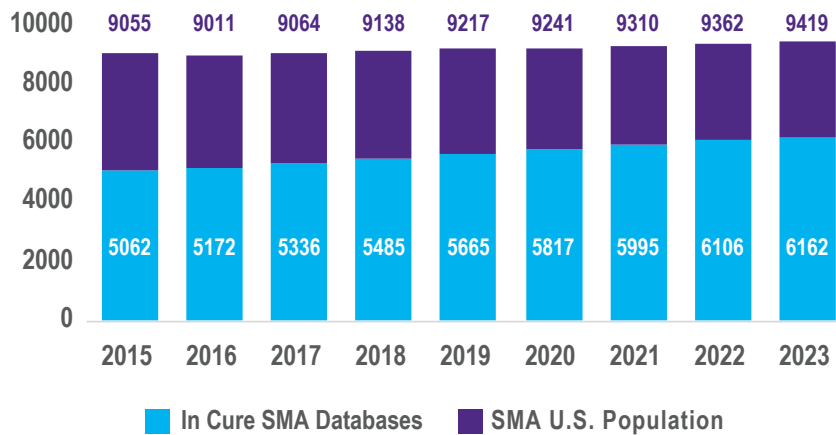
Unique Individuals Across Cure SMA Data Sources



Within the Cure SMA databases, there are currently 10,697 unique individuals within the Membership database, 1,137 unique individuals in the CDR, and 84 unique individuals within the NBSR. Databases are not mutually exclusive, and some individuals may participate in more than one database. There are currently 81 individuals in both the NBSR and Membership database, 24 individuals in both the NBSR and CDR, and 857 individuals in both the CDR and Membership database. Across all data sources, there are 906 unique individuals who are in two or more databases.

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 United States to gauge the coverage of the Cure SMA databases.

Cure SMA estimates there are currently **9,000-9,500** individual with SMA currently living in the U.S.



Cure SMA's databases cover about 65% of the current U.S. SMA population

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 15,000 based on data from U.S. public health state labs, U.S. public health state labs.*
- A decreasing mortality rate as reported by the CDC WONDER database² and the Cure SMA data, see [page 40](#).
- SMA subtype incidence of 50% for Type 1, 35% for Type 2, and 15% for Type 3/4³
- Race and ethnicity SMA carrier rates described by Sugarman et al.⁴

*DATA FROM U.S. PUBLIC HEALTH STATE LABS

Cure SMA has asked each state that has implemented SMA newborn screening for data on the number of individuals screened and the number of individuals that screened positive for SMA. This data will provide a better understanding of the number of individuals diagnosed with SMA in the United States each year (incidence rate).

As of December 2023, data from 42 states showed:

- More than 8 million infants have been screened for SMA to date
- An estimated 539 infants screened positive and SMA diagnosis confirmed
- The current incidence of SMA is approximately 1 in 15,000 births⁵

1. <https://www.cdc.gov/nchs/nvss/births.htm>

2. <https://wonder.cdc.gov/>

3. Verhaart IEC, Robertson A, Wilson IJ, Aartsma-Rus A, Cameron S, Jones CC, Cook SF, Lochmüller H. Prevalence, incidence and carrier frequency of 5q-linked spinal muscular atrophy - a literature review. *Orphanet J Rare Dis.* 2017 Jul 4;12(1):124.

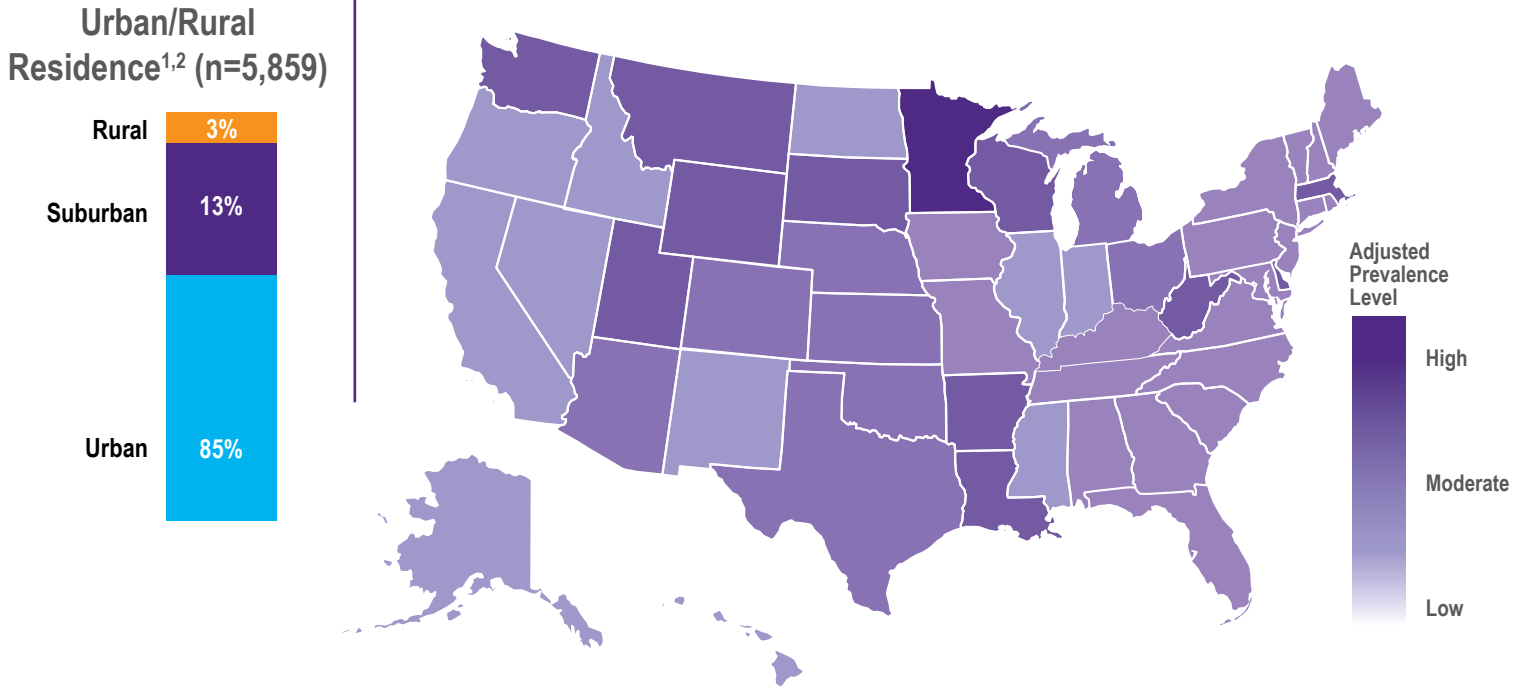
4. Sugarman EA, Nagan N, Zhu H, Akmaev VR, Zhou Z, Rohlfis EM, et al. Pan-ethnic carrier screening and prenatal diagnosis for spinal muscular atrophy: clinical laboratory analysis of >72,400 specimens. *Eur J Hum Genet.* 2012 Jan;20(1):27-32.

5. Internal Cure SMA data provided by U.S. State Public Health Labs

U.S. DISTRIBUTION

Cure SMA currently has 37 chapters throughout the United States, with a new chapter established in Puerto Rico, providing community and support for families and individuals affected by SMA.

U.S. Distribution of SMA Adjusted for State Population (n=5,943)



When adjusted for the state population density Minnesota, South Dakota, Utah, Wisconsin, and Michigan had the highest proportion of individuals with SMA. Additionally, majority of the population resides in an urban area (85%), similar to the general U.S. population (80.7%)³

Currently, Cure SMA has connected with 53 individuals living with SMA in Puerto Rico.

PUERTO RICO
‘queremos la cura

The Puerto Rico chapter was officially established in January of 2024, and hosted a Summit of Strength and Walk-n-Roll with over 100 people in attendance! The half-mile Walk-n-Roll was led by a police brigade and traditional Puerto Rican drummers and dancers.

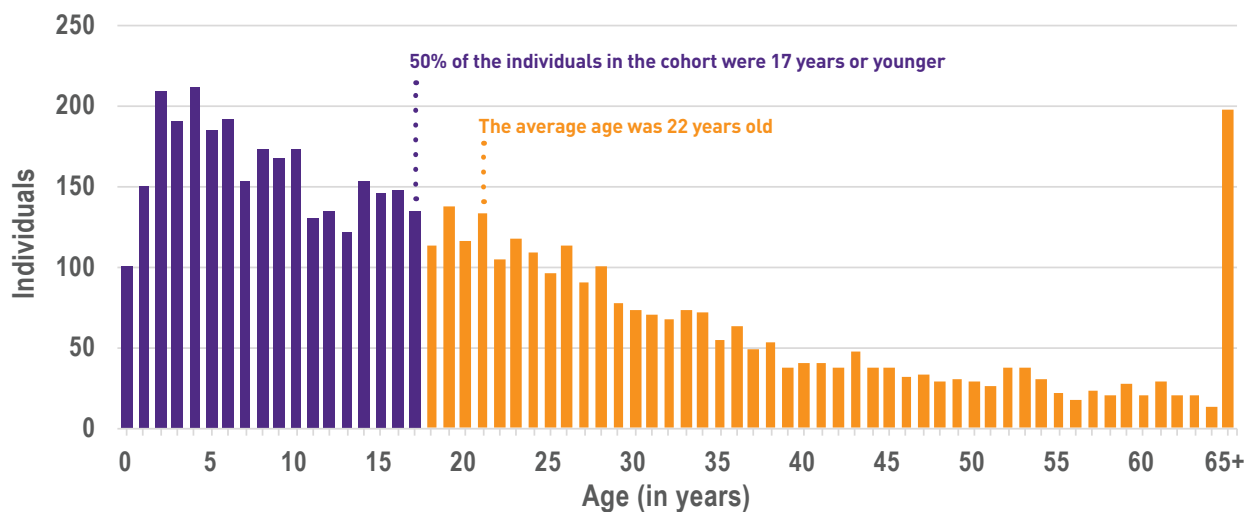
Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Urban/rural residence was determined using zip-code information (if available) and classifications determined by the U.S. Department of Agriculture (USDA)
2. Rural-urban commuting area codes. USDA ERS - Rural-Urban Commuting Area Codes. (2023, September 23). <https://www.ers.usda.gov/data-products/rural-urban-commuting-area-codes/>
3. <https://www.census.gov/programs-surveys/geography/guidance/geo-areas/urban-rural/ua-facts.html>

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, 2023

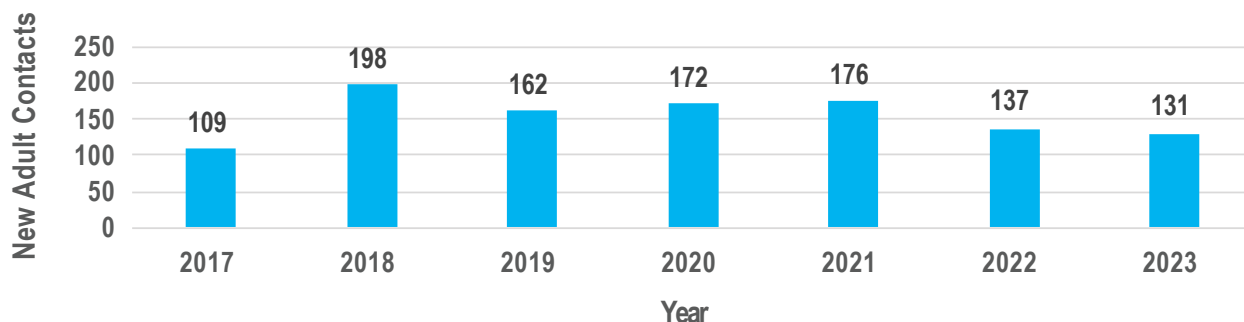
Age Distribution of Individuals in Cure SMA Data in 2023¹ (n=5,739)



Average Age: 22 years | Standard Deviation: 18 years | Median Age: 17 years

Newly Connected Adults

Since 2017, Cure SMA has connected with more than 1,000 adults affected with SMA



Teen & Adult Support Package



Teen & Adult Independence Assistance Package



Medical Alert Bracelet



LifeVac Support Program



SMA Conferences and Summit of Strengths

Cure SMA offers a variety of support programs to meet the evolving needs of teens and adults in our SMA community. Please visit www.curesma.org/teens-and-adults/#resources-for-adults to learn more.

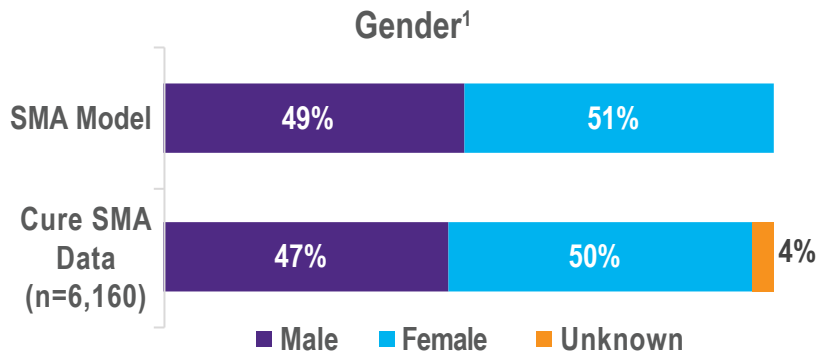
Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Includes individuals that were alive as of December 31, 2023

Analysis notes:

- Age was calculated as of December 31st of the calendar year being reported.

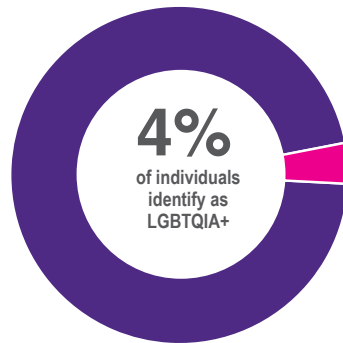
GENDER / GENDER IDENTITY



1.2%
of individuals identify with a gender that is different than their birth sex

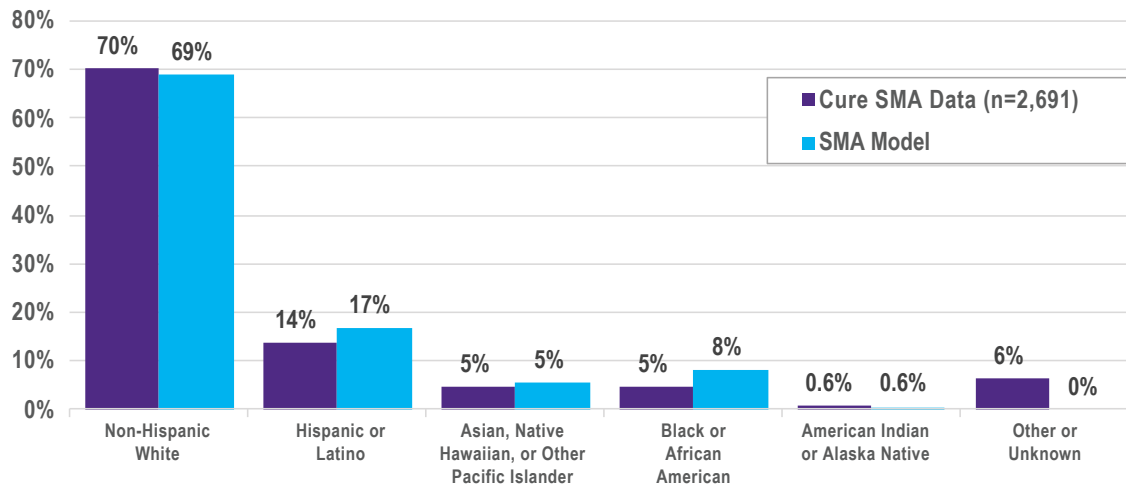
PATIENT/CAREGIVER REPORTED

LGBTQIA+ POPULATION²



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

RACE/ETHNICITY CATEGORIES



Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

- The CDR collects gender used for administrative purposes (ie. Insurance) that may or may not align with a person's gender in all context.
- 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, 2023
- If gender or race/ethnicity was provided for the same individual in multiple databases, the self-reported data was prioritized.

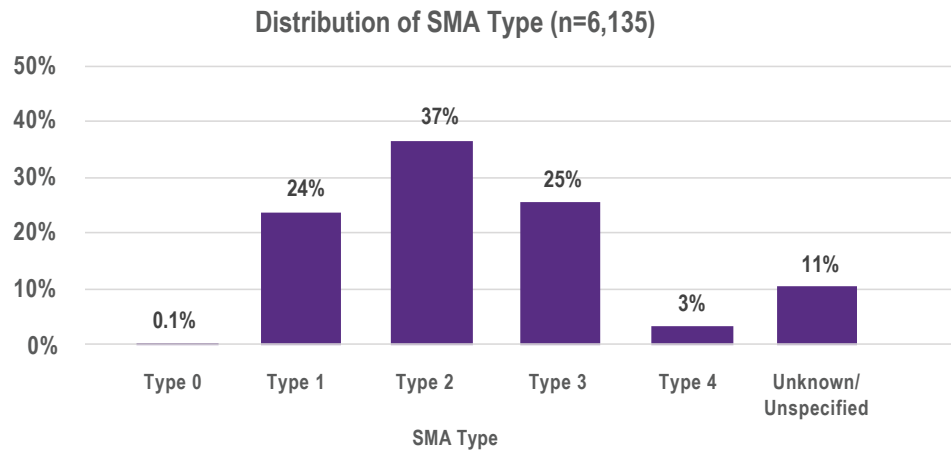
PREVALENCE OF SMA TYPE AND SMN2 COPY NUMBER:

Prevalence: The number of people currently living with SMA in the United States

SMA TYPE:

Historically, SMA was 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.

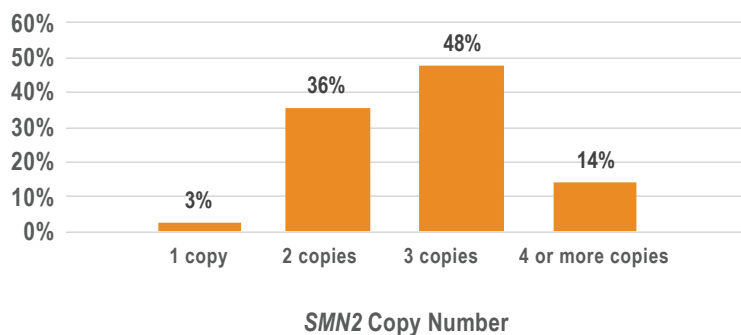
The largest proportion of individuals in Cure SMA data sources have Type 2 SMA



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 Number¹ (n=2,631)



The largest proportion of individuals in Cure SMA data sources have 3 copies of SMN2.

Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

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

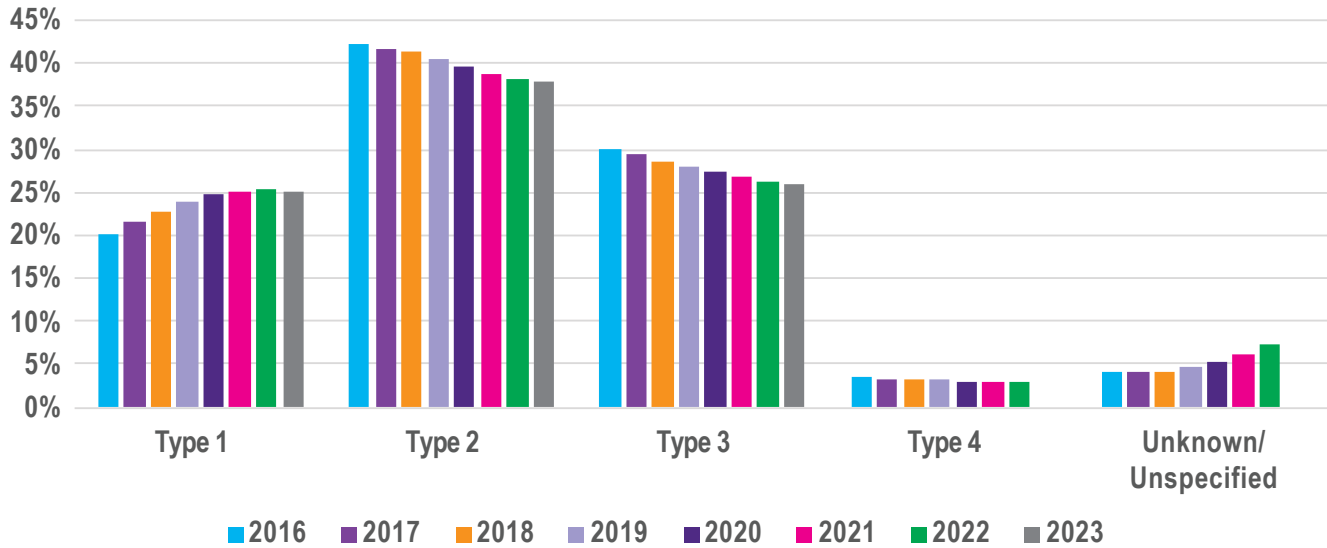
Analysis Notes:

- All graphics include individuals that were alive as of December 31, 2023

TRENDS IN OUR DATA:

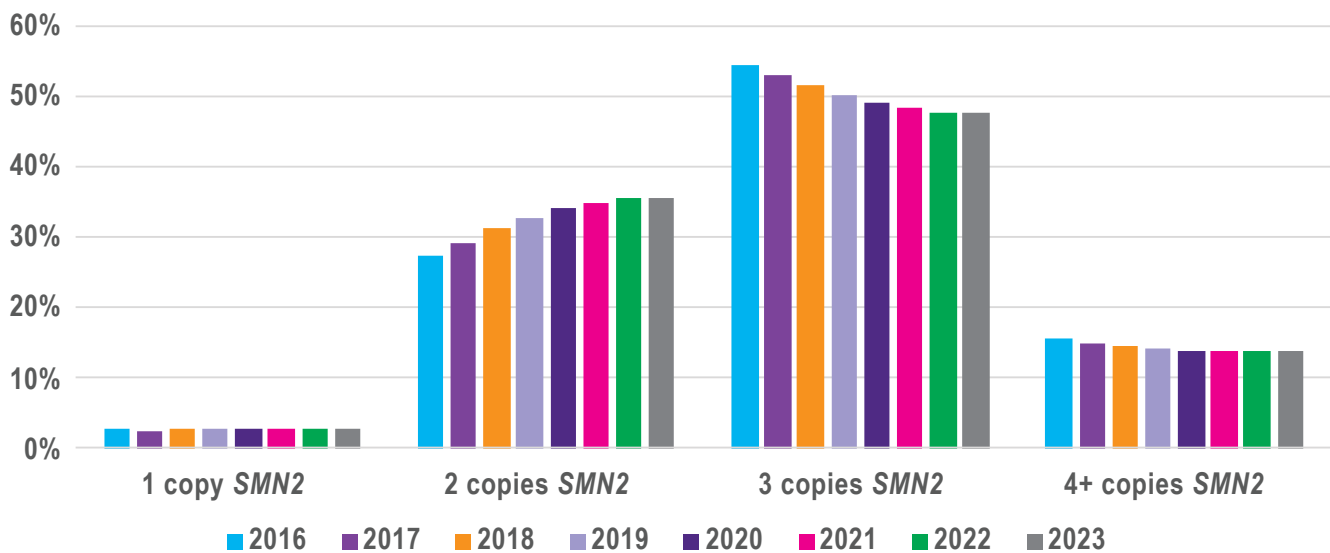
SMA TYPE & SMN2 COPY NUMBER

PREVALENCE OF SMA TYPE OVER TIME¹



We are observing rising proportions of individuals with SMA Type 1 and Unknown/Unspecified 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 the availability of treatments and diagnosis before symptom onset may be possible factors.

PREVALENCE OF SMN2 COPY NUMBER OVER TIME²

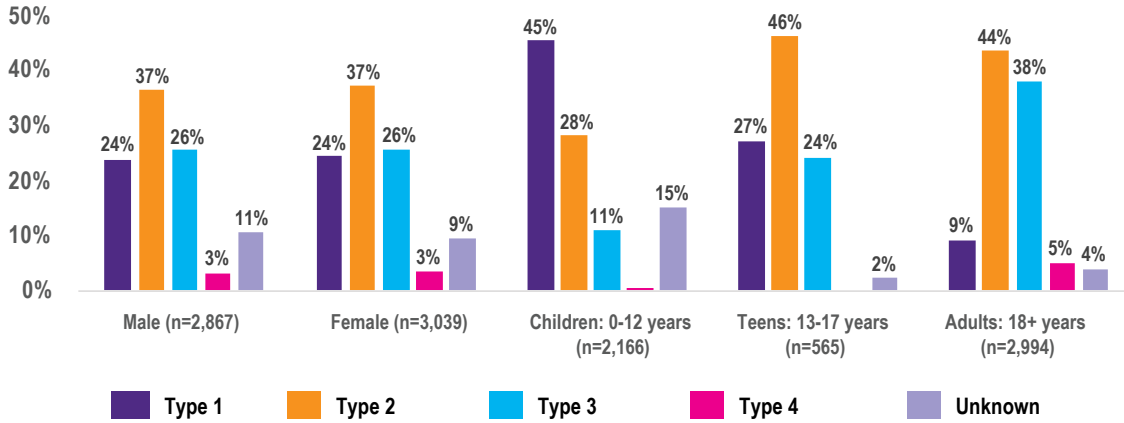


Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. SMA Type 0 was not included in the graphic due to small sample size.
2. Individuals with unknown SMN2 copy number were not included in this graphic

GENDER AND AGE GROUP DIFFERENCES IN SMA

Distribution of SMA Type¹



Overall, we are observing an even distribution of SMA type among males and females.

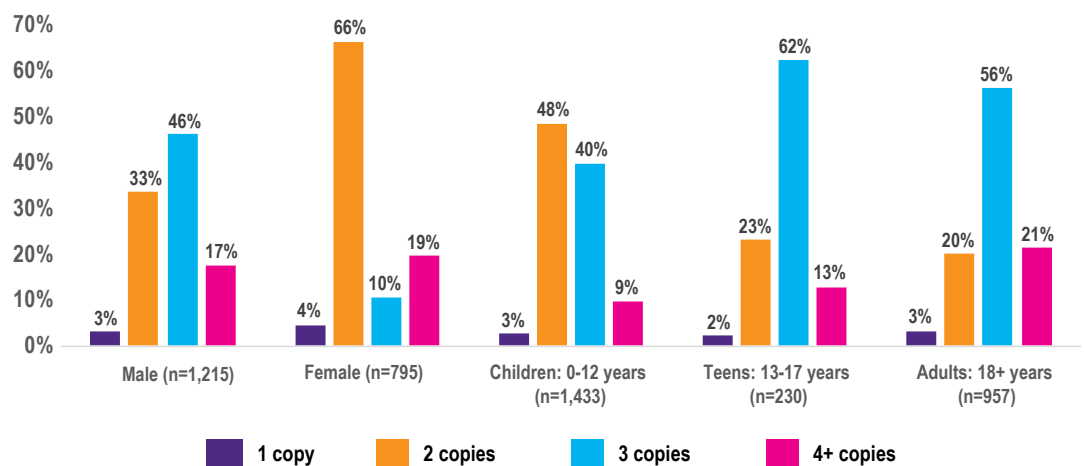
When stratified by age group, we are seeing that Type 1 is most prevalent among children and that prevalence decreases among teens and adults. Additionally, we are seeing that the proportions of Type 2 and Type 3 SMA are higher in teens and adults compared to children.

When broken down by gender, we are seeing a higher proportion of 2 copies of *SMN2* among females, and a higher proportion of 3 copies of *SMN2* among males.

When looking at *SMN2* distribution by age group we are seeing that the proportion of 2 copies is the highest among children and decreases among teens and adults.

Additionally, we are seeing the proportions of 3 copies and 4+ copies higher in teens and adults compared to children.

Distribution of *SMN2* Copy Number²



Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Individuals with Type 0 SMA were not included in the graphic due to small sample size
2. Unknown *SMN2* copies were excluded from this analysis

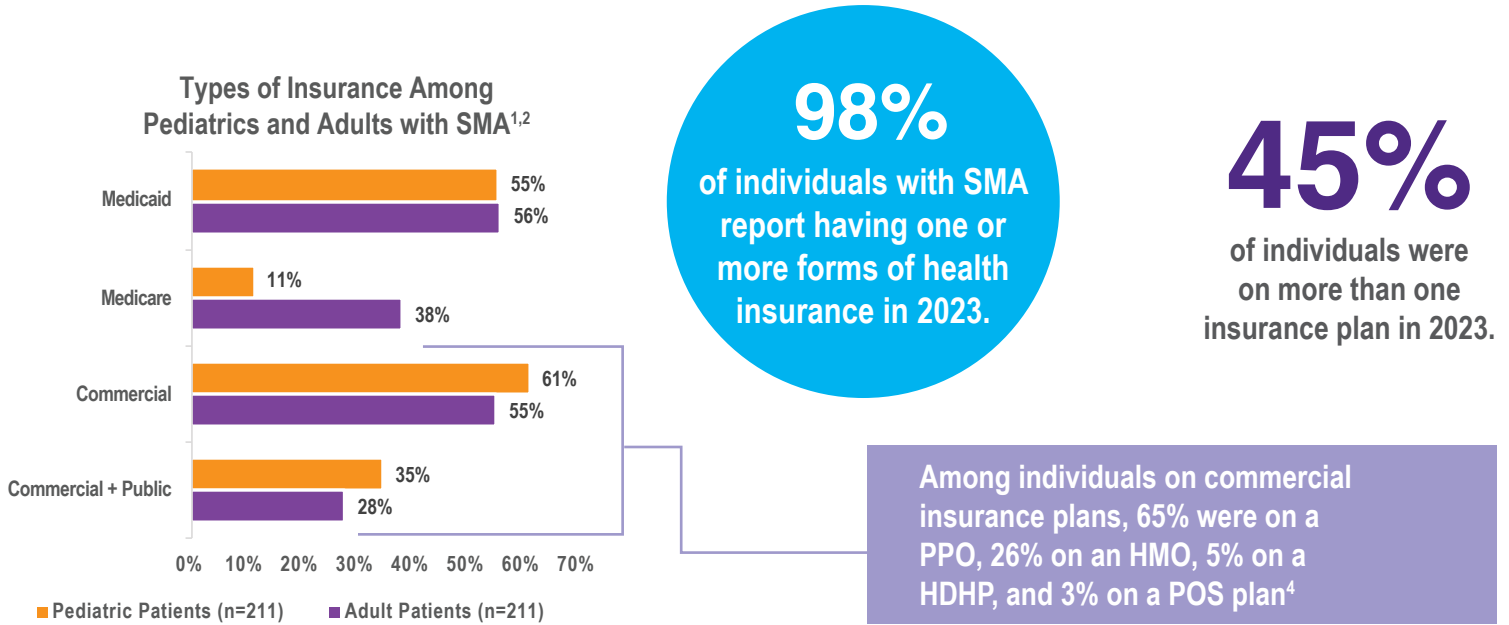
Analysis Notes:

- All graphics include individuals that were alive as of December 31, 2023

INSURANCE:

PATIENT/CAREGIVER REPORTED

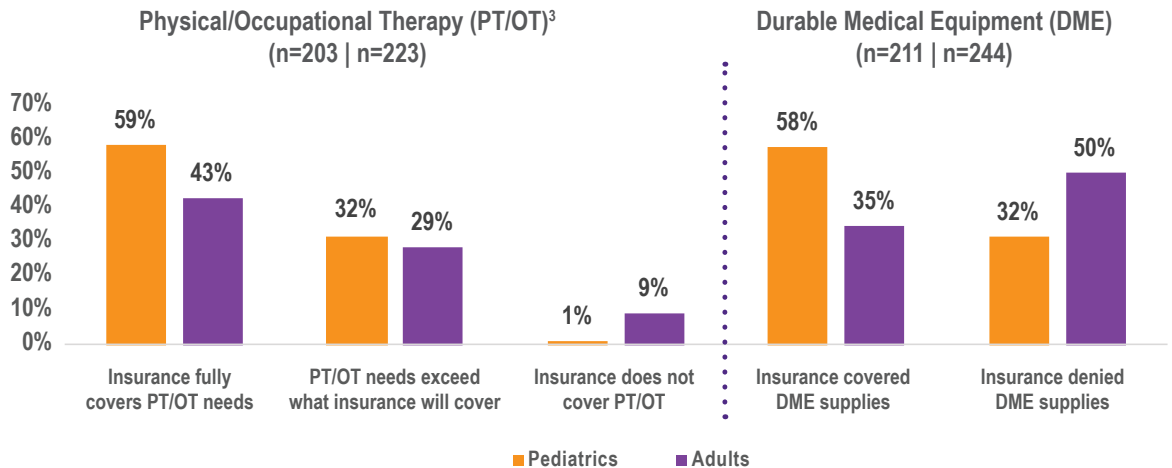
SMA care is expensive and includes many out-of-pocket costs for outpatient medical care, hospitalizations, and medications. Most children with SMA are enrolled in government-funded insurance programs.



INSURANCE COVERAGE

60% of individuals with SMA report having inadequate medical coverage for their treatment needs

32% of pediatric patients and 29% of adult patients have physical and occupational therapy needs that exceed their insurance coverage. Additionally, a greater proportion of adults (50%) reported having insurance denials for their durable medical equipment.



Cure SMA Data Sources: 2023 CUS data

- Insurance categories are not mutually exclusive
- Categories in insurance coverage of "Don't know" or "Not applicable" were excluded from the graph but were used in calculating proportion.
- If someone reported their insurance covers PT/OT regardless of if they need therapy, they were grouped into the category of "Insurance fully covered PT/OT needs"
- PPO stands for preferred provider organization; HMO stands for health maintenance organization; HDHP stands for high deductible health plan; POS stands for point of service.

Analysis Notes:

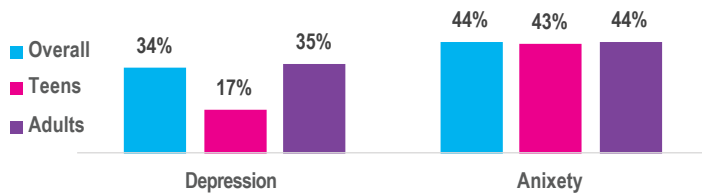
- For this analysis, the adult group was 18-64 years of age

MENTAL HEALTH: TEENS & ADULTS

PATIENT/CAREGIVER REPORTED

A qualitative study by Cure SMA found that individuals with SMA reported their mental health, including anxiety and depression, to be severely impacted by SMA¹.

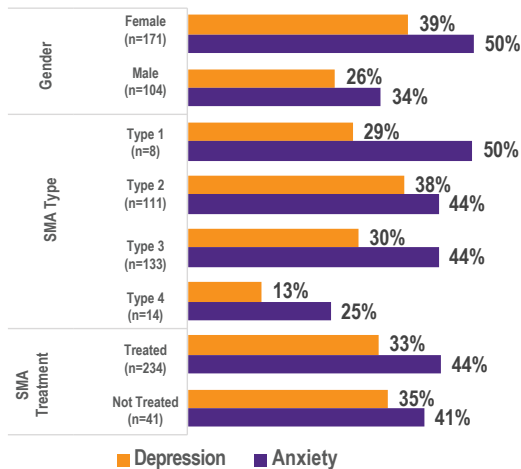
Prevalence of Depression and Anxiety Within the SMA Population (n=275)



Among teenagers who reported having a mental health illness, 100% reported having both depression and anxiety, compared to 61% of adults who reported having both.

In the last year, 65% of individuals with depression received treatment, and 2% were hospitalized due to their depression. Additionally, 63% of individuals with anxiety received treatment, and 4% were hospitalized due to their anxiety.

Prevalence of Depression and Anxiety by Characteristics of Individuals with SMA



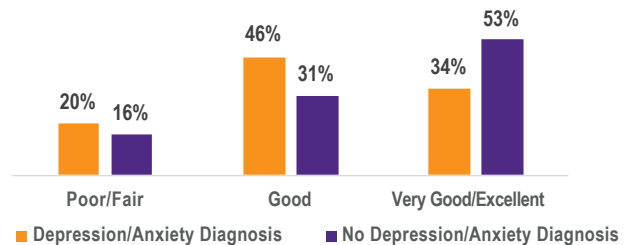
When stratified by gender, both depression and anxiety were found to be more prevalent in females compared to males, with females having a higher prevalence of anxiety than depression overall.

The prevalence of depression was the greatest among individuals with Type 2 SMA. Prevalence of anxiety was consistent among individuals with SMA Types 1, 2, and 3. Those with Type 4 SMA showed the lowest rates of both depression and anxiety.

Rates of both depression and anxiety were similar among individuals treated and not treated for SMA.

When asked to report self-perception of overall health, the majority of individuals with depression and/or anxiety reported their health as "good." Additionally, we saw that a greater proportion of individuals without depression and/or anxiety reported their health as very good or excellent compared to those with depression and/or anxiety (53% vs. 34%).

Self-Health Rating Among Individuals With and Without Depression or Anxiety (n=211)



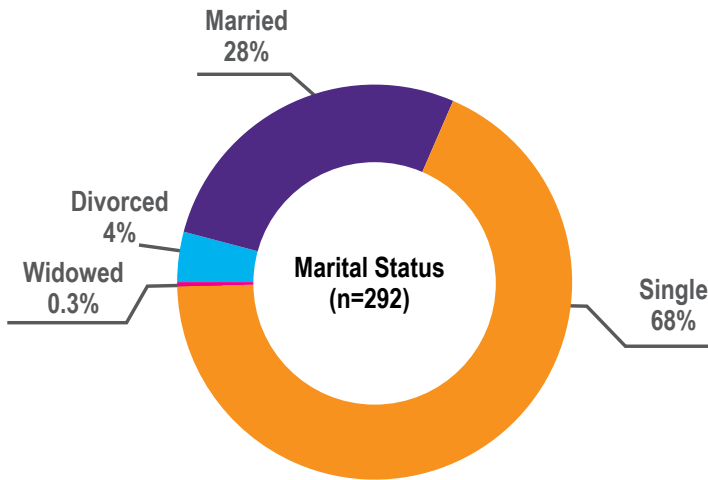
If you are experiencing anxiety or depression, please contact your healthcare provider. You are not alone.

Cure SMA Data Sources: 2023 CUS data

1. Mazzella A, Curry M, Belter L, Cruz R, Jarecki J. "I have SMA, SMA doesn't have me": a qualitative snapshot into the challenges, successes, and quality of life of adolescents and young adults with SMA. *Orphanet J Rare Dis.* 2021;16(1):96.

ADULTS WITH SMA

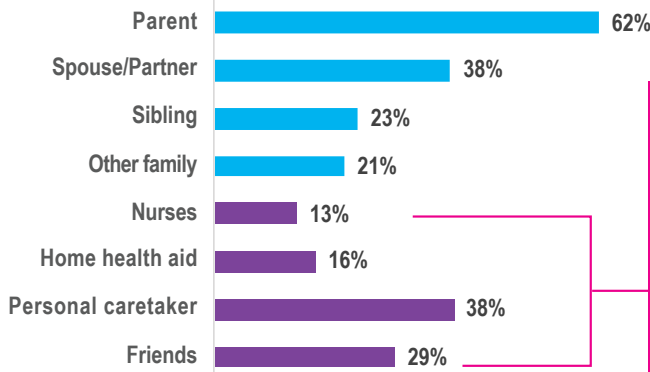
PATIENT/CAREGIVER REPORTED



18%
of women affected with SMA self-report a history of ever being pregnant¹

TYPES OF CAREGIVERS

Types of Caregivers Utilized by Adults (n=234)



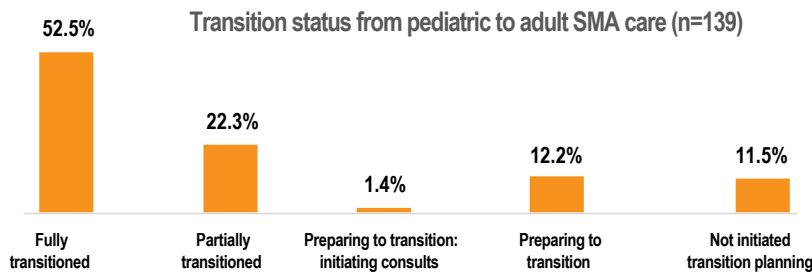
Caregivers outside of the immediate family are utilized in different capacities, with 28% being used < 20 hours weekly, 17% at 21-40 hours weekly, 22% at 41-80 hours weekly, and 36% at > 80 hours weekly.

91%
of adults with SMA report having one or more caregivers

Average Number of Caregivers: 3
Minimum: 1 | Maximum: 34

TRANSITION OF CARE

Findings from a 2021 Cure SMA survey on transition from pediatric to adult care found that 47.4% of adults ages 18-29 have not yet started transitioning or were not fully transitioned to adult care.²



Most commonly reported challenges when consulting adult healthcare providers during the transition process:

- Access to clinicians with experience in SMA
- Adult healthcare providers' knowledge of SMA
- Adult healthcare providers' responsiveness to respondent's specific concerns during visits

Cure SMA Data Sources: Membership data and 2023 CUS

1. Pregnancy data is from deduplicated individuals from the 2019-2023 CUS. Pregnancy is self-reported and outcomes of pregnancy are not collected.

2. Findings presented at 2023 MDA Clinical & Scientific Conference, Eisenman, et al.

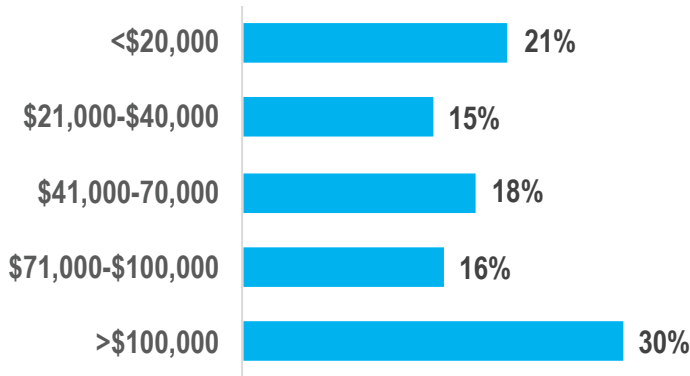
SOCIAL DETERMINANTS OF HEALTH

PATIENT/CAREGIVER REPORTED

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



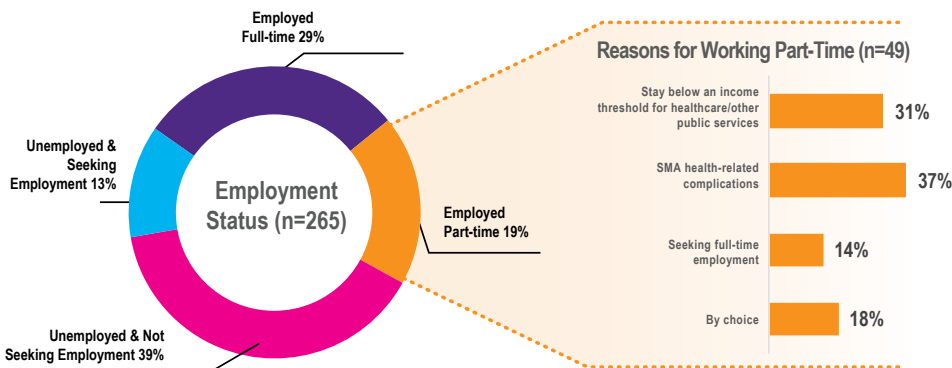
Household Income (n=234)



In the U.S., an average of 1 in 10 people currently live in poverty. Those living in poverty are less likely to have quality health care access, healthy food, and stable housing²

8% of adults with SMA reported that in the last 12 months they had to skip buying medications or going to doctor's appointments to save money¹

9% of adults with SMA reported in the last 12-months they worried that their food could run out before they got money to buy more¹



Cure SMA Data Sources: 2023 CUS

1. Data is from adults who self-completed questions from the Montefiore SDOH Assessment
2. US Department of Health and Human Services. (n.d.). Reduce the proportion of people living in poverty - SDOH-01. Reduce the proportion of people living in poverty - SDOH-01 - Healthy People 2030. <https://health.gov/healthypeople/objectives-and-data/browse-objectives/economic-stability/reduce-proportion-people-living-poverty-sdoh-01>

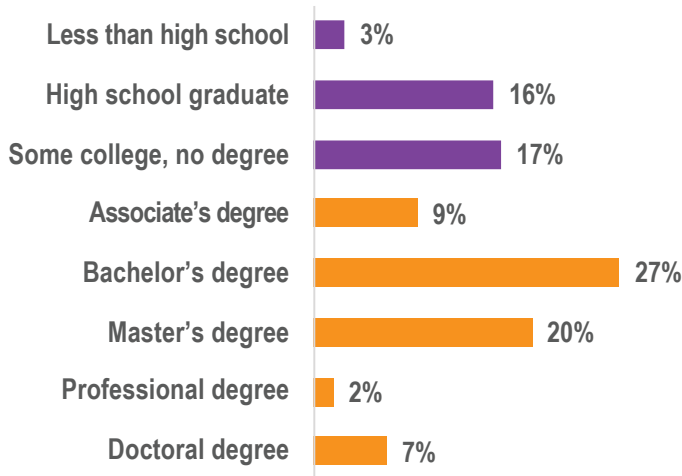
Analysis Notes:

- All graphics include individuals that were 18 years of age and older and living when completing the 2023 CUS

SOCIAL DETERMINANTS OF HEALTH

PATIENT/CAREGIVER REPORTED

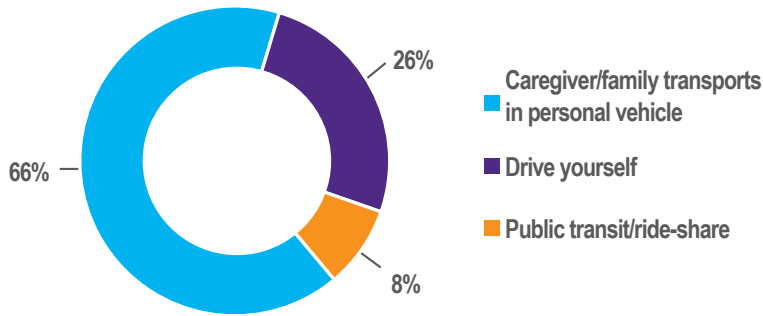
HIGHEST LEVEL OF EDUCATION COMPLETED IN ADULTS 18 YEARS AND OLDER (n=292)



Higher education has been shown to improve health and well-being and reduce risk of premature death. It also directly impacts employment options, economic security, and zip-code.¹

64% of adults with SMA have gone on to complete some form of higher education

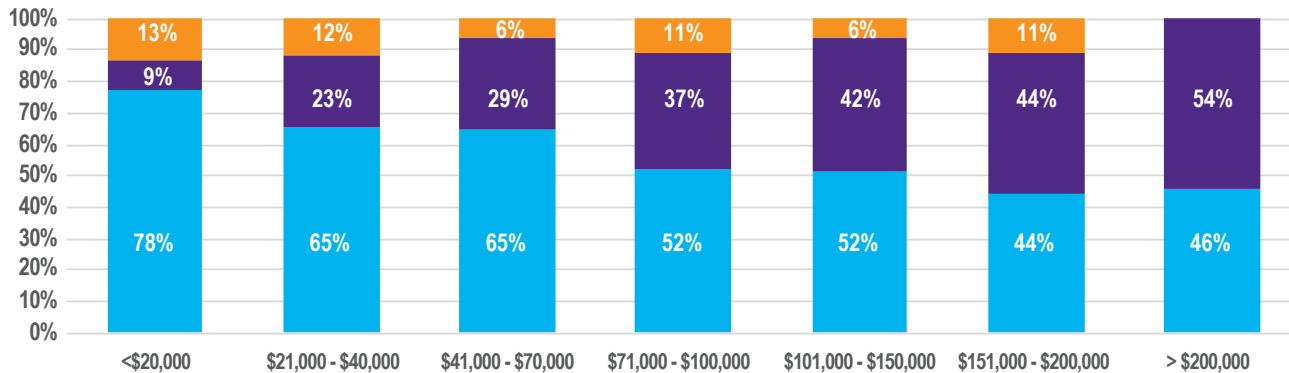
TRANSPORTATION: HOW OUR COMMUNITY IS ACCESSING THEIR HEALTHCARE SERVICES (n=222)



13% of adults with SMA reported that in the last 12 months lack of transportation kept them from medical appointments or getting their medications²

Transportation method was not shown to affect an individual's ability to access SMA treatment

Transportation Method by Household Income



Cure SMA Data Sources: 2023 CUS

1. US Department of Health and Human Services. (n.d.). Enrollment in Higher Education. Healthy People 2030. <https://health.gov/healthypeople/priority-areas/social-determinants-health/literature-summaries/enrollment-higher-education>
2. Data is from adults who self-completed questions from the Montefiore SDOH Assessment

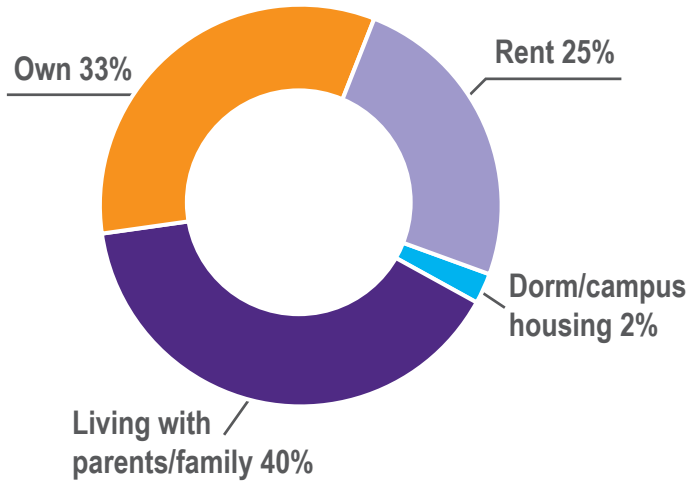
Analysis Notes:

- All graphics include individuals that were 18 years of age or older and living when completing the 2023 CUS.

SOCIAL DETERMINANTS OF HEALTH

PATIENT/CAREGIVER REPORTED

HOUSING: WHERE OUR COMMUNITY IS LIVING (n=244)



2% of adults with SMA reported that in the past 12-months their electric, gas, oil, or water company has threatened to shut off services to their home.¹

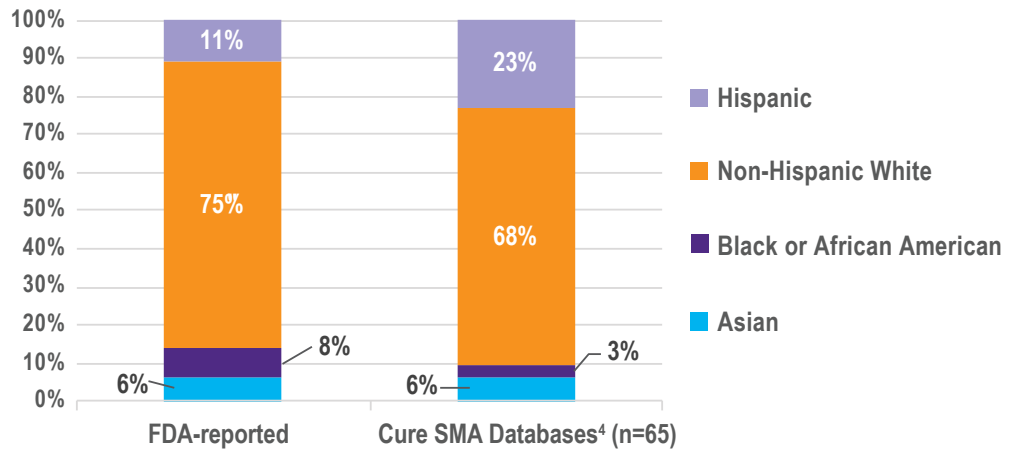
5% of adults with SMA reported that they are worried they may not have a safe or stable place to live in the next 2 months (i.e. eviction, being kicked out, homelessness).¹

5% of adults with SMA reported that they are worried the place they are currently living is making them sick (i.e. mold, bugs/rodents, water leaks, not enough heat).¹

As of 2020 FDA reported data, industry sponsored clinical trials underrepresented minority groups.²

Historically, clinical trials primarily recruited White male study participants.³ Inclusivity and diversity is pertinent in clinical trial recruitment to ensure representation of the whole population.

Clinical Trial Participation by Race



SAFETY AND WELL-BEING

Adults with SMA who completed the 2023 CUS themselves were asked a series of questions on their health and well-being using the Montefiore SDOH assessment.⁵ The following are self-reported statistics on the safety and well-being among adults with SMA.

7% of adults with SMA reported needing legal assistance

4% of adults with SMA reported that someone in their life hurts, threatens, or frightens them, or makes them feel unsafe

17% of adults with SMA reported that it is hard to get along with a partner, spouse, or family members

If you are feeling unsafe at home 24/7 confidential help can be reached by calling 1-800-799-SAFE (7233) or 911. You are not alone.

Cure SMA Data Sources: 2023 CUS

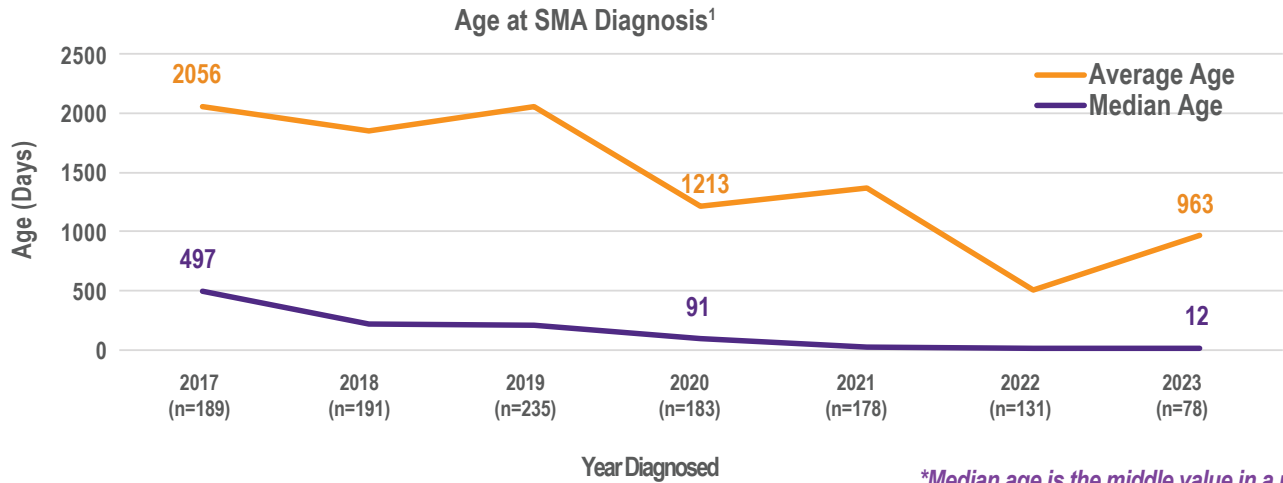
1. Data is from adults who self-completed questions from the Montefiore SDOH Assessment
2. Cavazzoni, P., Anagnostiadis, E., & Lolic, M. (2021, February). 2020 Drug Trials Snapshots Summary Report. <https://www.fda.gov/media/145718/download>
3. U.S. Department of Health and Human Services. (2023b, April 24). Diversity and inclusion in clinical trials. National Institute of Minority Health and Health Disparities. <https://www.nimhd.nih.gov/resources/understanding-health-disparities/diversity-and-inclusion-in-clinical-trials.html>
4. Individuals reporting they have participated in a clinical trial in the 2023 CUS.
5. NCQA. (2020, September). Social Determinants of Health. https://www.ncqa.org/wp-content/uploads/2020/10/20201009_SDOH-Resource_Guide.pdf

Analysis Notes:

- All graphics include individuals that were 18 years of age or older and living when completing the 2023 CUS.

DIAGNOSIS OF SMA

Our data shows that the average and median* time to diagnosis of SMA has decreased since 2017, which is likely impacted by the increase in individuals identified via prenatal screening (PS) or newborn screening (NBS).

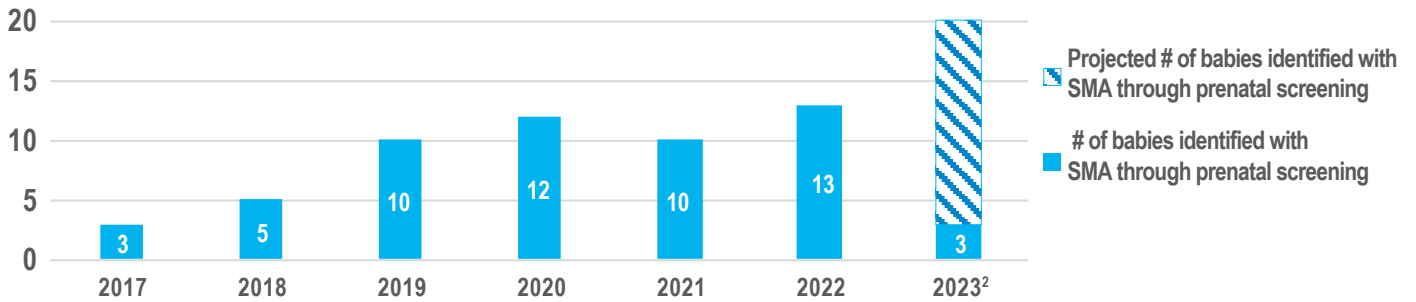


*Median age is the middle value in a range.

For example, at least 50% of individuals diagnosed in 2023 in our data were diagnosed by 12 days of age.

PATIENT/CAREGIVER REPORTED

Individuals Identified with SMA Through Prenatal Screening, by Year of Birth



We are observing an increase in the number of babies identified with SMA through prenatal screening since 2017.

Percent Diagnosed via Prenatal / Newborn Screening in 2023¹ (n=78)

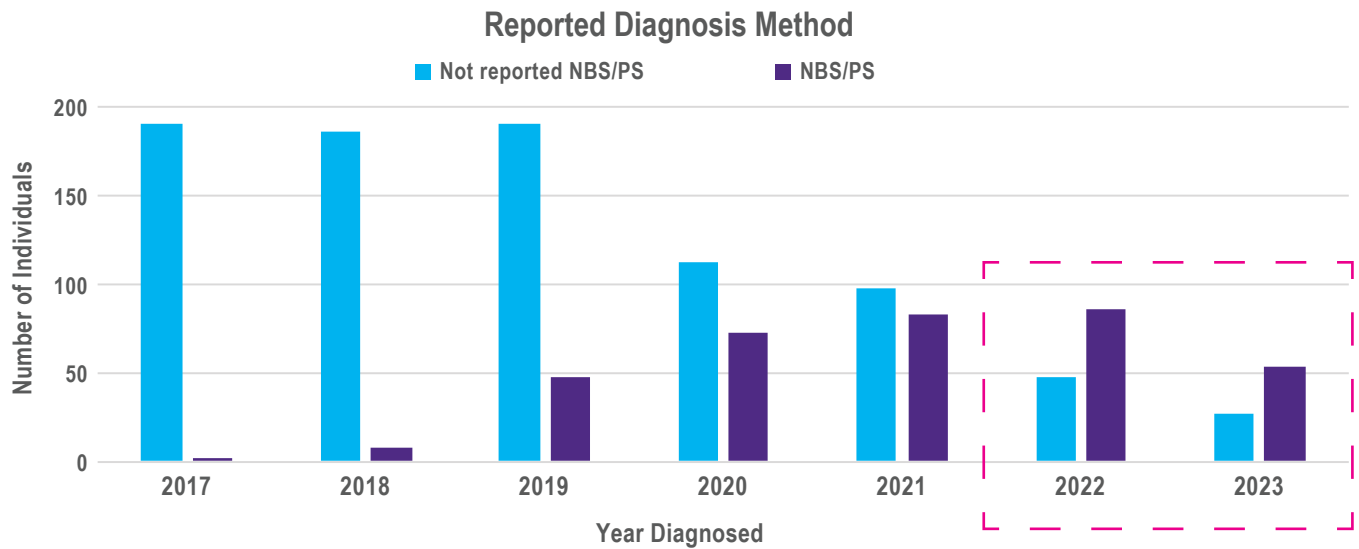


Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Included individuals where the date of diagnosis was not reported to be estimated. The minimum and maximum values were removed from each group.
 2. 2023 data collection is still in progress

LET'S TAKE A DEEPER LOOK

Now that more U.S. states have implemented newborn screening, our data is showing that the number of individuals diagnosed via NBS/PS are **higher** than the number of individuals that were not, which is likely driving the general decrease in overall average age at diagnosis.

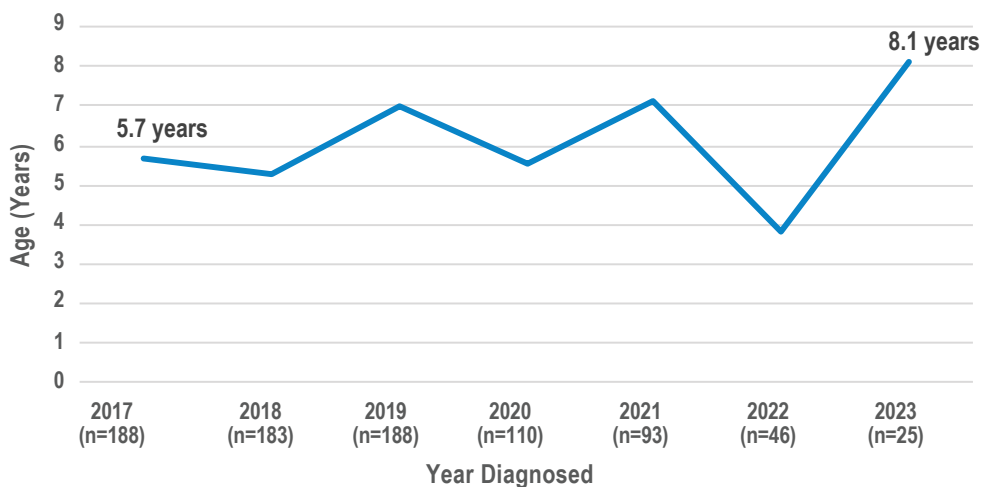


Over time, we may start to see the number of individuals identified by screening plateau or decrease due to decreases in the birth rate in the U.S., an increase in carrier screening, and 100% U.S. of states implementing NBS programs.

WHY DIDN'T THE 2023 AVERAGE AGE AT DIAGNOSIS CONTINUE TO DECREASE?

While the number of individuals identified via symptoms will likely continue to decrease, we anticipate that the average age at diagnosis of those individuals may increase because moving forward, most infants born with SMA will be identified by screening and more individuals diagnosed via symptoms may be diagnosed at an older age due to later-onset symptoms.

Individuals Not Identified by Screening: Average Age at SMA Diagnosis¹⁻²



In our data, the average age of diagnosis for individuals not reported to be diagnosed via screening in 2023 was 8.1 years old

Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Sample includes anyone not reported to be identified via newborn screening
2. The minimum and maximum values were removed from each year

SMA TREATMENTS IN THE U.S.

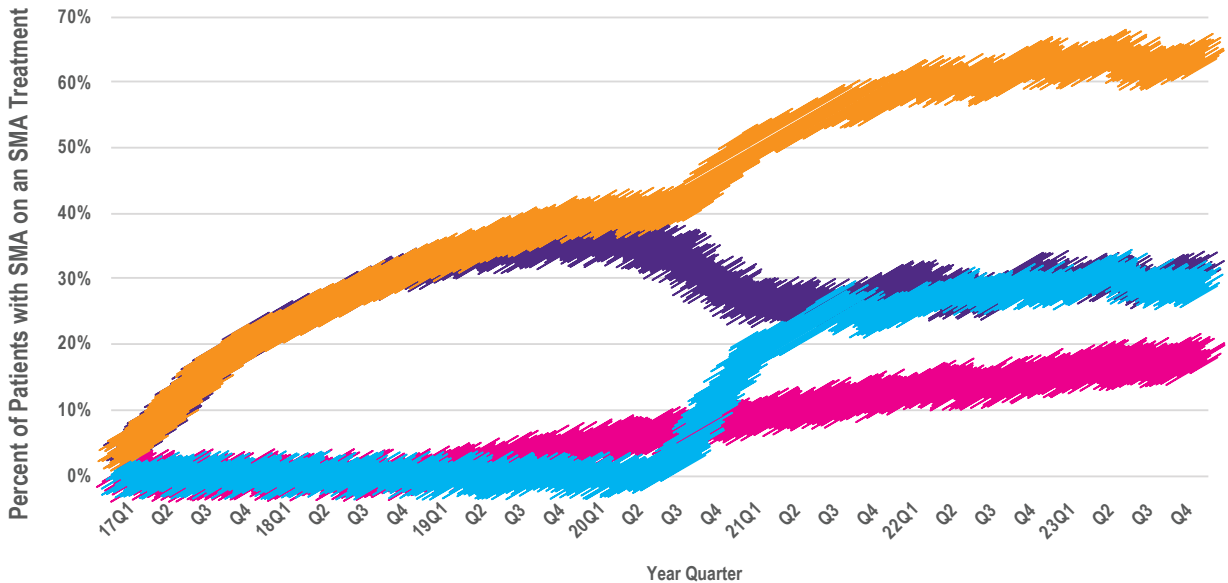
SMA TREATMENT PIPELINE

Organization/Drug Name	Mechanism of Action	Clinical Development Phase	FDA Approval Date	Age Eligibility	SMA Type Eligibility
Biogen/Spinraza® (nusinersen)	Modulation of <i>SMN2</i>		12/23/2016	All ages	All SMA types
Novartis/Zolgensma® (onasemnogene abeparvovec-xioi)	Gene Therapy		5/24/2019	Individuals < 2 years old	All SMA types
Roche-Genentech/Evrysdi® (risdiplam)	Modulation of <i>SMN2</i>		8/7/2020	All ages	All SMA types
Scholar Rock/Apitegromab	Muscle-directed therapy	Phase 3			
Novartis/OAV101	Gene Therapy	Phase 3			
Roche-Genentech/GYM329	Muscle-directed therapy	Phase 3			
Biohaven/Taldefgrobep alfa	Muscle-directed therapy	Phase 3			

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

APPROXIMATELY 60-70% OF INDIVIDUALS WITH SMA IN THE U.S. HAD RECEIVED AN FDA APPROVED TREATMENT IN Q4 2023¹⁻³

Percent of Patients Currently on SMA Treatment in the U.S.



Data Sources: Internal modeled estimates derived from quarterly earnings reports from Biogen, Roche, and Novartis.

These are estimates and may over or underrepresent treatment utilization

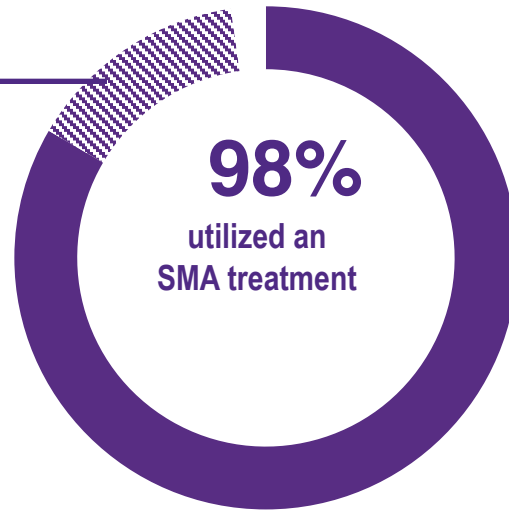
1. The sum of all treatments data is accounting for an estimated 15% of concurrent treatment use
2. Anyone treated with Zolgensma® was categorized as "currently on treatment" for all quarters following treatment
3. Data here is presented by standard calendar quarters: January, February, and March (Q1) April, May, and June (Q2) July, August, and September (Q3) October, November, and December (Q4)

TREATMENT: OBSERVATIONS IN OUR DATA

CHILDREN

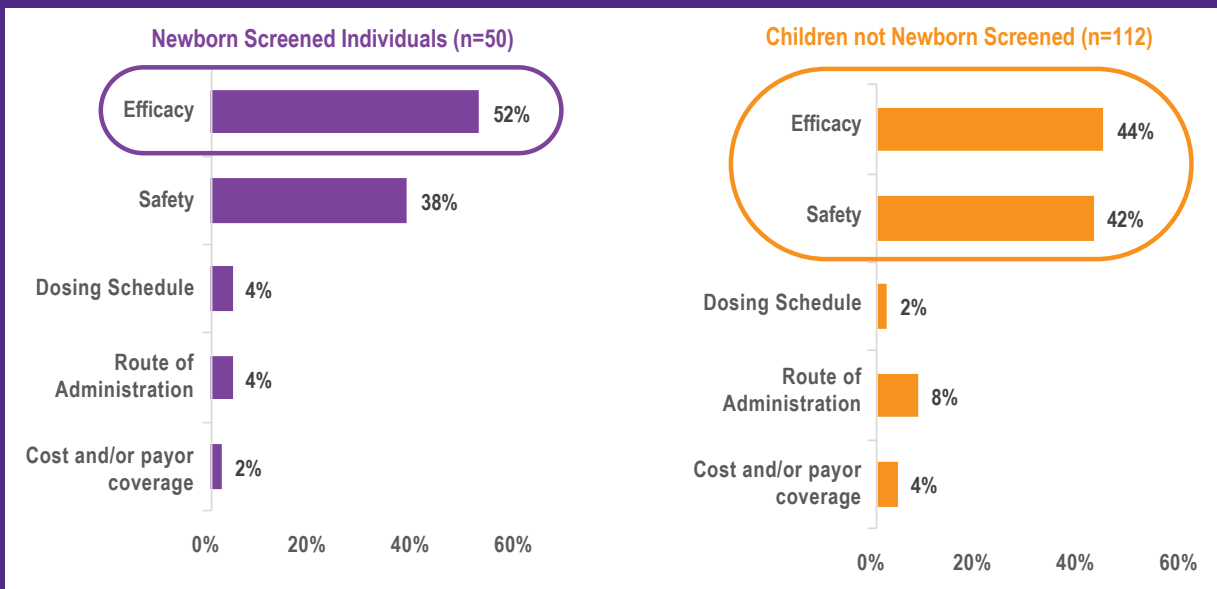
Utilization of SMA Treatment¹ (n=1,261)

~15% reported ≥ 1 treatment was received in a clinical trial



PATIENT/CAREGIVER REPORTED

MOST IMPORTANT FACTOR WHEN CHOOSING AN SMA TREATMENT²



Treatment efficacy stood out at the most important factor when choosing an SMA treatment among children. For children not diagnosed via newborn screening, safety of treatment was an equally important factor.

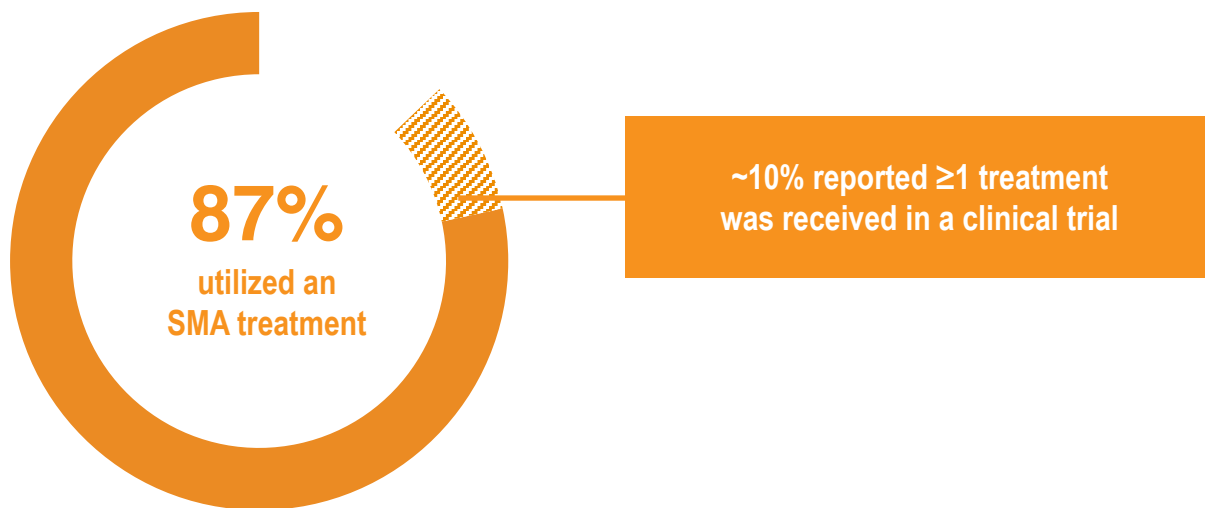
Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Based on individuals with treatment status information available – includes both FDA approved and investigational treatments
2. Based on data from the 2022-2023 CUS. Individuals who completed surveys in both years were de-duplicated, with data from 2023 survey being used in the analysis

TREATMENT: OBSERVATIONS IN OUR DATA

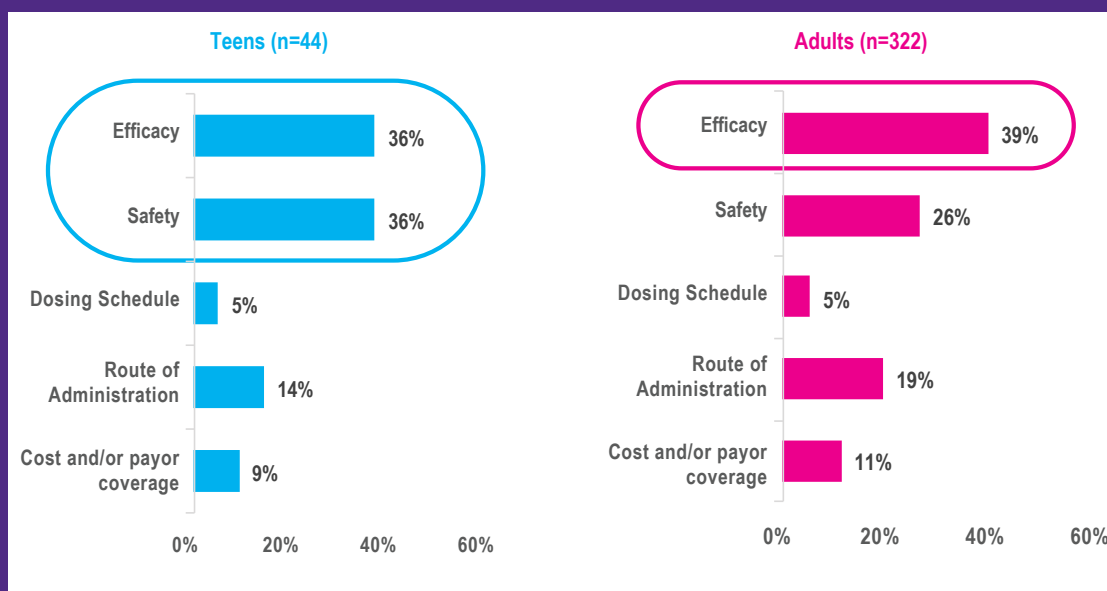
Utilization of SMA Treatment¹ (n=1,328)

TEENS AND ADULTS



PATIENT/CAREGIVER REPORTED

MOST IMPORTANT FACTOR WHEN CHOOSING AN SMA TREATMENT²



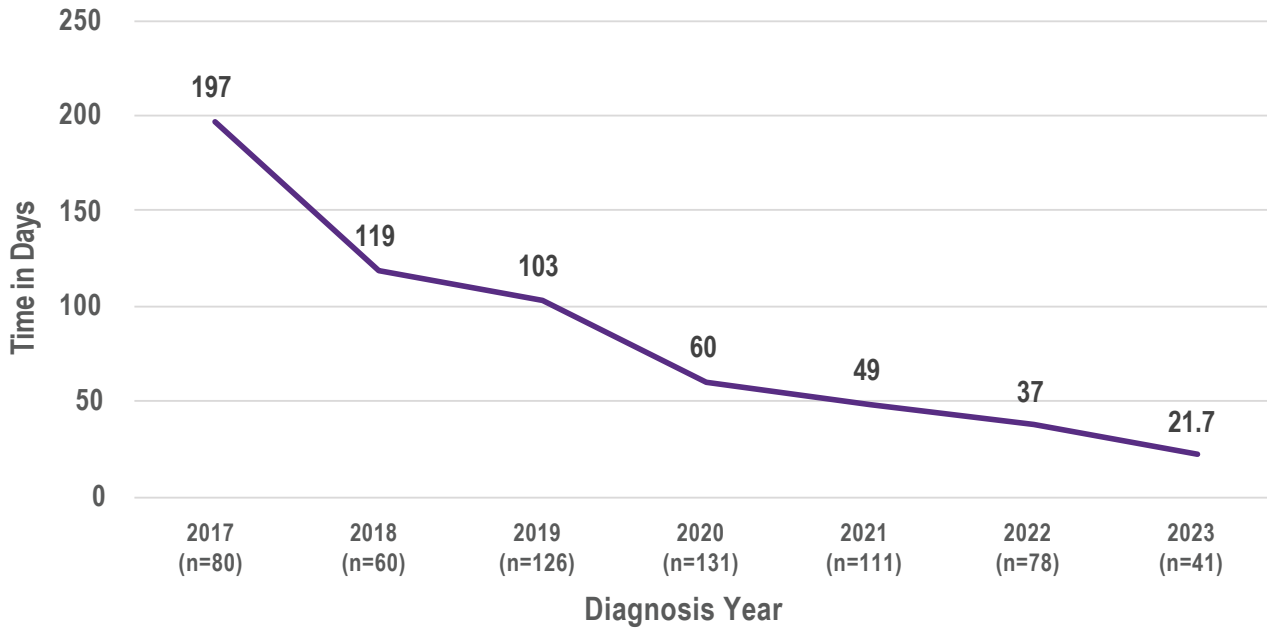
Similarly, treatment efficacy stood out at the most important factor when choosing an SMA treatment among teens and adults. Adults ranked treatment safety lower than other age groups. We also observed that route of administration and cost and/or payor coverage were ranked higher in teens and adults compared to children.

Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Based on individuals with treatment status information available – includes both FDA approved and investigational treatments
2. Based on data from the 2022-2023 CUS. Individuals who completed surveys in both years were de-duplicated, with data from 2023 survey being used in the analysis

TREATMENT: TIMING OF TREATMENT INITIATION

Average Time between Diagnosis and First SMA Treatment¹⁻³



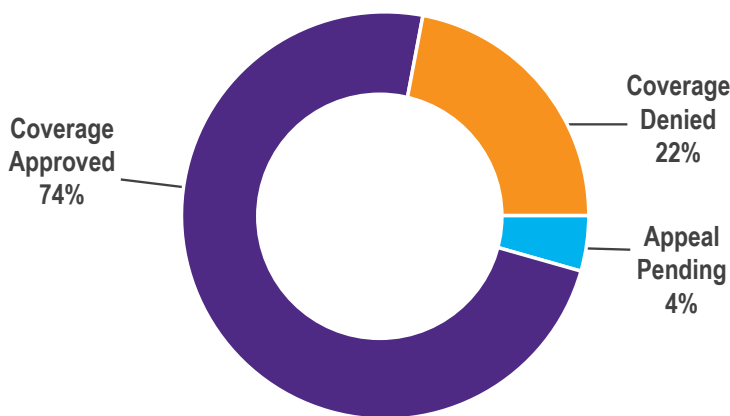
PATIENT/CAREGIVER REPORTED

INSURANCE APPEALS AND DENIALS

55%

of individuals treated with an SMA therapy have received an insurance denial related to SMA treatment coverage

Outcome of Appealed Insurance Denial (n=227)



After appealing initially denied coverage, nearly three-quarters of individuals received approval for SMA treatment.

When stratified by age, similar appeal outcomes were seen between both pediatrics and adults.

Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

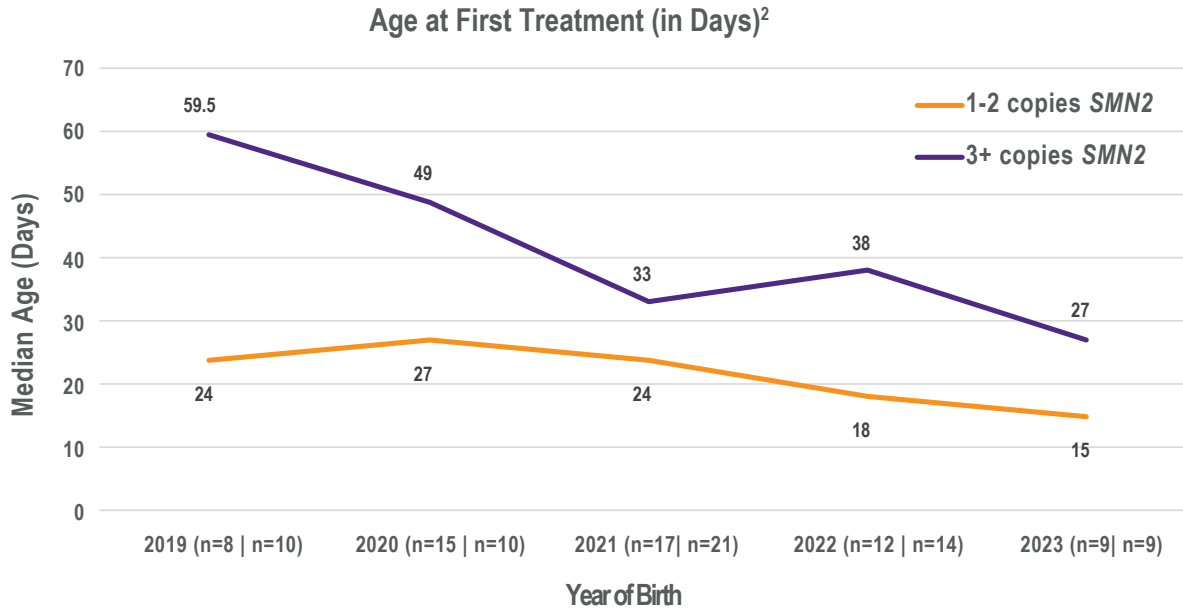
1. Treatment was defined as any evidence of utilization of an FDA approved therapy as of 12/2023.
2. The minimum and maximum values were removed from each year
3. Analysis included individuals where the date of diagnosis occurred on or before date of first treatment

TREATMENT: NEWBORN SCREENING

INDIVIDUALS IDENTIFIED BY NBS

CLINICIAN REPORTED DATA

Newborn screening allows for earlier diagnosis, which impacts the timing of intervention. The median age at first treatment has decreased since 2019 in our data.¹



TREATMENT BRIDGING



For individuals in the CDR, we ask clinicians from our Care Center Network about how they intended for SMA treatment to be used when it was prescribed (e.g., bridge, to be used at the same time as another SMA treatment, etc.):

19% of individuals with clinician-reported treatment intent received a bridge treatment at any time. Future analyses are planned to assess insurance coverage of bridge therapies.

Cure SMA Data Sources: CDR (individuals identified via NBS)

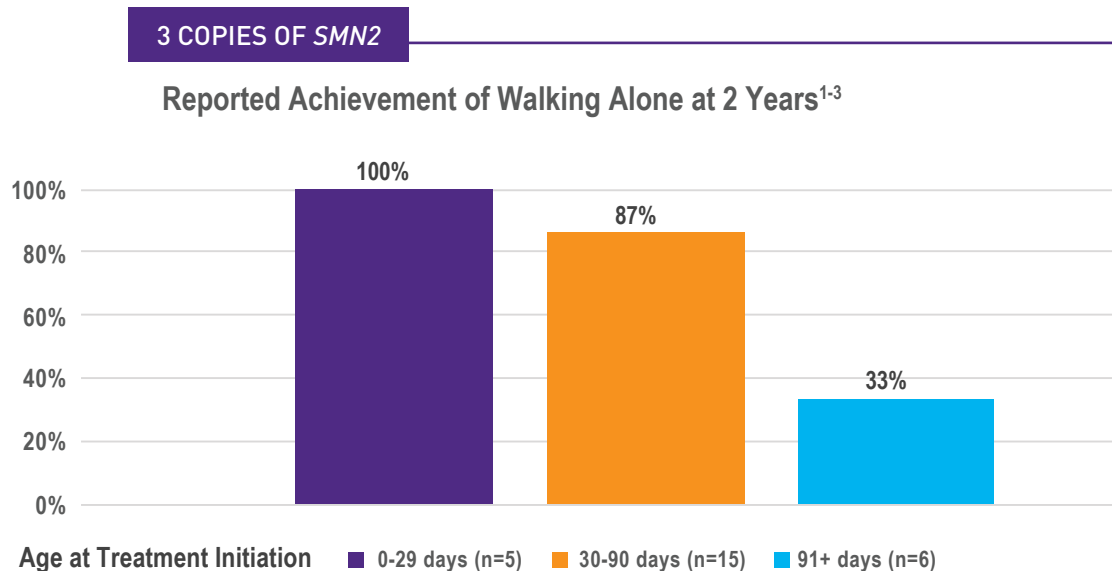
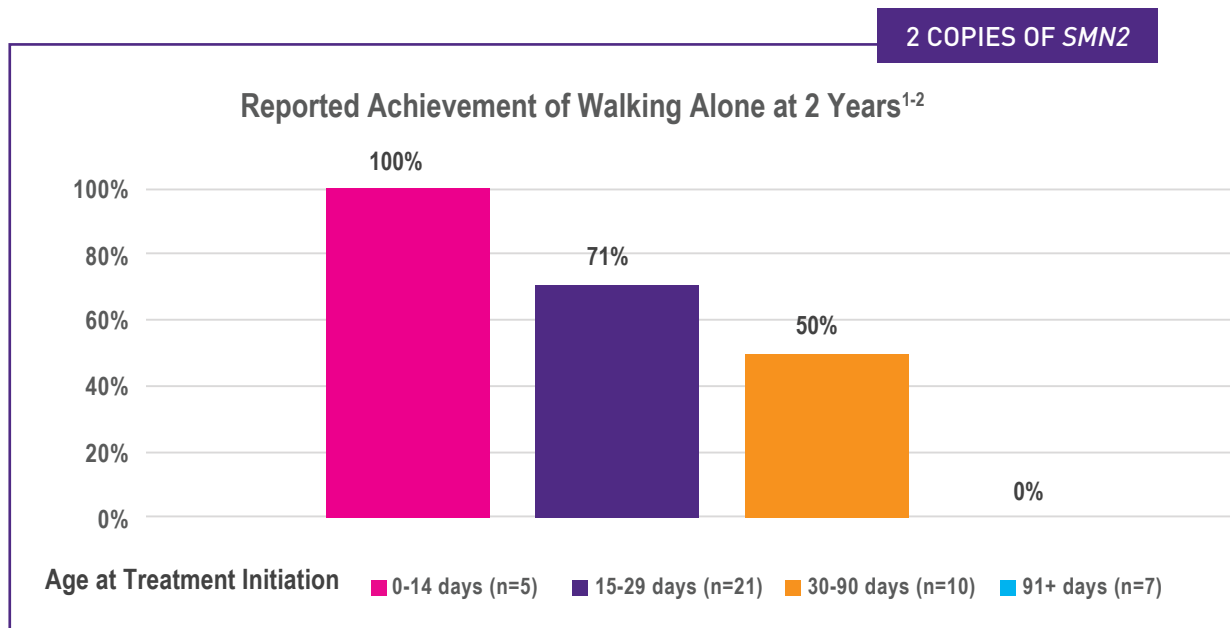
1. Median age is the middle value in a range
2. Individuals were removed if there any treatment utilization via clinical trial, their treatment start date was prior to their birthdate, or if any treatment start dates were unknown

TREATMENT: IMPACT OF EARLY TREATMENT

OUR DATA SHOWS THE IMPORTANCE OF EARLY TREATMENT INITIATION.

In our data, we observed that a higher percentage of individuals that initiated their first treatment before 30 days old were reported to walk alone at 2 years of age compared to individuals that initiated treatment later. This analysis used real world data, and individuals were 24-35 months when the data was collected. Parents and caregivers (CUS), or clinicians (CDR) used their discretion on if the individual could walk unassisted.

Importantly, walking unassisted is only one measure of success. These results are based on observations in our data and results may not be generalizable to all individuals with SMA as the overall clinical course for an individual patient is complex and multifactorial.



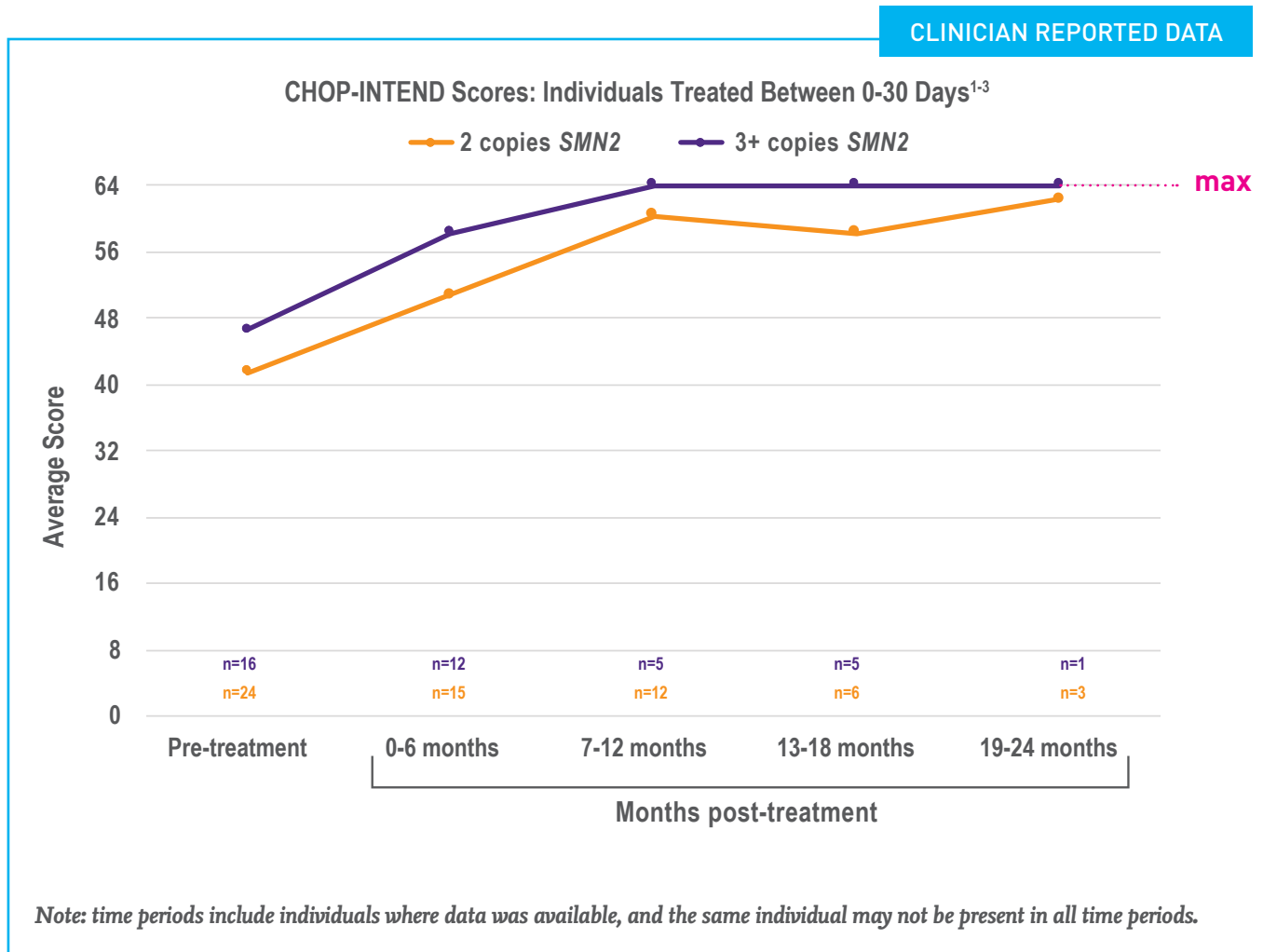
Cure SMA Data Sources: CDR (2022-2023) and CUS data (2021-2023)

1. This analysis included unique individuals with 5q SMA that resided in the United States (U.S.) who were 2 years old at the time of data collection (CDR eCRF or CUS completion) and had a known current motor milestone achievement reported
2. Sample sizes for 1 and 4+ copies were too small to report
3. Age at treatment initiation groups were collapsed into "0-29 days" for individuals with 3 copies of SMN2 due to limited sample size

TREATMENT: IMPACT OF EARLY TREATMENT

OUR DATA SHOWS THE IMPORTANCE OF EARLY TREATMENT INITIATION.

In our Clinical Data Registry, we collect motor function assessment scores over time. This graphic is plotting CHOP-INTEND scores over time of individuals who initiated treatment between 0-30 days. The motor function assessment data is collected during clinical care appointments and varies for each individual, which is why we collapsed the assessment timing into 6-month windows after treatment was initiated. The maximum CHOP-INTEND score is 64.



WHAT IS THE CHOP-INTEND?

The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) was developed to measure the motor skills of infants and young children with SMA Type 1 and other neuromuscular disorders of infancy.

The test is scored from 0-64, with 64 indicating higher strength and motor function.

This tool has been utilized in multiple natural history studies of SMA Type 1, as well as in multiple SMA clinical trials.

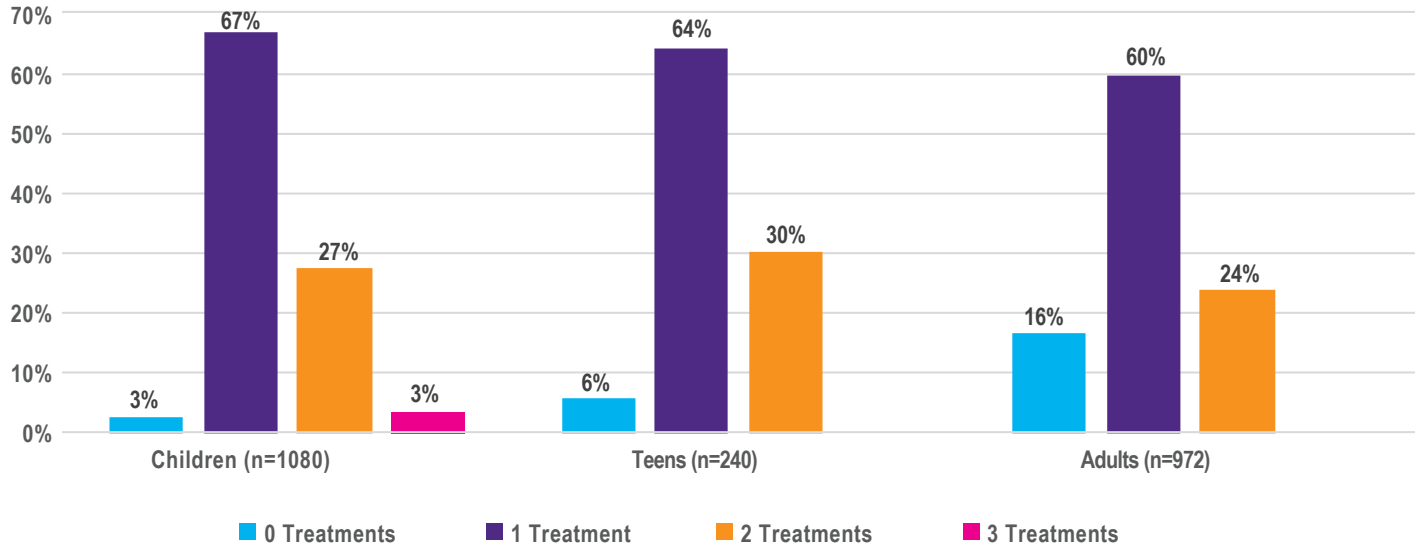
Cure SMA Data Sources: CDR

1. Analysis includes individuals treated between 0-30 days with at least one pre-treatment and one post-treatment CHOP-INTEND score.
2. This is a descriptive, non-adjusted analysis.
3. This is not truly longitudinal as the same individuals are not included in every time period.

TREATMENT: USE OF MULTIPLE SMA TREATMENTS

Approximately 28% of individuals that have not participated in a clinical trial have received 2 or more FDA approved SMA treatments in our data.

Number of FDA Approved SMA Treatments Utilized¹ (n=2,292)



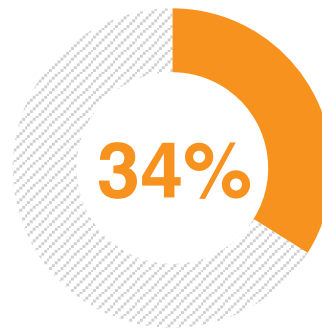
CLINICIAN REPORTED DATA

Use of Concurrent Treatment in Individuals Utilizing Multiple Treatments (Based on Prescriber Intent)¹

In the CDR we collect information on how the prescriber intended for the SMA treatment to be used. For children, any SMA treatments initiated after Zolgensma® were considered concurrent.

For individuals who have utilized multiple treatments outside of a clinical trial, it was reported that the prescriber intended for treatment to be used at the same time (concurrently) for 34% of children and 0% of teens and adults.

Children (n=171)



Teens and Adults (n=139)



Cure SMA Data Sources: Membership data, CUS, CDR, and NBSR data combined. Individuals participating in multiple sources were de-duplicated.

1. Based on data from individuals with treatment status information on FDA approved treatments only. Individuals who have participated in a clinical trial have been removed from this analysis.

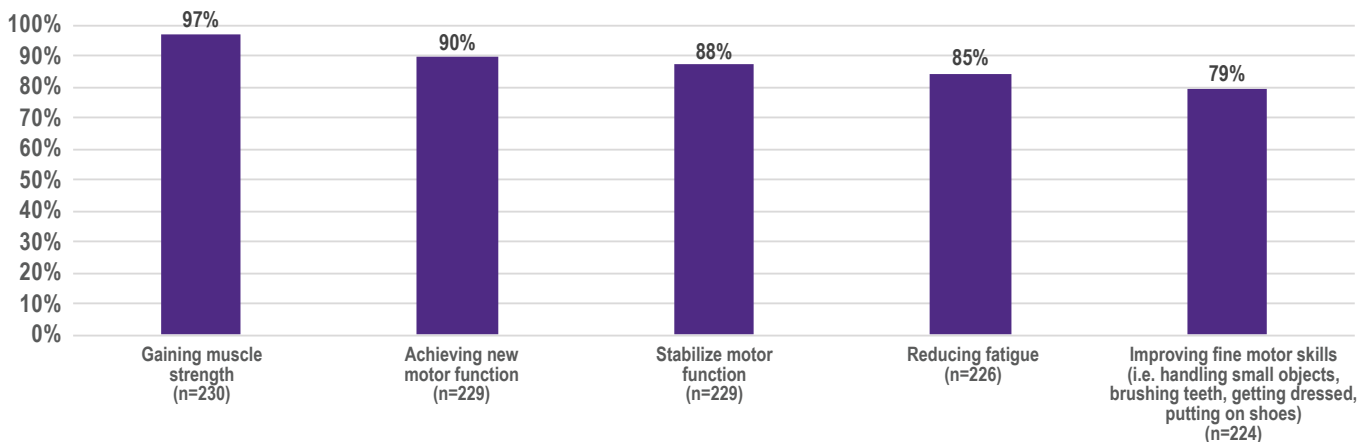
UNMET NEEDS

PATIENT/CAREGIVER REPORTED

Although advances in SMA treatment, early diagnosis and improved care have made substantial progress in the SMA community, significant unmet needs persists among the community, especially for those who may not have been treated early due to later disease onset, clinical parameter restrictions and/or age restrictions. Through the CUS, Cure SMA seeks to understand what are the current unmet needs among adults affected with SMA as well as the unmet needs of caregivers of both children and adults affected with SMA.

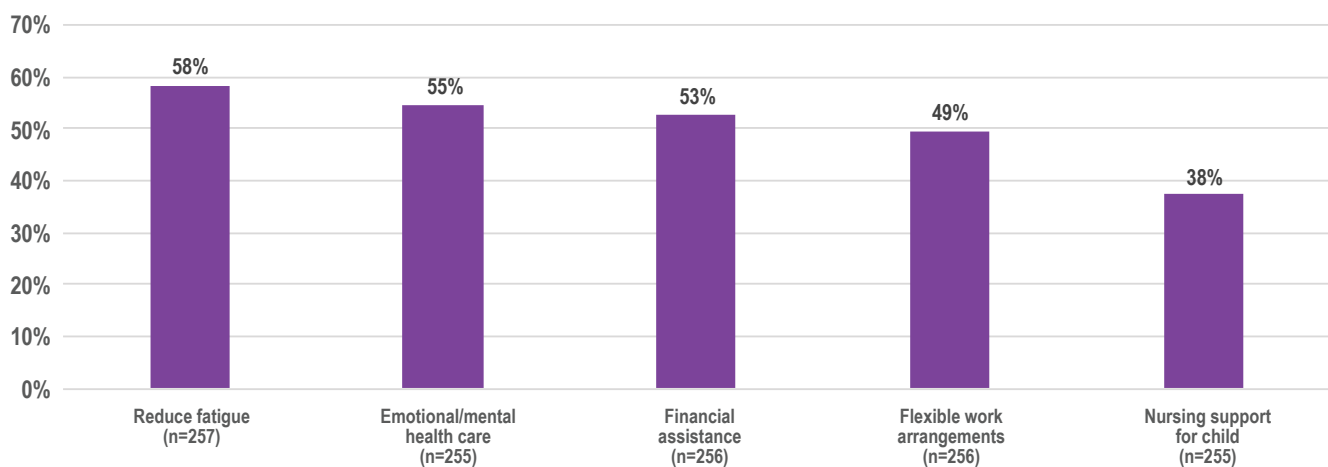
ADULTS

What are your most significant current unmet needs that you hope new therapies would address?



CAREGIVERS

What are your most significant unmet needs that you currently face caring for an individual with SMA?

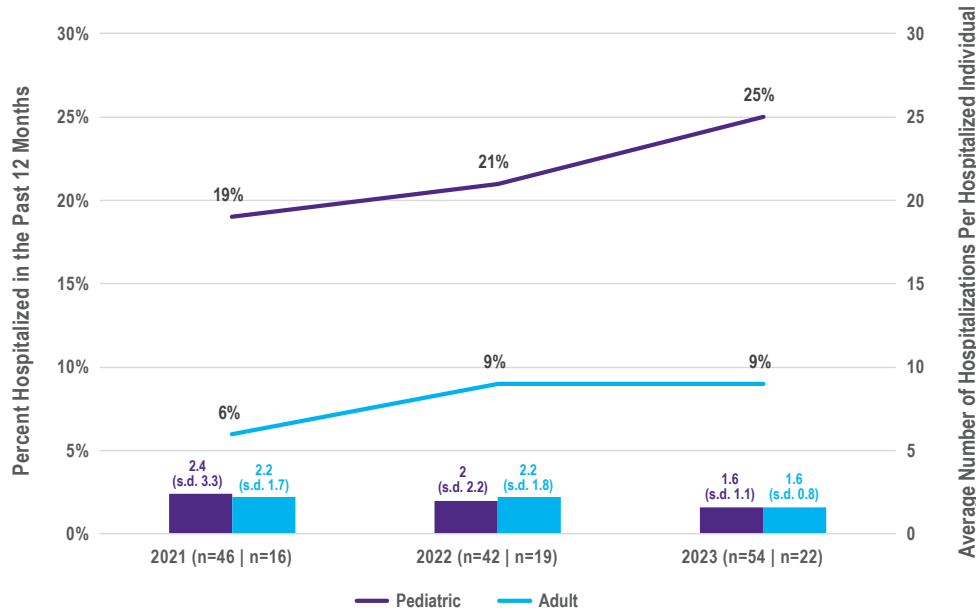


HOSPITALIZATIONS

PATIENT/CAREGIVER REPORTED

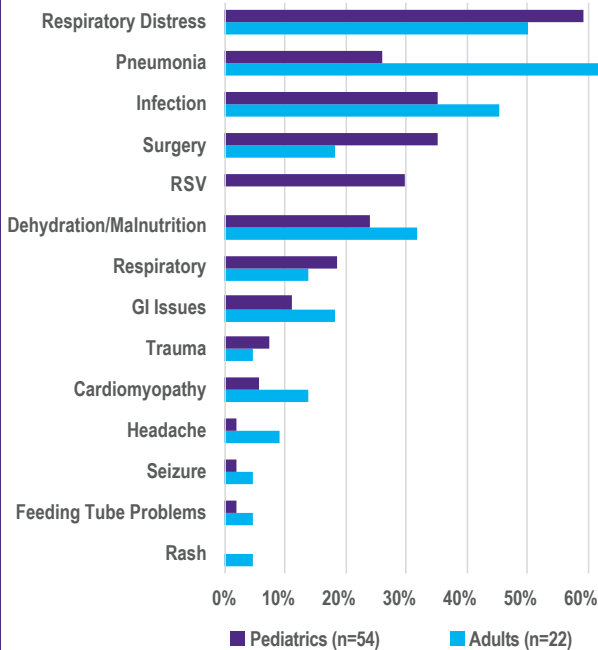
In 2023, 16% of individuals with SMA reported being hospitalized within the past 12-months. Among the pediatric population, 25% reported a hospitalization, and among adults, 9% reported a hospitalization.

Hospitalization Rate Among Pediatrics and Adults, and Average Number of Hospitalizations per Year: 2021-2023¹



Our data shows the proportion of both pediatric and adult individuals who report being hospitalized within a given year is increasing, however, the average number of hospitalizations per person per year is decreasing

REASONS FOR HOSPITALIZATION IN 2023 AMONG PEDIATRICS AND ADULTS



Individuals who reported being hospitalized in 2023 were asked to list reasons for their hospitalization(s).

Among pediatric individuals, respiratory distress (59%), surgery (35%), infection (35%), and respiratory syncytial virus (RSV) (30%) were the most common reasons for hospitalization.

Among adults, pneumonia (64%), respiratory distress (59%), infection (45%), and dehydration/malnutrition (24%) were the most common reasons for hospitalization.

Cure SMA Data Sources: CUS data (2021-2023)

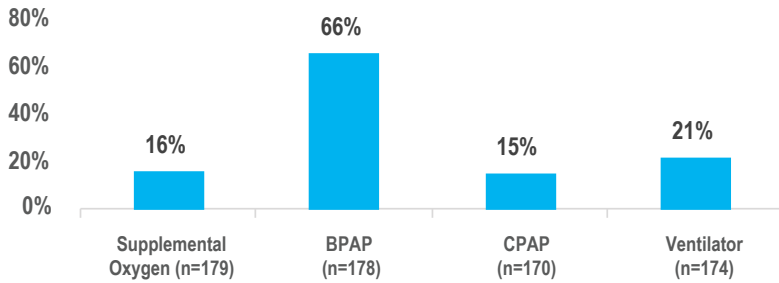
1. This is not truly longitudinal as the same individuals are not included in every time period

THERAPY: BREATHING SUPPORT

PATIENT/CAREGIVER REPORTED

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

Forms of Breathing Support Among Individuals Currently Requiring Breathing Support¹



38%

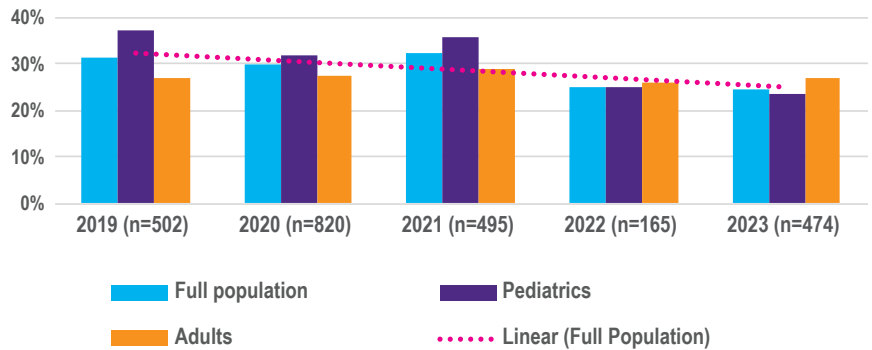
of individuals with SMA report currently using some form of breathing support. Among those on breathing support, BPAP use is the most prevalent form.

BPAP UTILIZATION

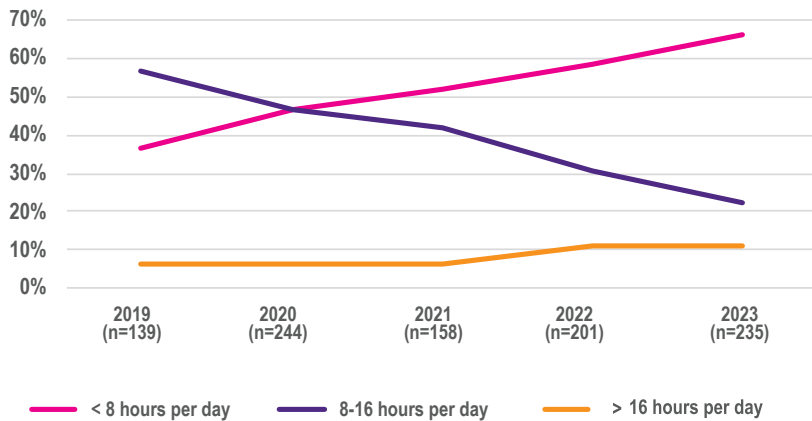
Bi-level positive airway pressure (commonly known under the trade name BiPAP), is a non-invasive form of breathing support that provides higher air pressure and volume during inhalation, and lower air pressure and volume during exhalation, allowing for a more normal breathing pattern.

We are seeing a decrease in the overall proportion of individuals who are using BPAP for breathing support. Similar trends are observed among pediatrics, with usage among adults remaining stable.

BPAP Utilization Among the Overall SMA Population and by Age Groups²



Hours per Day of BPAP Use²



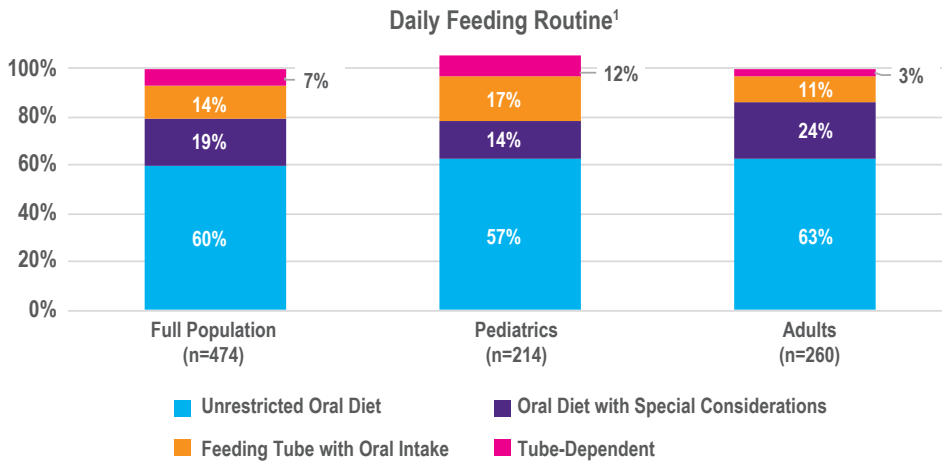
We observed an increase in individuals using BPAP for < 8 hours per day and a simultaneous decrease in those using BPAP for 8-16 hours per day, suggesting those in the intermediate usage group are requiring less usage per day. We are also observing a slight increase in those using BPAP for > 16 hours per day.

Cure SMA Data Sources: CUS data (2019-2023)

1. Data on forms of breathing support is of current data from 2023 CUS only
2. This is not truly longitudinal as the same individuals are not included in every time period

THERAPY: NUTRITION AND FEEDING

PATIENT/CAREGIVER REPORTED

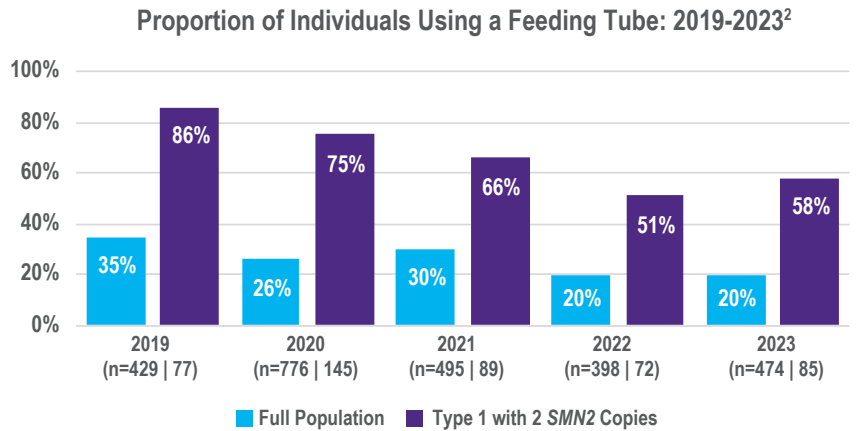


60%
of individuals report being on an unrestricted oral diet. When stratified by age, a higher proportion of pediatric individuals utilized feeding tubes compared to adults.

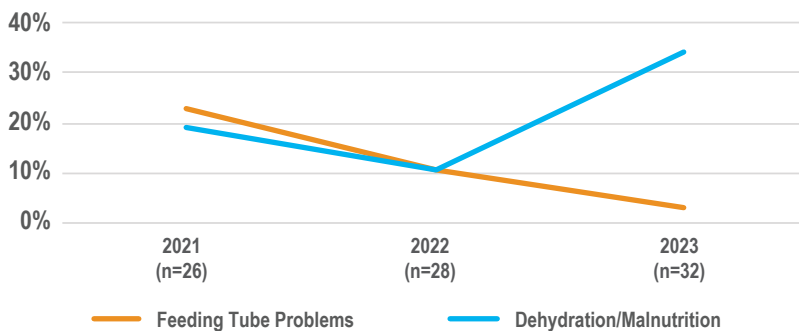
FEEDING TUBE USE

Feeding tubes may be necessary for some individuals with SMA to provide partial or total nutritional needs. Types of feeding tubes include a gastrostomy tube or nasogastric tube into the stomach, or a jejunostomy tube into the small intestine.

Individuals with Type 1 SMA with 2 copies of *SMN2* report much higher feeding tube utilization than the overall population. Our data shows the proportion of individuals using a feeding tube to be decreasing overall, except in 2023 when it increased among individuals with Type 1 SMA with 2 copies of *SMN2*.



Yearly Hospitalizations Due to Nutrition and Feeding Tubes Issues Among Individuals with Type 1 SMA with 2 *SMN2* Copies²



Among individuals with Type 1 SMA with 2 *SMN2* copies who have been hospitalized within a given year, we are observing a decrease in hospitalizations due to feeding tube problems, however, from 2022 – 2023 we see an increase in hospitalizations due to dehydration and/or malnutrition issues.

Cure SMA Data Sources: CUS data (2019-2023)

1. Data on daily feeding routine is of current data from 2023 CUS only
2. This is not truly longitudinal as the same individuals are not included in every time period

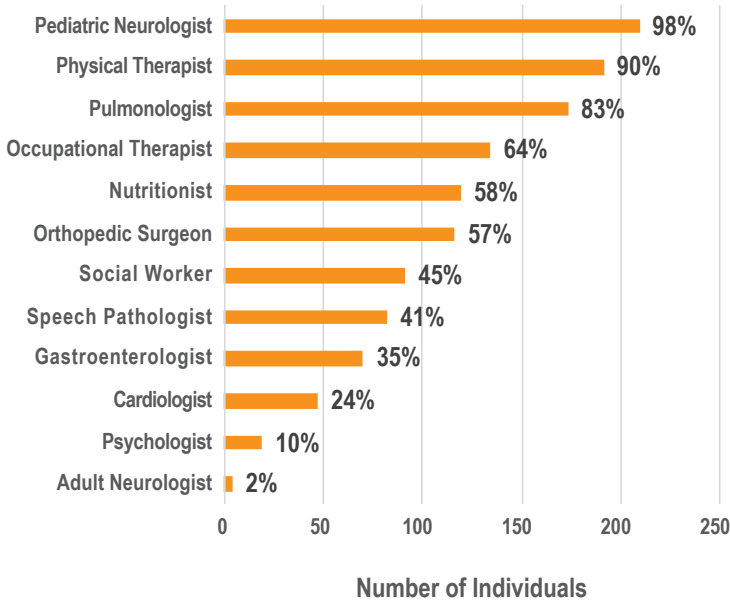
SPECIALISTS

PATIENT/CAREGIVER REPORTED

In 2023, 88% of pediatrics with SMA and 85% of adults with SMA reported having an in-person appointment with a physician or specialist for SMA related care.

PEDIATRIC INDIVIDUALS WITH SMA

Types of Specialists Seen in Past 12-months

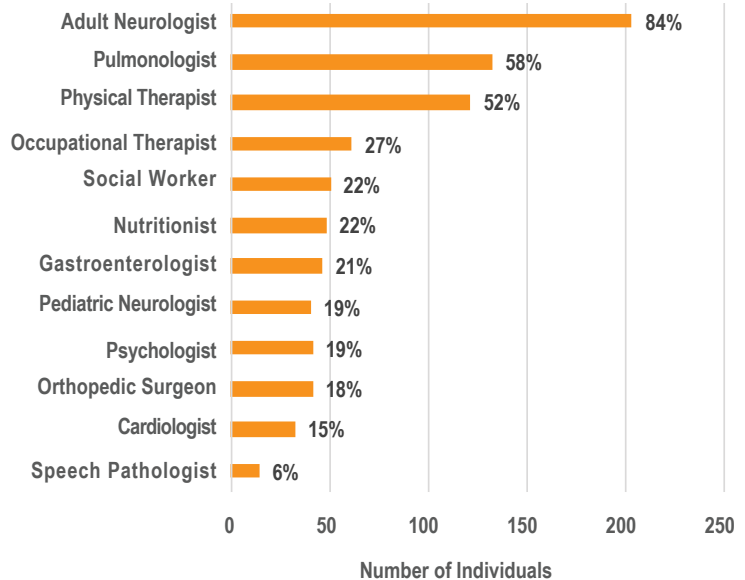


5.8
average number of specialists
seen within the year by
pediatric individuals

The most commonly seen specialists among pediatric individuals are pediatric neurologists, physical therapists, and pulmonologists.

ADULTS WITH SMA

Types of Specialists Seen in Past 12-months



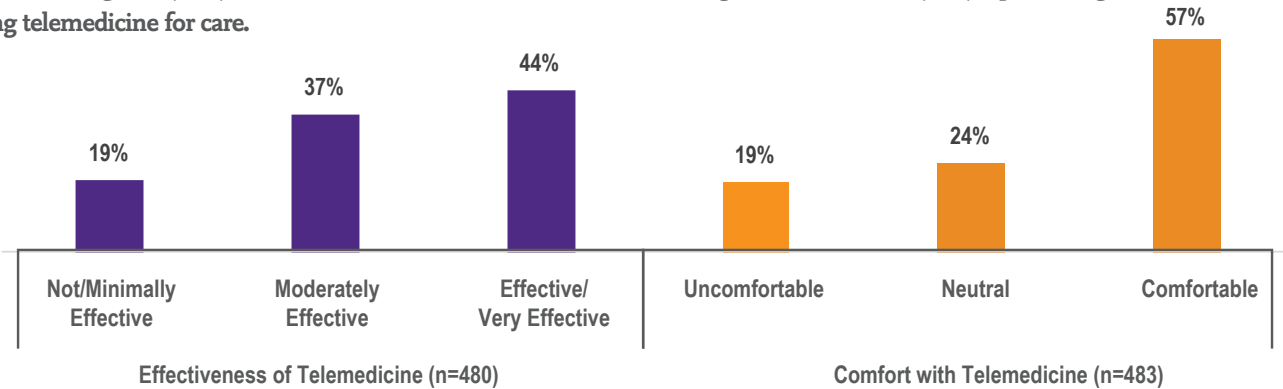
3.3
average number of
specialists seen within
the year by adults

The most commonly seen specialists among adults are adult neurologists, pulmonologists, and physical therapists.

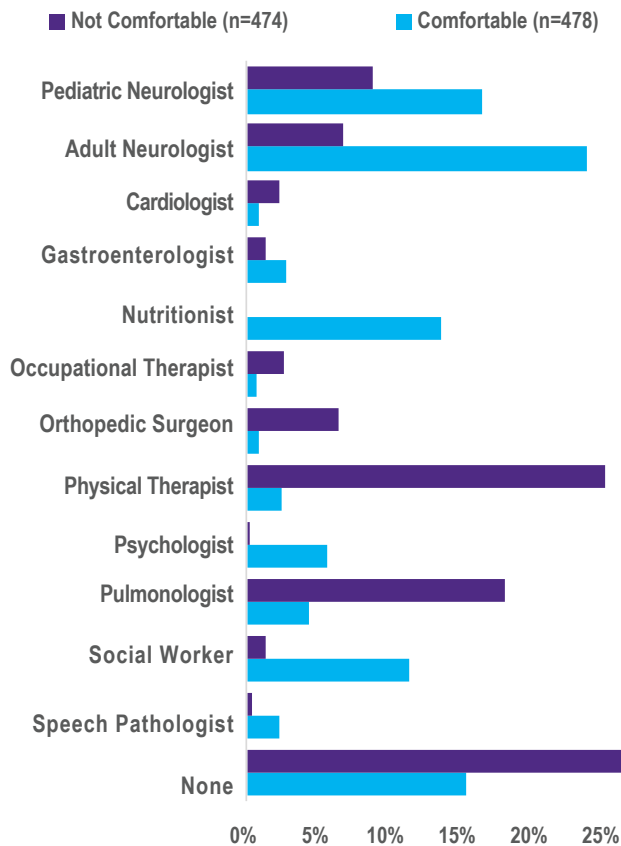
PATIENT/CAREGIVER REPORTED

79% of individuals with SMA reported utilizing telemedicine at least once. Among those individuals, 41% report that they prefer to use telemedicine for their medical appointments.

When asked about personal views of using telemedicine for SMA management and treatment, the majority of affected individuals and their caregivers (81%) believe telemedicine to be effective to some degree, and over half (57%) report being comfortable with using telemedicine for care.



Affected individuals and their caregivers were also asked to report which providers they would be most and least comfortable seeing via telemedicine for SMA treatment and care.

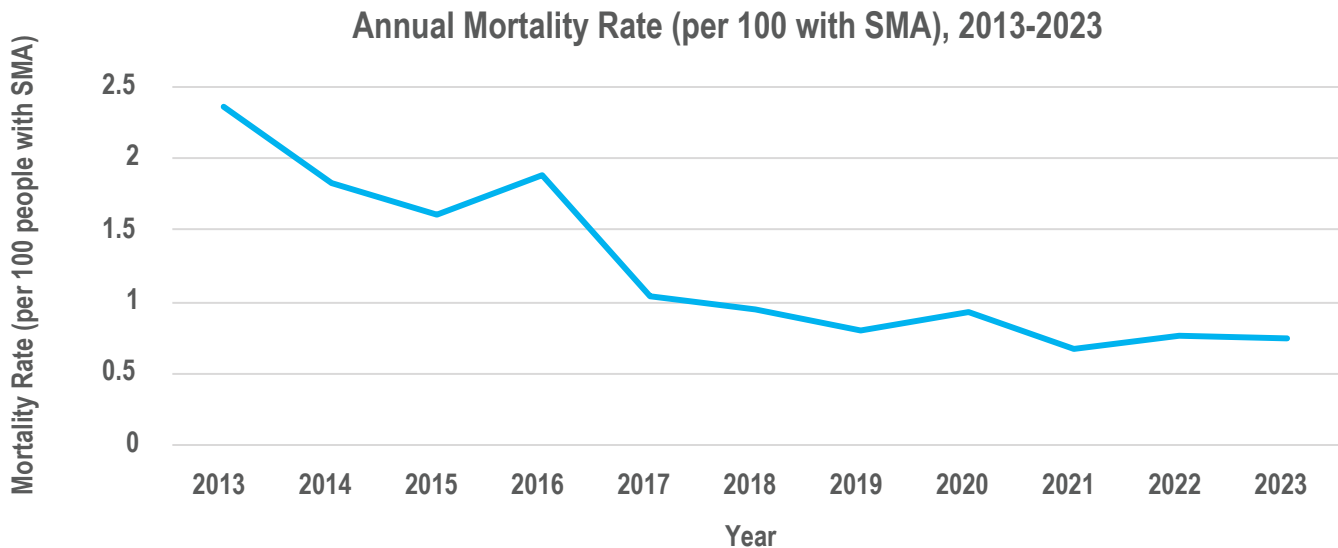


Individuals and their caregivers are most comfortable seeing specialists such as adult neurologists (24%), pediatric neurologists (16%), nutritionists (14%) and social workers (11%) via telemedicine.

15% report there being no specialists they are comfortable seeing via telemedicine.

Individuals and their caregivers are least comfortable seeing specialists such as physical therapists (25%) and pulmonologists (18%) via telemedicine.

The mortality rate of SMA in 2023 was approximately one-third of what it was in 2013, having decreased from **2.36 per 100** individuals to **0.75 per 100** individuals with SMA.



Cure SMA Data Sources: Membership Data & CDR

Analysis Notes:

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

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.

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