

COMPASS

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Using Data to Improve Research, Care and Coverage in the SMA Community

The spinal muscular atrophy (SMA) community is strong. For the families and individuals that SMA affects, however, more work remains to be done in order to continue to improve care and coverage—and ultimately to cure the disease.

Cure SMA has been collecting data through our database and various projects over the past several years. These projects have helped measure the impact of SMA, and helped identify areas where additional research, advocacy, and support are needed. To further advance this effort, in 2017, we initiated our first annual community survey to learn more about the clinical health care, family, and quality of life of those affected by SMA. We plan to build upon our initial results by releasing a survey each year and tracking changes in our community over time as new therapies are approved. Our next survey will be released in early 2018.

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It is our hope that these annual surveys will begin to demonstrate an evolving narrative of the SMA community — one that reflects the goals, hopes, and challenges of those impacted by the disease. Ultimately, our findings from these surveys will elevate awareness of the experiences of people living with SMA and their families.

Through the information gathered in the Cure SMA annual community survey, we intend to communicate our priorities to the SMA community, regulatory agencies, health care professionals, payers, and the research community, as well as provide insights on daily life with SMA and ongoing, related medical challenges.

Cure SMA's primary objective of the survey series is to develop communications, programs, and initiatives that will improve outcomes for individuals with SMA, raise awareness about SMA in the research community, and increase understanding of the burden of SMA.

For example, this information can:

- Highlight treatment patterns and health statuses to improve outcomes for individuals living with SMA;
- Ensure favorable insurance coverage for newly approved or future treatments; and
- Lead to a greater understanding of the impact of SMA.

Collectively, the information gathered through the Cure SMA annual community survey will help facilitate broad awareness and understanding of this complex disease

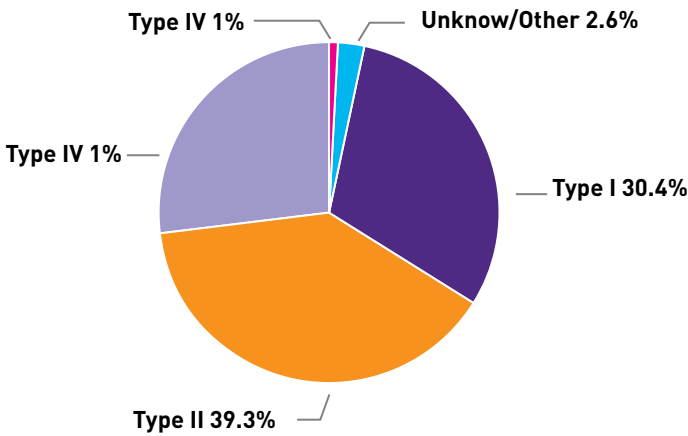
For more information on our surveys visit
cureSMA.org/communitysurvey



Recognizing the needs of the SMA community

Since 1996, Cure SMA has maintained a database of patient-reported data. Today, there are more than 7,000 patients recorded in the database, making it the largest SMA database in the world. This allows Cure SMA to have a wide understanding of the many people and families that are affected by the disease. While the existing member database contains information such as demographics, type of SMA, and diagnosis date, more data is needed to create a holistic picture of the needs and challenges of the SMA community. For this reason, in February 2017, Cure SMA initiated an annual community survey to collect new information, including data on clinical health care, family, and quality of life.

Surveys by SMA Type



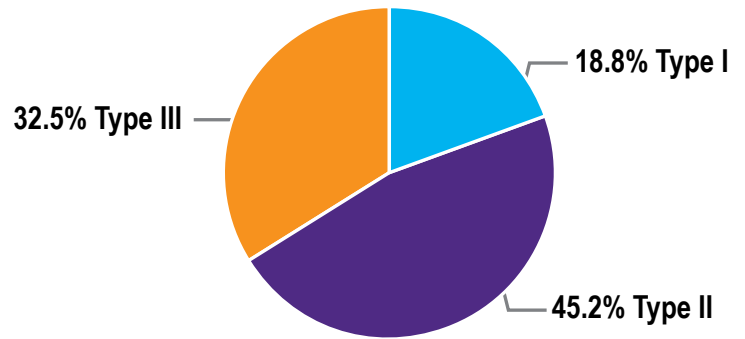
To advance these efforts, Cure SMA's annual community survey was developed and disseminated. Invitations to complete the survey were sent via email to the SMA community. Additionally, participation was advertised on Facebook to invite new participants to both join the Cure SMA database and to complete its annual survey. Participants included individuals with SMA over the age of 18 and caregivers of individuals with SMA. Surveys were de-identified to protect patient privacy. Ultimately, data was gathered to represent responses for nearly 700 individuals with SMA.

After analyzing the information collected, the 2017 Cure SMA community survey highlighted three key priorities of the SMA community, including:

Foster a greater understanding of SMA through research

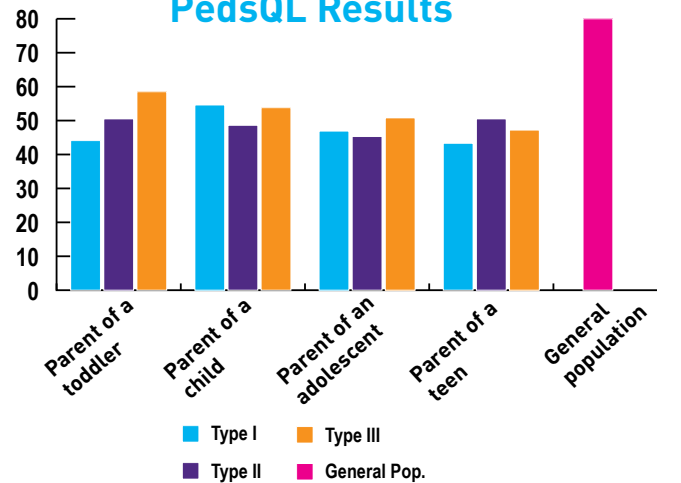
SMA can have a far-reaching and severe impact on patients and families that are affected by the disease. In order to overcome challenges that come with an SMA diagnosis and improve

Total PedsQL Surveys Completed



outcomes for patients, researchers must have a greater awareness and understanding of SMA. The Cure SMA community survey is the first study that aims to assess and understand the health-related quality of life of patients with SMA, as well as the family impact of the disease. This fact alone, that it is the first study intended to understand patients' health-related quality of life, demonstrates the need for greater research on and understanding of the patient impact of SMA.

PedsQL Results



This chart depicts the average quality of life scores in SMA-affected individuals, compared to the general population (in pink).

Through the community survey, Cure SMA was able to quantify patients' health-related quality of life based on physical, social, emotional, and cognitive functioning. Additionally, it quantified family functioning based on daily activities and familial relationships. The survey showed that parents of a child with SMA reported Generic Core Scale quality-of-life scores of 54.6, 48.6, and 53.8, for SMA type I, II, and III, respectively. Additionally, parents reported lower scores over time, demonstrating a decline in quality of life with age. For comparison, children without SMA have a reported quality-of-life score of 80.97 — much higher than those living with the disease. Without greater research on statistics such as these, researchers may remain unaware of the significant toll that SMA takes on patients and families' quality of life.

Research on quality of life and family impact can help advance our understanding of the SMA disease burden and highlight the necessity for patient and family advocacy, as well as support services. By raising awareness of SMA among the research community, we hope that researchers will be able to better identify gaps in care or unmet needs that can be studied further to improve outcomes for those affected by SMA. In turn, a wider understanding of the effects of SMA can help drive advocacy and improve public policy to create changes that benefit treatment and health coverage for patients.

Emphasize the urgency for early diagnosis

Using Cure SMA's community survey, experts have already identified a key area that can help improve outcomes for those living with SMA. The survey showed that the average age of diagnosis for patients with SMA type I is 4.9 months, which is particularly problematic, as evidence from previous research shows that the best treatment outcomes are for patients diagnosed before 3 months of age. This highlights the need for early diagnosis in order to create the best possible outcomes for patients with SMA type I.

It is critical that patients with SMA type I are able to receive early diagnoses, as the community survey found that the majority of deceased individuals represented had SMA type I. This statistic shows the immediate need to improve care for individuals with SMA type I, beginning with early diagnosis. We additionally learned that, in terms of family history, 10 percent of individuals with SMA had at least one sibling who is a known SMA carrier. Information such as this reveals complex truths about SMA, such as who could possibly be affected in the future and where innovation is most needed.

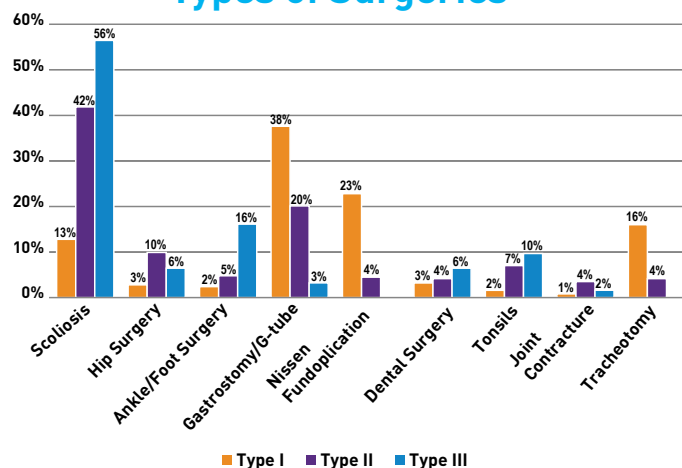
As researchers consider ways to improve outcomes for individuals living with SMA, statistics such as these can emphasize the urgency and necessity for innovative therapies and early diagnosis. This research can also help identify key areas on which health care professionals and researchers should focus their efforts to improve outcomes for those living with SMA. While some advances have been made, the complex nature of SMA types, diagnoses, and effects requires a greater understanding by researchers and health care professionals. We look forward to continuing to share insights and support public-private collaborations that emphasize the need for the early diagnosis of SMA in order to ultimately improve outcomes for those living with the disease.

Ensure favorable insurance coverage to limit financial burdens

It is also critical that individuals and families who are affected by SMA are able to work with insurers to secure favorable coverage as new and innovative therapies are approved. SMA requires an extensive continuum of care and the community survey revealed something we already knew to be true — care for individuals living with SMA is as complex as the disease itself. The survey found that:

- The most common types of specialists in SMA care teams are neurologists, pulmonologists, and physical therapists.
- Surgeries such as G-Tube placement (38 percent) and scoliosis surgery (42 percent and 55 percent) are commonly needed for individuals with SMA type I, II, and III, respectively.

Types of Surgeries



The financial burden of ongoing specialist care, treatments, and surgeries is growing, and can impact the quality of life of individuals and families affected by SMA. Now, many health insurers are charging high premiums and imposing large out-of-pocket costs on patients, which can exacerbate financial burdens even more for patients living with SMA, as well as for caregivers. In fact, this survey demonstrated that most families affected by SMA spend between \$5,000 and \$15,000 out-of-pocket each year, and many have to spend even more. As many have referred to large financial burdens as a “side effect” that can impact patient health, treatment adherence, and quality of life, favorable insurance coverage has become a top priority for the SMA community.

If you are struggling with expenses related to SMA care, Cure SMA has developed tools for patients and families to understand insurance coverage and manage health care costs. Our coverage roadmap, titled “Choice and Connection to Care: A Health Insurance Roadmap for People Living with Spinal Muscular Atrophy (SMA) and Their Caregivers,” can be found at www.cureSMA.org

Action and awareness for the SMA community

The information gathered in Cure SMA's annual community survey will help us understand the changes in the burden of illness, treatment patterns, and patient health as usage of FDA-approved drugs becomes more widespread. The data that patients and families affected by SMA shared with us will help inform future Cure SMA outreach activities and the planning and coordination of health care services. Cure SMA has used prior surveys and data collection projects to advance the needs of the SMA community and we hope that similarly effective uses of this survey will help us to continue to improve outcomes for patients and families.

Additionally, annual tracking of the SMA experience will help Cure SMA develop a working natural history of those affected by SMA. We will continue to build on our initial results in the hopes of demonstrating an evolving narrative and identifying changes in the SMA community over time as new, innovative therapies are approved. This work will help guide Cure SMA's efforts as we continue to advocate for the issues that matter most to our entire community. We will make certain that regulators, payers, and industry partners understand the impact of SMA and can create

solutions that will address our most important and most urgent priorities.

As an immediate next step to advance the priorities of the SMA community, Cure SMA will use these survey results to answer research questions and will disseminate them to the research community via papers and conference presentations.

We welcome your questions.

Lastly, we would like to extend our sincerest thanks to each individual and family that participated in the Cure SMA survey — a task we know can be both taxing and cathartic. The active involvement of our members is what supports our efforts and is what has allowed us to help advance the priorities of the SMA community. By participating in the Cure SMA annual community survey, you have become a champion for the SMA community and a leader in advancing our mission. We encourage you to continue talking, continue telling stories, and continue spreading awareness of SMA.

We look forward to hearing from you during our next annual community survey, which will be sent out in early 2018.

For more information on our surveys visit cureSMA.org/communitysurvey



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