

# Supplementary Materials

## The Cure SMA Clinical Trial Experience Survey: A Study of Trial Participant Perspectives on Clinical Trial Management and Patient-Centric Management Practices

Ilse S. Peterson<sup>1</sup>, Allison J. Mazzella<sup>2</sup>, Lisa T. Belter<sup>2</sup>, Mary A. Curry<sup>2</sup>, Rosángel E. Cruz<sup>2</sup>, Jill Jarecki<sup>2</sup>

1 Faegre Drinker Consulting, Washington, DC, United States

2 Cure SMA, Chicago, IL, United States

Corresponding Author: Mary Curry ([mary.curry@curesma.org](mailto:mary.curry@curesma.org))

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# Supplement 1

## Survey Tool

The Cure SMA Clinical Trial Experience Survey: A Study of Trial Participant Perspectives on Clinical Trial Management and Patient-Centric Management Practices

## Introduction

Thank you for your interest in the Cure SMA Clinical Trial Experience Survey! The purpose of this survey is to gather insights on patient experiences in SMA clinical trials. Cure SMA is interested in learning about key aspects of the trial process, including recruitment and enrollment, trial logistics, and engagement with research teams. With this survey, Cure SMA hopes to identify steps that trial sites and sponsors can take to support patient-centered trial management in the future. We appreciate your time and effort in helping us to gain this understanding.

### Before You Get Started

Before you get started, we would like to share some important information:

1. This survey should take about 15 minutes. Once you begin this survey, you can take up to 48 hours to complete it as long as you use the same computer or device to access the survey. If you have any trouble, please contact the researchers.

2. This survey is intended to reflect a single clinical trial experience for a person with SMA who participated in an SMA clinical trial to evaluate the safety and efficacy of an SMA therapeutic.

Individuals eligible to complete the survey include adults of majority age who:

- are themselves affected with SMA and have participated in an SMA clinical trial; or
- are parents or caregivers of a child with SMA who has participated in an SMA clinical trial; and
- can read and understand English to provide informed consent and complete the survey instrument.

If you or your child have participated in multiple clinical trials, or if you have multiple children who participated in clinical trials, you may submit separate surveys for each individual person and each clinical trial experience.

3. The survey respondent must be of the age of majority, which is 19 in Alabama and Nebraska, 21 in Puerto Rico, and 18 in all other states.

4. This survey is NOT designed to gather information about individual drugs. Rather, the purpose of this survey is to gather information on the clinical trial experience as a whole, and aspects of trial management that may affect your experience participating in a clinical trial. As such, please refrain from mentioning specific drugs in your response.

5. Natural history studies are out of scope for this survey.

1. If you would like to participate, please check the box below and click "Next".

☐ I am not a robot

## Cure SMA Consent Form for Research Participation

**Cure SMA is asking you to participate in a research study. This form has important information about the reason for doing this study, what we will ask you to do if you decide to be in this study, and the way we would like to use information about you if you choose to be in the study.**

### **Why are you doing this study?**

You are being asked to participate in a research study about your experience participating in an SMA clinical trial. Using a series of structured questions, the survey seeks to understand patient and family experiences throughout the trial process, including enrollment, coordination and communication with PI and research staff. The goals of the survey are to better understand the SMA community's experience with trials, and identify opportunities for sites and sponsors to improve the trial experience as drugs advance through the clinical pipeline.

### **What will I do if I choose to be in this study?**

You will be asked to complete a survey. Participation should take about 15-20 minutes total, and it is a one-time commitment. If you choose to participate, we may quote your remarks in presentations or articles resulting from this work. Your name and any identifying marks will be removed from your statements.

### **What are the possible risks or discomforts?**

Your participation in this study does not involve any physical or emotional risk to you/your child beyond that of everyday life. As with all research, there is a chance that confidentiality of the information we collect about you could be breached. We take steps to minimize this risk by collecting this data using a survey platform that utilizes secure protocols and encryption for data transfer and storage, and by saving the data in secure drives and a licensed and secure cloud-based data warehouse that is only accessible to approved employees of Cure SMA.

### **What are the possible benefits for me or others?**

You are not likely to have any direct benefit from being in this research study. This study is designed to learn more about the SMA trial experience. The study results may be used in the future to help other patients and families with SMA by improving clinical trial management.

### **How will you protect the information you collect about me, and how will that information be shared?**

Results of this study may be used in publications and presentations, and in the ways described under the question about Cure SMA's privacy policy for survey information below. Your study data will be handled as confidentially as possible. If results of this study are published or presented, individual names and other personally identifiable information will not be used. We may share the data we collect from you for use in future research studies or with other researchers. If we share the data that we collect about you, we will remove any information that could identify you before we share it. If we think that you intend to harm yourself or others, we will notify the appropriate people with this information.

**What is Cure SMA's privacy policy for information collected through this survey?**

Participation in any survey, focus group, or other patient voice project is entirely voluntary. You may complete all, some or none of questions asked in each exercise. Before deciding to participate, please take a moment to review this privacy notice. This explains how the information in the survey might be used, and the steps that we take to safeguard your information.

Information collected through this survey may be shared with Cure SMA grant recipients, content providers, sponsors, service providers or other third parties to advance the goals of research, education, dissemination of information and otherwise in fulfillment of the goals of Cure SMA. Cure SMA uses Personal Information to prepare aggregate disease statistics to provide to third parties, provide de-identified individual patient disease information to third parties, and contribute de-identified data to third party journal publications. Such third parties that receive Personal Information will be obligated to maintain the confidentiality of Personal Information and are not authorized to use such information for any purpose other than its intended purpose. Unless we have consent or as required by law, Cure SMA will not share Personal Information with any person or entity other than those affiliated with Cure SMA, entities acting on behalf of Cure SMA and relevant third parties such as Cure SMA research grant recipients or as may be required by law. By submitting Personal Information through this survey or to a Cure SMA contact, participants authorize Cure SMA to share this Personal Information for the purposes identified above. Participants also authorize Cure SMA to contact them directly with specific questions related to the information provided in this survey.

**Financial Information**

Participation in this study will involve no cost to you. As a token of appreciation for your time, you will receive a \$30 Amazon gift card for completing this survey.

**What are my rights as a research participant?**

Participation in this study is voluntary. You do not have to answer any question you do not want to answer. If at any time and for any reason, you would prefer not to participate in this study, please feel free not to. You may withdraw from this study at any time, and you will not be penalized in any way for deciding to stop participation.

**Who can I contact if I have questions or concerns about this research study?**

If you have questions, you may contact the researcher Allison Mazzella at [allison.mazzella@curesma.org](mailto:allison.mazzella@curesma.org) or you may reach me directly at: 847-264-1192.

If you have any questions about your rights as a participant in this research, you can contact the following office:

*Western Institutional Review Board*

*1019 39th Avenue SE Suite 120*

*Puyallup, WA 98374-2115*

1. I have read this consent form, or it has been read to me. I understand the information presented, have been given the opportunity to ask questions, and have had my questions answered. If I have additional questions, I have been told whom to contact. I agree to participate in the research study described above and the use of information collected through this survey as described in this form. I understand that I will receive a copy of this consent form.

Electronic Signature

Today's Date

2. In addition to providing your consent above, please electronically initial one of the following to indicate whether the researchers may use information collected in this study to contact you about participation in future research studies:

- ☐ Yes, I agree to allow the researchers to use information collected during this study to contact me about participating in future research studies.
- ☐ No, I do agree to allow the researchers to use information collected during this study to contact me about participating in future research studies.

## Information about Survey Respondent

### 1. Full Name of Survey Respondent

First

Last

### 2. Education Level of Survey Respondent

- ☐ Some high school
- ☐ High school graduate
- ☐ Some college
- ☐ Associates degree
- ☐ Bachelors degree
- ☐ Masters or higher degree

### 3. I am completing this survey on behalf of...

- ☐ Myself, as a clinical trial participant
- ☐ My child whom I enrolled in a clinical trial, or a child of whom I am the legal guardian

### 4. If you are a parent completing this on behalf of a child, what is the name of the child enrolled in the trial? (If no, please type NA.)

### 5. Are you completing this survey on behalf of a trial participant who is now deceased?

- ☐ Yes
- ☐ No

**We express our sincerest condolences for the loss of your loved one. We greatly appreciate you taking the time to participate in our survey. All questions to come are optional, please feel free to skip and respond to only questions that you feel comfortable answering. Thank you so much for participating in this survey and sharing your clinical trial experience.**



## Information about Trial Participant

**Questions in this section are about the trial participant (the person enrolled in the clinical trial), their SMA diagnosis, and their status at the time of enrollment in the clinical trial.**

1. What is the gender of the trial participant?

- ☐ Female
- ☐ Male
- ☐ Non-Binary

2. What is the date of birth of the trial participant?

Date / Time

MM/DD/YYYY

3. What is the ethnicity of the trial participant?

- ☐ White
- ☐ Black or African American
- ☐ Native American or American Indian
- ☐ Hispanic or Latino
- ☐ Asian or Pacific Islander
- ☐ Other

4. What was the trial participant's 5-digit zip code when first enrolled in the clinical trial?

5. When was the trial participant diagnosed with SMA?

- ☐ Prenatally
- ☐ Within the first month of life
- ☐ Between 1-6 months
- ☐ Between 7-12 months
- ☐ Between 1-5 years
- ☐ Between 6-17 years
- ☐ 18 or older

6. What is the trial participant's SMA Type?

- ☐ Type I
- ☐ Type II
- ☐ Type III
- ☐ Type IV
- ☐ Unknown

7. What is the trial participant's SMN2 Copy Number?

- ☐ 1
- ☐ 2
- ☐ 3
- ☐ 4 or more
- ☐ Unknown

8. Did the trial participant use any SMA-related supportive equipment **at time of enrollment in the clinical trial?** *Please select all that apply.*

- ☐ Oxygen
- ☐ BiPAP machine
- ☐ CPAP machine
- ☐ Cough machine
- ☐ Ventilator
- ☐ Tracheostomy with breathing machine
- ☐ None
- ☐ Other (please specify)

9. Had the trial participant had any SMA-related surgeries **at time of enrollment in the clinical trial**? *Please select all that apply.*

- ☐ Scoliosis
- ☐ Hip Surgery
- ☐ Ankle/Foot Surgery
- ☐ Eye Surgery
- ☐ Gastronomy
- ☐ Nissen Fundoplication (stomach wrap to prevent vomiting)
- ☐ Ileostomy
- ☐ Colostomy
- ☐ Dental Surgery
- ☐ Joint Contracture
- ☐ G-tubes
- ☐ Tracheostomy
- ☐ None
- ☐ Other (please specify)

10. What was the trial participant's maximum motor function **at time of enrollment in the clinical trial**?

- ☐ Could not sit, stand, or walk
- ☐ Sat with support
- ☐ Sat without support
- ☐ Stood with support
- ☐ Stood without support
- ☐ Walked with support
- ☐ Walked independently
- ☐ Do not remember

## Trial Information

**Questions from this point forward will focus on the clinical trial in which you or your child participated. If you are an adult who participated in a trial, please focus on your experience. If you are the parent or caregiver of a trial participant, please answer these questions to reflect the collective experience of you and your child.**

1. In what year did trial enrollment occur?

2. In what month did trial enrollment occur?

3. What site did you go to for this trial? *Please note that the list of sites is organized by state.*

4. Is the trial participant actively participating in this trial at this time?

- ☐ Yes
- ☐ No, the initial trial is complete but I have transitioned to a NEW open-label trial, or an extension trial
- ☐ No, the trial is complete and I (my child) participated through the conclusion of the trial
- ☐ No, the trial is ongoing, but my (my child's) participation was terminated
- ☐ No, the trial is ongoing but I (my child) withdrew
- ☐ No, the trial is complete but I (my child) withdrew before the trial concluded

1. When did withdrawal occur?

Date / Time

MM/DD/YYYY

2. What was the reason for withdrawal?

## Trial Experience: Finding and Enrolling in Clinical Trials

1. Prior to pursuing enrollment in this trial, how knowledgeable did you feel about the following?

|                                                        | Not<br>knowledgeable  | Slightly knowledgeable | Moderately knowledgeable | Very knowledgeable    | Extremely knowledgeable |
|--------------------------------------------------------|-----------------------|------------------------|--------------------------|-----------------------|-------------------------|
| SMA                                                    | <input type="radio"/> | <input type="radio"/>  | <input type="radio"/>    | <input type="radio"/> | <input type="radio"/>   |
| SMA clinical trials                                    | <input type="radio"/> | <input type="radio"/>  | <input type="radio"/>    | <input type="radio"/> | <input type="radio"/>   |
| What was involved in participating in a clinical trial | <input type="radio"/> | <input type="radio"/>  | <input type="radio"/>    | <input type="radio"/> | <input type="radio"/>   |

2. How did you find out about this clinical trial? *Please select all that apply.*

- ☐ Referral by pediatrician or general practitioner
- ☐ Referral by neurologist
- ☐ Referral by specialist other than neurologist
- ☐ Recruitment by researcher
- ☐ Clinicaltrials.gov
- ☐ Patient advocacy organization (e.g. Cure SMA, SMA Foundation, MDA)
- ☐ Social media
- ☐ Other (please specify)

3. Was the informed consent form for the trial shared with you prior to the screening visit, and did you have enough time to review it before that visit?

- ☐ Yes, it was shared and I had enough time
- ☐ Yes, it was shared but I did NOT have enough time
- ☐ No, it was not shared
- ☐ I do not remember

4. Before making a decision about participation, did you have sufficient opportunity to address questions with your study team?

- ☐ Yes
- ☐ No
- ☐ I do not remember

5. How significant were the following factors in your decision to pursue participation in the trial?

|                                                          | Not a significant factor | Moderately significant factor | Significant factor    |
|----------------------------------------------------------|--------------------------|-------------------------------|-----------------------|
| Access to investigational drug                           | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Access to medical experts                                | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Desire for clinical benefit and improved quality of life | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Opportunity to help others with SMA                      | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Opportunity to contribute to science                     | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Support from your doctor                                 | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |
| Positive interactions with the study team                | <input type="radio"/>    | <input type="radio"/>         | <input type="radio"/> |

Other (please specify factor and significance)

6. Did any of the following factors cause concern about participation?

|                                                                            | No concern            | Moderate concern      | Significant concern   |
|----------------------------------------------------------------------------|-----------------------|-----------------------|-----------------------|
| Time required for study                                                    | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Travel required for study                                                  | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Long-term commitment required                                              | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Complexity of the study logistics                                          | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Fear of physical and/or mental pain that could accompany required tests    | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Uncertainty about the safety of the study drug                             | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Uncertainty about whether potential benefits would justify potential risks | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Concern about loss of control                                              | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

Other (please specify factor and significance)

7. Would any of the factors below have **significantly** improved your experience with recruitment and enrollment? *Please select all that apply.*

- ☐ More discussion of trial options with my primary care provider
- ☐ More discussion of trial options with principal investigator(s)
- ☐ More written materials about the trial(s) being considered
- ☐ More written materials about the informed consent process
- ☐ More materials written in laymen's terms
- ☐ Receiving study information further ahead of my screening visit
- ☐ More opportunity to discuss the study and informed consent process specifically



## Trial Experience: Trial Logistics

1. In general, how well-organized were your study visits?

| Very poorly           | Poorly                | Neutral               | Well                  | Very well             |
|-----------------------|-----------------------|-----------------------|-----------------------|-----------------------|
| <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

2. Do you feel that your needs were considered when visits were scheduled?

- ☐ Yes, consistently
- ☐ Yes, most of the time
- ☐ Sometimes
- ☐ No, not really

3. Were any of the following **significant** issues for you when visits were scheduled? *Please select all that apply.*

- ☐ Visits scheduled last-minute, with little notice
- ☐ Visits scheduled at inconvenient time of day
- ☐ Time required for travel
- ☐ Conflicts with work and school
- ☐ Conflicts between eating and visit schedules
- ☐ Other (please specify)

#### 4. Were any of the following sources of stress during the trial?

|                                                                         | Not at all            | Moderate source       | Significant source    |
|-------------------------------------------------------------------------|-----------------------|-----------------------|-----------------------|
| Time required for study                                                 | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Travel required for study                                               | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Long-term commitment required                                           | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Complexity of the study logistics                                       | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Fear of physical and/or mental pain that could accompany required tests | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Concern about potential adverse events                                  | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Challenges managing medical complications related to SMA                | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Challenges balancing daily life with study requirements                 | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Feeling a loss of control                                               | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

#### 5. Did any of the following help to reduce your stress during the trial?

|                                                                          | Did not help          | Moderately helped     | Significantly helped  |
|--------------------------------------------------------------------------|-----------------------|-----------------------|-----------------------|
| Receiving thorough, timely updates                                       | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Responsiveness of your study team                                        | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Trust in your study team                                                 | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Understanding expressed by your study team                               | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Effective coordination between your study team and primary care provider | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Witnessing improvements in me/my child(ren) during the trial             | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

6. How did your overall stress level change throughout the trial?

- ☐ It went down significantly
- ☐ It went down somewhat
- ☐ It remained constant
- ☐ It went up somewhat
- ☐ It went up significantly

7. If your stress level changed, what was the main reason for this?

8. Would any of the following have **significantly** improved your experience during the trial? *Please select all that apply.*

- ☐ Efforts to accommodate your availability when scheduling research visits
- ☐ Having visit schedules well in advance of research visits
- ☐ Materials explaining trial instructions in layman's terms
- ☐ Reordering procedures to reduce fatigue
- ☐ More breaks to reduce fatigue
- ☐ Improving transitions between procedures
- ☐ Timely updates on trial results as they are released
- ☐ Timely, current information on adverse events
- ☐ Greater contingency planning
- ☐ Other (please specify)

## Trial Experience: Trial Communication

1. Which methods of communication did you and your primary contact use? *Please select all that apply.*

- ☐ Phone calls
- ☐ Emails
- ☐ Text messages
- ☐ Mobile app
- ☐ Web platform (e.g. MyChart)

2. What are your preferred methods of communication? *Please select all that apply.*

- ☐ Phone calls
- ☐ Emails
- ☐ Text messages
- ☐ Mobile app
- ☐ Web platform (e.g. MyChart)

3. How responsive, understanding, and trustworthy was your study coordinator? If you had multiple study coordinators, please indicate how responsive, understanding, and trustworthy the coordinators were on average.

|               | Not at all            | Slightly              | Moderately            | Very                  | Extremely             |
|---------------|-----------------------|-----------------------|-----------------------|-----------------------|-----------------------|
| Responsive    | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Trustworthy   | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Understanding | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

4. How responsive, understanding, and trustworthy was your principal investigator?

|               | Not at all            | Slightly              | Moderately            | Very                  | Extremely             |
|---------------|-----------------------|-----------------------|-----------------------|-----------------------|-----------------------|
| Responsive    | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Trustworthy   | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Understanding | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

5. Do you have any suggestions for how clinical trial sites and sponsors could strengthen communication and trust?

- ☐ Greater responsiveness to needs
- ☐ Greater effort to understand my experience as a person affected by SMA
- ☐ More frequent communication and check-ins
- ☐ Use of layman's terms instead of medical jargon
- ☐ More information regarding tests and the "why" behind testing
- ☐ Other (please specify)

## Overall Trial Experience

1. Overall, how did you feel about your experience with each of these aspects of the clinical trial?

|                                     | Very negative         | Negative              | Neutral               | Positive              | Very positive         |
|-------------------------------------|-----------------------|-----------------------|-----------------------|-----------------------|-----------------------|
| Finding information about the trial | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Screening and enrollment            | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Trial logistics                     | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Communication with your study team  | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

2. What benefits have come from participation in this trial, and how significant have they been?

|                                               | Not a benefit         | Moderate benefit      | Significant benefit   |
|-----------------------------------------------|-----------------------|-----------------------|-----------------------|
| Access to study drug                          | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Access to medical experts                     | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Clinical benefit and improved quality of life | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Opportunity to help others with SMA           | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Opportunity to contribute to science          | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Positive relationships with the study team    | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |
| Hope for a better future                      | <input type="radio"/> | <input type="radio"/> | <input type="radio"/> |

Other (please specify)

3. Is there else anything you would like us to know regarding this clinical trial experience?

## Thank You!

Thank you very much for your participation in this survey. We are grateful for the time and energy that you've dedicated to supporting our research, and look forward to using the insights that you've shared to develop recommendations to help sponsors and clinical research improve clinical trial experiences for future clinical trial participants.

1. Once this research has been completed, Cure SMA anticipates publishing this work in a peer-reviewed journal. If you would like to receive an email from Cure SMA when the results of this research are published, please opt-in by checking the box below.

☐ Yes, I would like to receive a notification when the results of this research have been published

### Connect with Cure SMA

Cure SMA leads the way to a world without spinal muscular atrophy. We fund and direct comprehensive research that drives breakthroughs in treatment and care, and we provide families the support they need for today. If you are not already connected with us, Cure SMA invites you to join our community. You can become involved by visiting us at [www.curesma.org](http://www.curesma.org), where you can learn more about the resources we offer to the SMA community as well as opportunities to engage.

### Questions

If you have any questions about this survey or need to contact us about your response in the future, please reach out to [allison.mazzella@curesma.org](mailto:allison.mazzella@curesma.org).

## Supplement 2

### Complete Survey Results for Group Included in Final Analysis

The Cure SMA Clinical Trial Experience Survey: A Study of Trial Participant Perspectives on Clinical Trial Management and Patient-Centric Management Practices

#### *Disclaimer:*

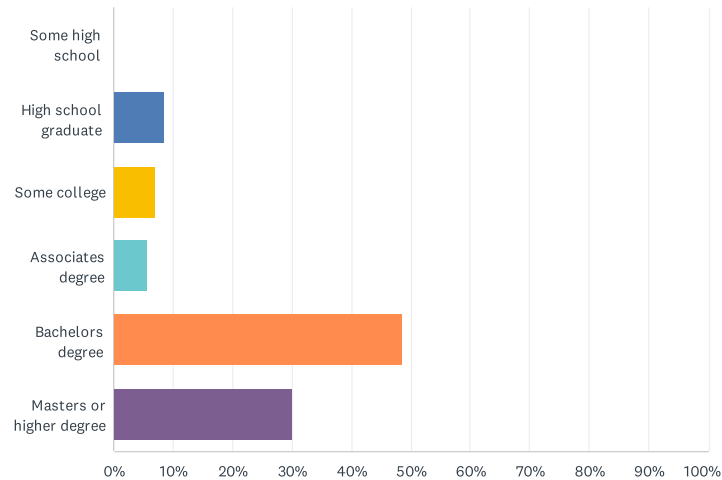
These results are the aggregate survey results for the surveys included in group analysis for this paper. The information presented here was exported Survey Monkey. Individual information (e.g., on dates of birth, enrollment, and withdrawal) and most free text responses have been removed from these results to protect the privacy of survey respondents.

There may be instances where the data presented here appears differently than what is in the final manuscript. For example, there are seven categories for motor function at the time of enrollment in these results, but motor function has been simplified into three categories in the table of survey respondent characteristics that is presented in the manuscript. Please contact the authors with any questions about these results.



Q5 Education Level of Survey Respondent

Answered: 70 Skipped: 0

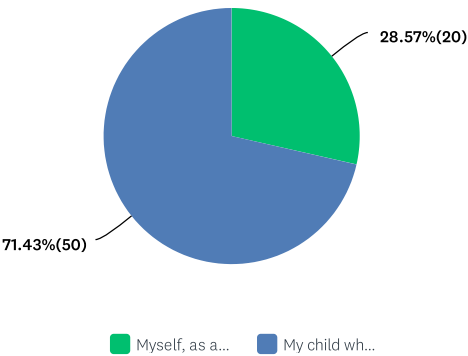


| ANSWER CHOICES               |  | RESPONSES |    |
|------------------------------|--|-----------|----|
| Some high school (1)         |  | 0.00%     | 0  |
| High school graduate (2)     |  | 8.57%     | 6  |
| Some college (3)             |  | 7.14%     | 5  |
| Associates degree (4)        |  | 5.71%     | 4  |
| Bachelors degree (5)         |  | 48.57%    | 34 |
| Masters or higher degree (6) |  | 30.00%    | 21 |
| TOTAL                        |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 2.00             | 6.00    | 5.00   | 4.84 | 1.18               |

Q6 I am completing this survey on behalf of...

Answered: 70 Skipped: 0

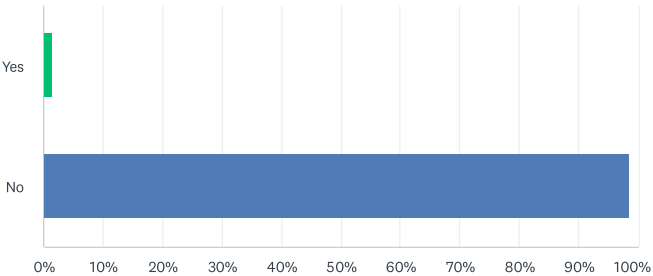


| ANSWER CHOICES                                                                               |  | RESPONSES |    |
|----------------------------------------------------------------------------------------------|--|-----------|----|
| Myself, as a clinical trial participant (1)                                                  |  | 28.57%    | 20 |
| My child whom I enrolled in a clinical trial, or a child of whom I am the legal guardian (2) |  | 71.43%    | 50 |
| TOTAL                                                                                        |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 2.00    | 2.00   | 1.71 | 0.45               |

Q8 Are you completing this survey on behalf of a trial participant who is now deceased?

Answered: 70 Skipped: 0

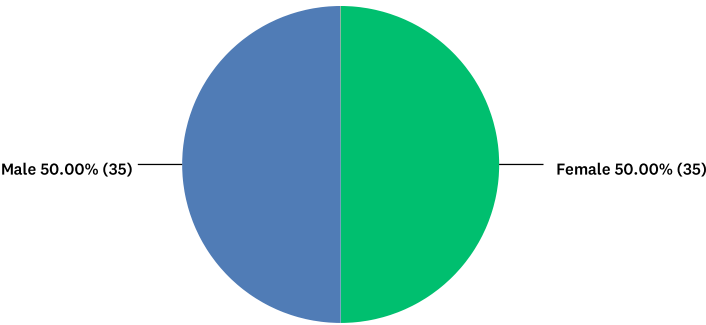


| ANSWER CHOICES |  | RESPONSES |    |
|----------------|--|-----------|----|
| Yes (1)        |  | 1.43%     | 1  |
| No (2)         |  | 98.57%    | 69 |
| TOTAL          |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 2.00    | 2.00   | 1.99 | 0.12               |

Q9 What is the gender of the trial participant?

Answered: 70 Skipped: 0

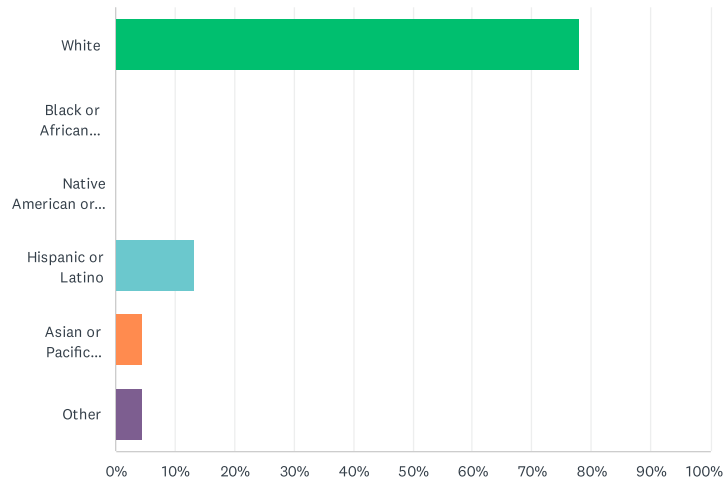


| ANSWER CHOICES |  | RESPONSES |    |
|----------------|--|-----------|----|
| Female (1)     |  | 50.00%    | 35 |
| Male (2)       |  | 50.00%    | 35 |
| Non-Binary (3) |  | 0.00%     | 0  |
| TOTAL          |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 2.00    | 1.50   | 1.50 | 0.50               |

## Q11 What is the ethnicity of the trial participant?

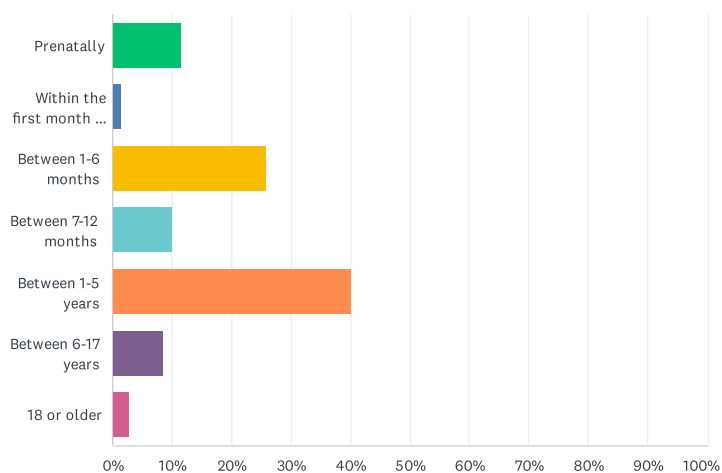
Answered: 68 Skipped: 2



| ANSWER CHOICES                         |         | RESPONSES |                    |
|----------------------------------------|---------|-----------|--------------------|
| White (1)                              |         | 77.94%    | 53                 |
| Black or African American (2)          |         | 0.00%     | 0                  |
| Native American or American Indian (3) |         | 0.00%     | 0                  |
| Hispanic or Latino (4)                 |         | 13.24%    | 9                  |
| Asian or Pacific Islander (5)          |         | 4.41%     | 3                  |
| Other (6)                              |         | 4.41%     | 3                  |
| TOTAL                                  |         |           | 68                 |
| BASIC STATISTICS                       |         |           |                    |
| Minimum                                | Maximum | Median    | Mean               |
| 1.00                                   | 6.00    | 1.00      | 1.79               |
|                                        |         |           | Standard Deviation |
|                                        |         |           | 1.54               |

## Q13 When was the trial participant diagnosed with SMA?

Answered: 70 Skipped: 0

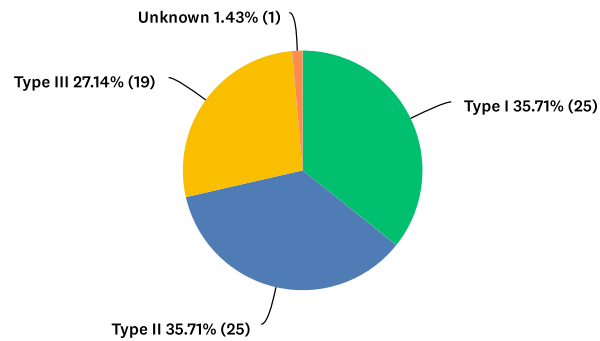


| ANSWER CHOICES                     |  | RESPONSES |    |
|------------------------------------|--|-----------|----|
| Prenatally (1)                     |  | 11.43%    | 8  |
| Within the first month of life (2) |  | 1.43%     | 1  |
| Between 1-6 months (3)             |  | 25.71%    | 18 |
| Between 7-12 months (4)            |  | 10.00%    | 7  |
| Between 1-5 years (5)              |  | 40.00%    | 28 |
| Between 6-17 years (6)             |  | 8.57%     | 6  |
| 18 or older (7)                    |  | 2.86%     | 2  |
| TOTAL                              |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 7.00    | 5.00   | 4.03 | 1.53               |

## Q14 What is the trial participant's SMA Type?

Answered: 70 Skipped: 0

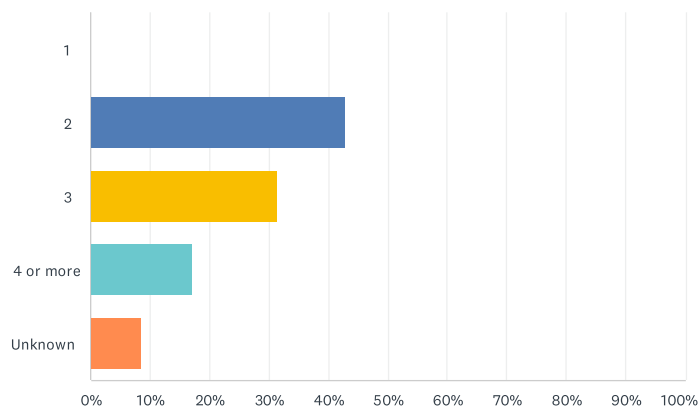


| ANSWER CHOICES |  | RESPONSES |    |
|----------------|--|-----------|----|
| Type I (1)     |  | 35.71%    | 25 |
| Type II (2)    |  | 35.71%    | 25 |
| Type III (3)   |  | 27.14%    | 19 |
| Type IV (4)    |  | 0.00%     | 0  |
| Unknown (5)    |  | 1.43%     | 1  |
| TOTAL          |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 5.00    | 2.00   | 1.96 | 0.87               |

## Q15 What is the trial participant's SMN2 Copy Number?

Answered: 70 Skipped: 0

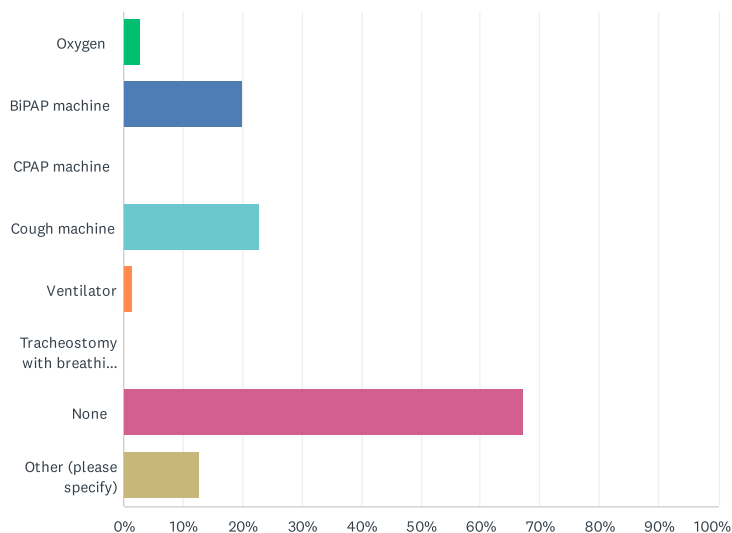


| ANSWER CHOICES   |                 |                | RESPONSES    |                            |
|------------------|-----------------|----------------|--------------|----------------------------|
| 1 (1)            |                 |                | 0.00%        | 0                          |
| 2 (2)            |                 |                | 42.86%       | 30                         |
| 3 (3)            |                 |                | 31.43%       | 22                         |
| 4 or more (4)    |                 |                | 17.14%       | 12                         |
| Unknown (5)      |                 |                | 8.57%        | 6                          |
| TOTAL            |                 |                |              | 70                         |
| BASIC STATISTICS |                 |                |              |                            |
| Minimum<br>2.00  | Maximum<br>5.00 | Median<br>3.00 | Mean<br>2.91 | Standard Deviation<br>0.97 |



Q16 Did the trial participant use any SMA-related supportive equipment at time of enrollment in the clinical trial? Please select all that apply.

Answered: 70 Skipped: 0

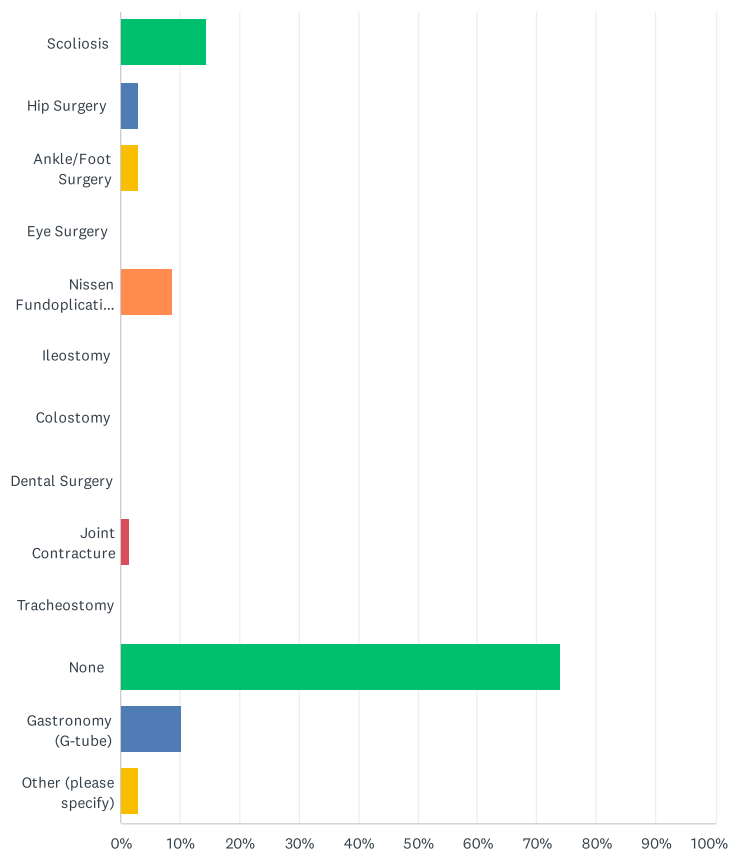


| ANSWER CHOICES                          | RESPONSES |    |
|-----------------------------------------|-----------|----|
| Oxygen (1)                              | 2.86%     | 2  |
| BiPAP machine (2)                       | 20.00%    | 14 |
| CPAP machine (3)                        | 0.00%     | 0  |
| Cough machine (4)                       | 22.86%    | 16 |
| Ventilator (5)                          | 1.43%     | 1  |
| Tracheostomy with breathing machine (6) | 0.00%     | 0  |
| None (7)                                | 67.14%    | 47 |
| Other (please specify) (8)              | 12.86%    | 9  |
| Total Respondents: 70                   |           |    |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 8.00    | 7.00   | 5.62 | 2.14               |

**Q17 Had the trial participant had any SMA-related surgeries at time of enrollment in the clinical trial? Please select all that apply.**

Answered: 69 Skipped: 1

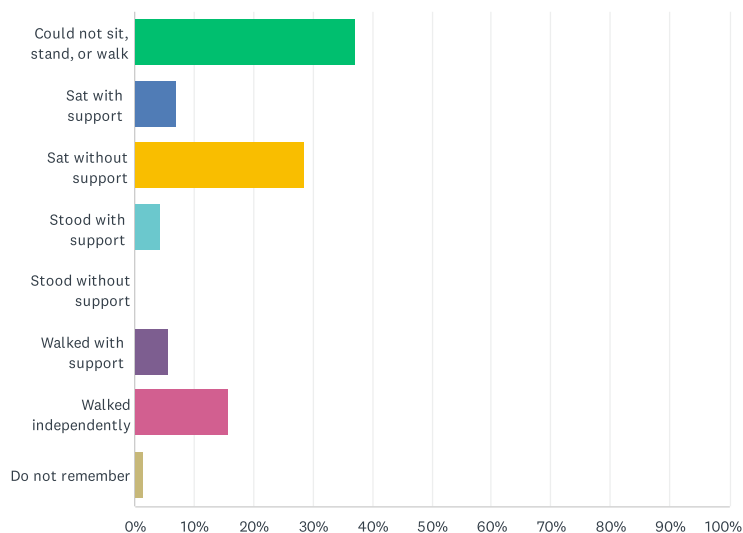


| ANSWER CHOICES                                           |  | RESPONSES |    |
|----------------------------------------------------------|--|-----------|----|
| Scoliosis                                                |  | 14.49%    | 10 |
| Hip Surgery                                              |  | 2.90%     | 2  |
| Ankle/Foot Surgery                                       |  | 2.90%     | 2  |
| Eye Surgery                                              |  | 0.00%     | 0  |
| Nissen Fundoplication (stomach wrap to prevent vomiting) |  | 8.70%     | 6  |
| Ileostomy                                                |  | 0.00%     | 0  |
| Colostomy                                                |  | 0.00%     | 0  |
| Dental Surgery                                           |  | 0.00%     | 0  |
| Joint Contracture                                        |  | 1.45%     | 1  |
| Tracheostomy                                             |  | 0.00%     | 0  |
| None                                                     |  | 73.91%    | 51 |
| Gastronomy (G-tube)                                      |  | 10.14%    | 7  |
| Other (please specify)                                   |  | 2.90%     | 2  |
| Total Respondents: 69                                    |  |           |    |

| BASIC STATISTICS |         |        |       |                    |
|------------------|---------|--------|-------|--------------------|
| Minimum          | Maximum | Median | Mean  | Standard Deviation |
| 1.00             | 14.00   | 13.00  | 10.07 | 4.58               |

### Q18 What was the trial participant's maximum motor function at time of enrollment in the clinical trial?

Answered: 70 Skipped: 0



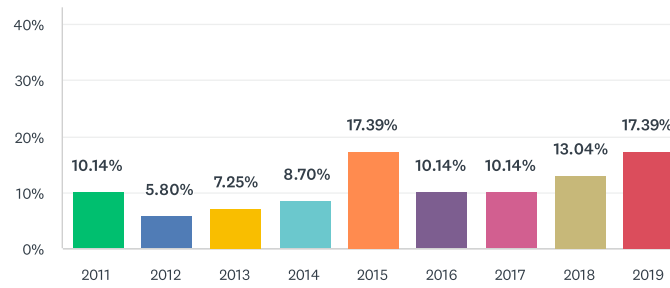
| ANSWER CHOICES                    |  | RESPONSES |    |
|-----------------------------------|--|-----------|----|
| Could not sit, stand, or walk (1) |  | 37.14%    | 26 |
| Sat with support (2)              |  | 7.14%     | 5  |
| Sat without support (3)           |  | 28.57%    | 20 |
| Stood with support (4)            |  | 4.29%     | 3  |
| Stood without support (5)         |  | 0.00%     | 0  |
| Walked with support (6)           |  | 5.71%     | 4  |
| Walked independently (7)          |  | 15.71%    | 11 |
| Do not remember (8)               |  | 1.43%     | 1  |
| TOTAL                             |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 8.00    | 3.00   | 3.10 | 2.23               |

# Cure SMA Clinical Trial Experience Survey

## Q19 In what year did trial enrollment occur?

Answered: 69 Skipped: 1

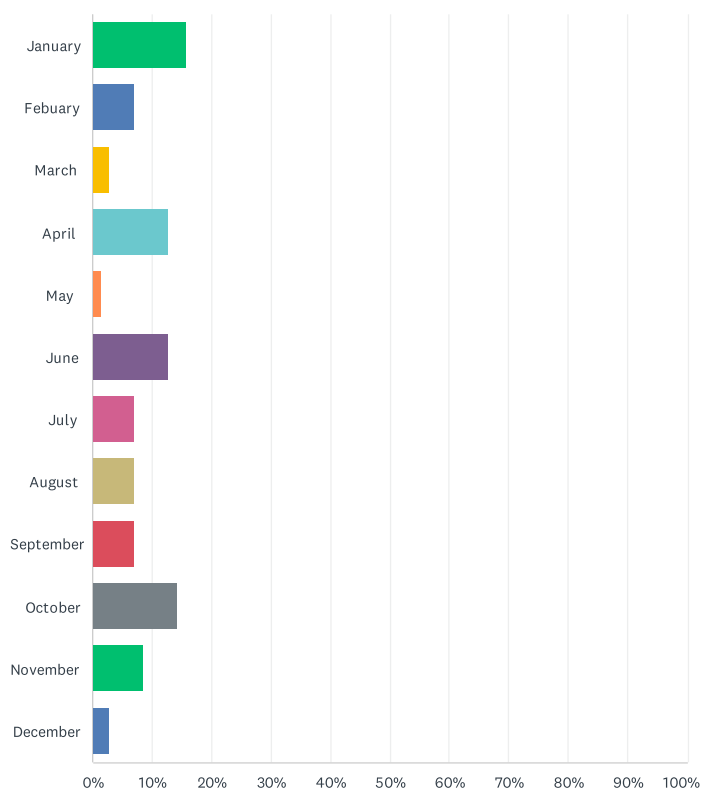


| ANSWER CHOICES | RESPONSES |    |
|----------------|-----------|----|
| 2011 (1)       | 10.14%    | 7  |
| 2012 (2)       | 5.80%     | 4  |
| 2013 (3)       | 7.25%     | 5  |
| 2014 (4)       | 8.70%     | 6  |
| 2015 (5)       | 17.39%    | 12 |
| 2016 (6)       | 10.14%    | 7  |
| 2017 (7)       | 10.14%    | 7  |
| 2018 (8)       | 13.04%    | 9  |
| 2019 (9)       | 17.39%    | 12 |
| TOTAL          |           | 69 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 9.00    | 6.00   | 5.58 | 2.58               |

## Q20 In what month did trial enrollment occur?

Answered: 70 Skipped: 0

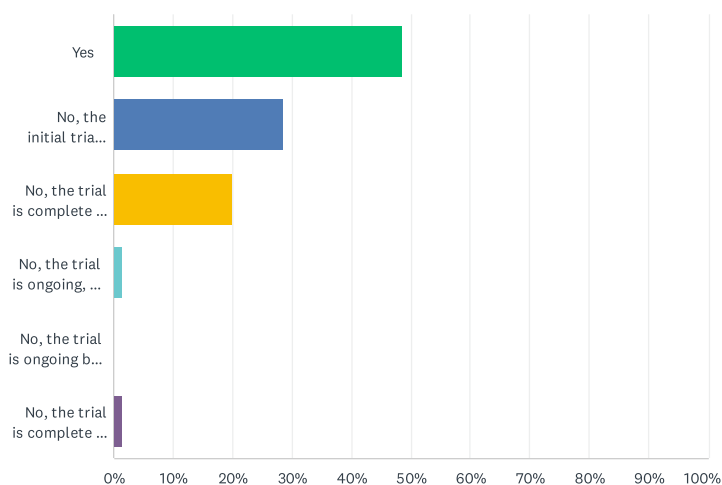


| ANSWER CHOICES |  | RESPONSES |    |
|----------------|--|-----------|----|
| January (1)    |  | 15.71%    | 11 |
| February (2)   |  | 7.14%     | 5  |
| March (3)      |  | 2.86%     | 2  |
| April (4)      |  | 12.86%    | 9  |
| May (5)        |  | 1.43%     | 1  |
| June (6)       |  | 12.86%    | 9  |
| July (7)       |  | 7.14%     | 5  |
| August (8)     |  | 7.14%     | 5  |
| September (9)  |  | 7.14%     | 5  |
| October (10)   |  | 14.29%    | 10 |
| November (11)  |  | 8.57%     | 6  |
| December (12)  |  | 2.86%     | 2  |
| TOTAL          |  |           | 70 |

| BASIC STATISTICS |                  |                |              |                            |
|------------------|------------------|----------------|--------------|----------------------------|
| Minimum<br>1.00  | Maximum<br>12.00 | Median<br>6.00 | Mean<br>6.17 | Standard Deviation<br>3.50 |

## Q22 Is the trial participant actively participating in this trial at this time?

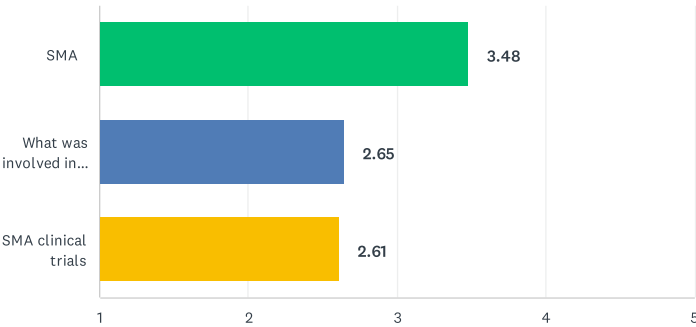
Answered: 70 Skipped: 0



| ANSWER CHOICES                                                                                                 |                 |                |              |                            | RESPONSES |    |
|----------------------------------------------------------------------------------------------------------------|-----------------|----------------|--------------|----------------------------|-----------|----|
| Yes (1)                                                                                                        |                 |                |              |                            | 48.57%    | 34 |
| No, the initial trial is complete but I have transitioned to a NEW open-label trial, or an extension trial (2) |                 |                |              |                            | 28.57%    | 20 |
| No, the trial is complete and I (my child) participated through the conclusion of the trial (3)                |                 |                |              |                            | 20.00%    | 14 |
| No, the trial is ongoing, but my (my child's) participation was terminated (4)                                 |                 |                |              |                            | 1.43%     | 1  |
| No, the trial is ongoing but I (my child) withdrew (5)                                                         |                 |                |              |                            | 0.00%     | 0  |
| No, the trial is complete but I (my child) withdrew before the trial concluded (6)                             |                 |                |              |                            | 1.43%     | 1  |
| TOTAL                                                                                                          |                 |                |              |                            |           | 70 |
| BASIC STATISTICS                                                                                               |                 |                |              |                            |           |    |
| Minimum<br>1.00                                                                                                | Maximum<br>6.00 | Median<br>2.00 | Mean<br>1.80 | Standard Deviation<br>0.97 |           |    |

Q25 Prior to pursuing enrollment in this trial, how knowledgeable did you feel about the following?

Answered: 70 Skipped: 0

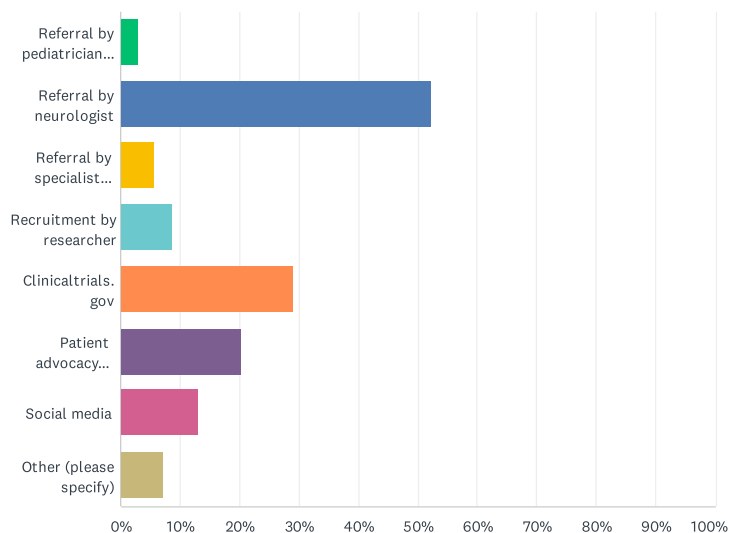


|                                                        | NOT KNOWLEDGEABLE (1) | SLIGHTLY KNOWLEDGEABLE (2) | MODERATELY KNOWLEDGEABLE (3) | VERY KNOWLEDGEABLE (4) | EXTREMELY KNOWLEDGEABLE (5) |
|--------------------------------------------------------|-----------------------|----------------------------|------------------------------|------------------------|-----------------------------|
| SMA                                                    | 11.59%<br>8           | 8.70%<br>6                 | 26.09%<br>18                 | 27.54%<br>19           |                             |
| What was involved in participating in a clinical trial | 26.09%<br>18          | 21.74%<br>15               | 24.64%<br>17                 | 15.94%<br>11           |                             |
| SMA clinical trials                                    | 28.57%<br>20          | 18.57%<br>13               | 31.43%<br>22                 | 5.71%<br>4             |                             |

| BASIC STATISTICS                                       |         |         |        |      |                    |
|--------------------------------------------------------|---------|---------|--------|------|--------------------|
|                                                        | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| SMA                                                    | 1.00    | 5.00    | 4.00   | 3.48 | 1.28               |
| SMA clinical trials                                    | 1.00    | 5.00    | 3.00   | 2.61 | 1.37               |
| What was involved in participating in a clinical trial | 1.00    | 5.00    | 3.00   | 2.65 | 1.33               |

## Q26 How did you find out about this clinical trial? Please select all that apply.

Answered: 69 Skipped: 1



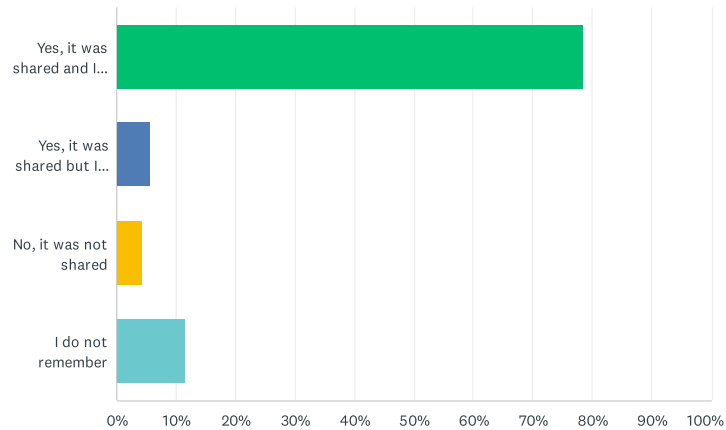
| ANSWER CHOICES                                                         |  | RESPONSES |    |
|------------------------------------------------------------------------|--|-----------|----|
| Referral by pediatrician or general practitioner (1)                   |  | 2.90%     | 2  |
| Referral by neurologist (2)                                            |  | 52.17%    | 36 |
| Referral by specialist other than neurologist (3)                      |  | 5.80%     | 4  |
| Recruitment by researcher (4)                                          |  | 8.70%     | 6  |
| Clinicaltrials.gov (5)                                                 |  | 28.99%    | 20 |
| Patient advocacy organization (e.g. Cure SMA, SMA Foundation, MDA) (6) |  | 20.29%    | 14 |
| Social media (7)                                                       |  | 13.04%    | 9  |
| Other (please specify) (8)                                             |  | 7.25%     | 5  |
| Total Respondents: 69                                                  |  |           |    |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 8.00    | 4.50   | 4.14 | 2.04               |



Q27 Was the informed consent form for the trial shared with you prior to the screening visit, and did you have enough time to review it before that visit?

Answered: 70 Skipped: 0

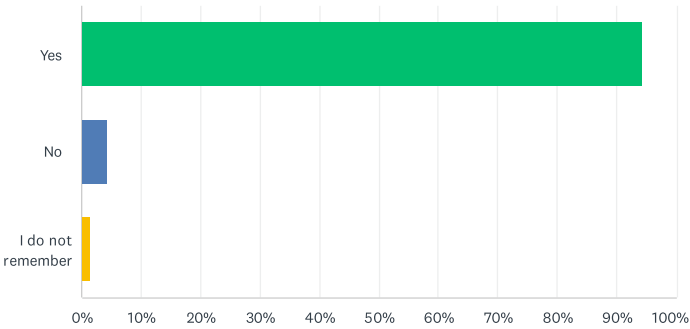


| ANSWER CHOICES                                        |  | RESPONSES |    |
|-------------------------------------------------------|--|-----------|----|
| Yes, it was shared and I had enough time (1)          |  | 78.57%    | 55 |
| Yes, it was shared but I did NOT have enough time (2) |  | 5.71%     | 4  |
| No, it was not shared (3)                             |  | 4.29%     | 3  |
| I do not remember (4)                                 |  | 11.43%    | 8  |
| TOTAL                                                 |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 4.00    | 1.00   | 1.49 | 1.01               |

Q28 Before making a decision about participation, did you have sufficient opportunity to address questions with your study team?

Answered: 70 Skipped: 0

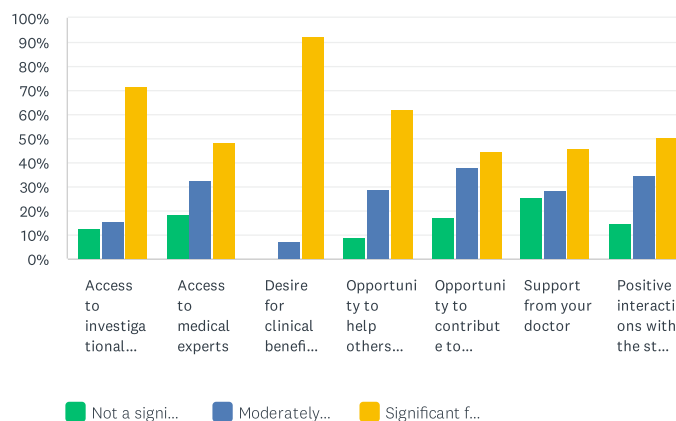


| ANSWER CHOICES        |  | RESPONSES |    |
|-----------------------|--|-----------|----|
| Yes (1)               |  | 94.29%    | 66 |
| No (2)                |  | 4.29%     | 3  |
| I do not remember (3) |  | 1.43%     | 1  |
| TOTAL                 |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 3.00    | 1.00   | 1.07 | 0.31               |

## Q29 How significant were the following factors in your decision to pursue participation in the trial?

Answered: 70 Skipped: 0

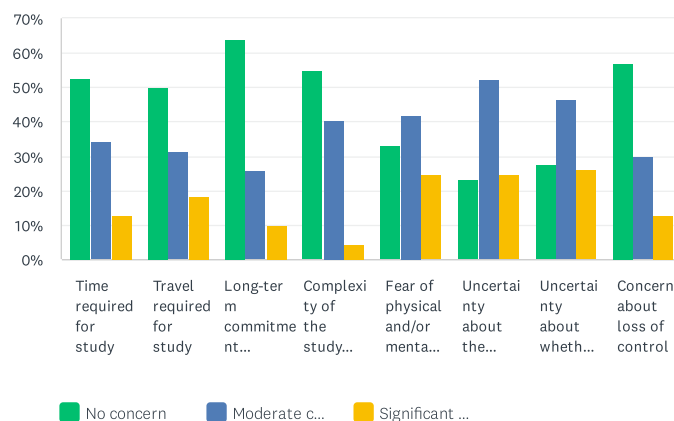


|                                                          | NOT A SIGNIFICANT FACTOR (1) | MODERATELY SIGNIFICANT FACTOR (2) | SIGNIFICANT FACTOR (3) | TOTAL | WEIGHTED AVERAGE |
|----------------------------------------------------------|------------------------------|-----------------------------------|------------------------|-------|------------------|
| Access to investigational drug                           | 12.86%<br>9                  | 15.71%<br>11                      | 71.43%<br>50           | 70    | 2.59             |
| Access to medical experts                                | 18.57%<br>13                 | 32.86%<br>23                      | 48.57%<br>34           | 70    | 2.30             |
| Desire for clinical benefit and improved quality of life | 0.00%<br>0                   | 7.25%<br>5                        | 92.75%<br>64           | 69    | 2.93             |
| Opportunity to help others with SMA                      | 8.70%<br>6                   | 28.99%<br>20                      | 62.32%<br>43           | 69    | 2.54             |
| Opportunity to contribute to science                     | 17.39%<br>12                 | 37.68%<br>26                      | 44.93%<br>31           | 69    | 2.28             |
| Support from your doctor                                 | 25.71%<br>18                 | 28.57%<br>20                      | 45.71%<br>32           | 70    | 2.20             |
| Positive interactions with the study team                | 14.49%<br>10                 | 34.78%<br>24                      | 50.72%<br>35           | 69    | 2.36             |

| BASIC STATISTICS                                         |         |         |        |      |                    |
|----------------------------------------------------------|---------|---------|--------|------|--------------------|
|                                                          | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| Access to investigational drug                           | 1.00    | 3.00    | 3.00   | 2.59 | 0.71               |
| Access to medical experts                                | 1.00    | 3.00    | 2.00   | 2.30 | 0.76               |
| Desire for clinical benefit and improved quality of life | 2.00    | 3.00    | 3.00   | 2.93 | 0.26               |
| Opportunity to help others with SMA                      | 1.00    | 3.00    | 3.00   | 2.54 | 0.65               |
| Opportunity to contribute to science                     | 1.00    | 3.00    | 2.00   | 2.28 | 0.74               |
| Support from your doctor                                 | 1.00    | 3.00    | 2.00   | 2.20 | 0.82               |
| Positive interactions with the study team                | 1.00    | 3.00    | 3.00   | 2.36 | 0.72               |

## Q30 Did any of the following factors cause concern about participation?

Answered: 70 Skipped: 0

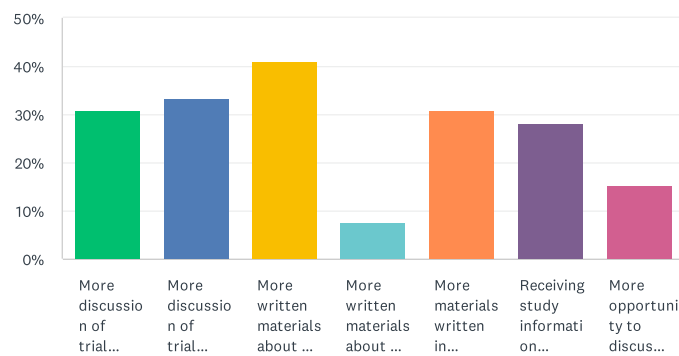


|                                                                            | NO CONCERN (1) | MODERATE CONCERN (2) | SIGNIFICANT CONCERN (3) | TOTAL | WEIGHTED AVERAGE |
|----------------------------------------------------------------------------|----------------|----------------------|-------------------------|-------|------------------|
| Time required for study                                                    | 52.86%<br>37   | 34.29%<br>24         | 12.86%<br>9             | 70    | 1.60             |
| Travel required for study                                                  | 50.00%<br>35   | 31.43%<br>22         | 18.57%<br>13            | 70    | 1.69             |
| Long-term commitment required                                              | 64.29%<br>45   | 25.71%<br>18         | 10.00%<br>7             | 70    | 1.46             |
| Complexity of the study logistics                                          | 55.07%<br>38   | 40.58%<br>28         | 4.35%<br>3              | 69    | 1.49             |
| Fear of physical and/or mental pain that could accompany required tests    | 33.33%<br>23   | 42.03%<br>29         | 24.64%<br>17            | 69    | 1.91             |
| Uncertainty about the safety of the study drug                             | 23.19%<br>16   | 52.17%<br>36         | 24.64%<br>17            | 69    | 2.01             |
| Uncertainty about whether potential benefits would justify potential risks | 27.54%<br>19   | 46.38%<br>32         | 26.09%<br>18            | 69    | 1.99             |
| Concern about loss of control                                              | 57.14%<br>40   | 30.00%<br>21         | 12.86%<br>9             | 70    | 1.56             |

| BASIC STATISTICS                                                           |         |         |        |      |                    |
|----------------------------------------------------------------------------|---------|---------|--------|------|--------------------|
|                                                                            | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| Time required for study                                                    | 1.00    | 3.00    | 1.00   | 1.60 | 0.71               |
| Travel required for study                                                  | 1.00    | 3.00    | 1.50   | 1.69 | 0.77               |
| Long-term commitment required                                              | 1.00    | 3.00    | 1.00   | 1.46 | 0.67               |
| Complexity of the study logistics                                          | 1.00    | 3.00    | 1.00   | 1.49 | 0.58               |
| Fear of physical and/or mental pain that could accompany required tests    | 1.00    | 3.00    | 2.00   | 1.91 | 0.76               |
| Uncertainty about the safety of the study drug                             | 1.00    | 3.00    | 2.00   | 2.01 | 0.69               |
| Uncertainty about whether potential benefits would justify potential risks | 1.00    | 3.00    | 2.00   | 1.99 | 0.73               |
| Concern about loss of control                                              | 1.00    | 3.00    | 1.00   | 1.56 | 0.71               |

Q31 Would any of the factors below have significantly improved your experience with recruitment and enrollment? Please select all that apply.

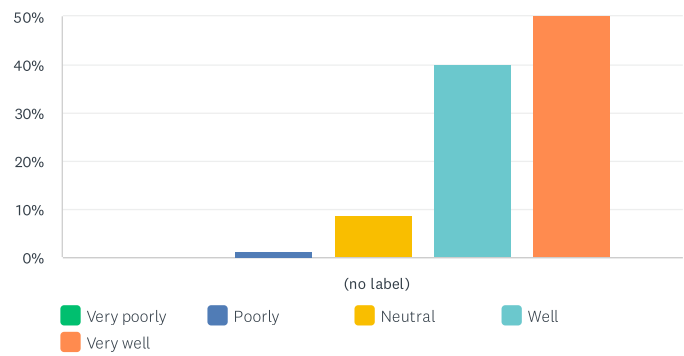
Answered: 39 Skipped: 31



| ANSWER CHOICES                                                                      |         |        |      |                    | RESPONSES |    |
|-------------------------------------------------------------------------------------|---------|--------|------|--------------------|-----------|----|
| More discussion of trial options with my primary care provider (1)                  |         |        |      |                    | 30.77%    | 12 |
| More discussion of trial options with principal investigator(s) (2)                 |         |        |      |                    | 33.33%    | 13 |
| More written materials about the trial(s) being considered (3)                      |         |        |      |                    | 41.03%    | 16 |
| More written materials about the informed consent process (4)                       |         |        |      |                    | 7.69%     | 3  |
| More materials written in laymen's terms (5)                                        |         |        |      |                    | 30.77%    | 12 |
| Receiving study information further ahead of my screening visit (6)                 |         |        |      |                    | 28.21%    | 11 |
| More opportunity to discuss the study and informed consent process specifically (7) |         |        |      |                    | 15.38%    | 6  |
| Total Respondents: 39                                                               |         |        |      |                    |           |    |
| BASIC STATISTICS                                                                    |         |        |      |                    |           |    |
| Minimum                                                                             | Maximum | Median | Mean | Standard Deviation |           |    |
| 1.00                                                                                | 7.00    | 3.00   | 3.64 | 1.95               |           |    |

Q32 In general, how well-organized were your study visits?

Answered: 70 Skipped: 0

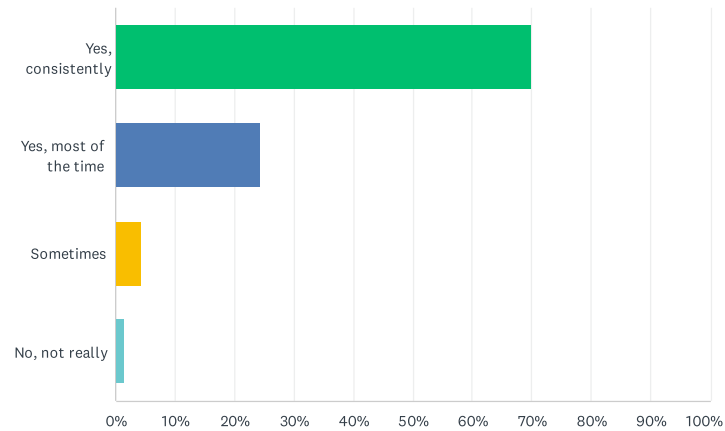


|            | VERY POORLY (1) | POORLY (2) | NEUTRAL (3) | WELL (4)     | VERY WELL (5) | TOTAL | WEIGHTED AVERAGE |
|------------|-----------------|------------|-------------|--------------|---------------|-------|------------------|
| (no label) | 0.00%<br>0      | 1.43%<br>1 | 8.57%<br>6  | 40.00%<br>28 | 50.00%<br>35  | 70    | 4.39             |

| BASIC STATISTICS |  |         |        |      |                    |
|------------------|--|---------|--------|------|--------------------|
| Minimum          |  | Maximum | Median | Mean | Standard Deviation |
| 2.00             |  | 5.00    | 4.50   | 4.39 | 0.70               |

Q33 Do you feel that your needs were considered when visits were scheduled?

Answered: 70 Skipped: 0

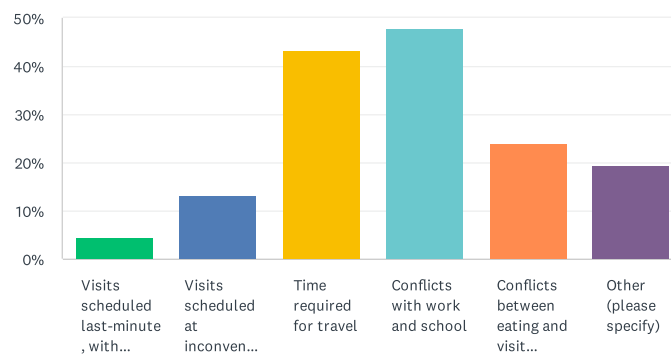


| ANSWER CHOICES            |  | RESPONSES |    |
|---------------------------|--|-----------|----|
| Yes, consistently (1)     |  | 70.00%    | 49 |
| Yes, most of the time (2) |  | 24.29%    | 17 |
| Sometimes (3)             |  | 4.29%     | 3  |
| No, not really (4)        |  | 1.43%     | 1  |
| TOTAL                     |  |           | 70 |

| BASIC STATISTICS |         |        |      |                    |
|------------------|---------|--------|------|--------------------|
| Minimum          | Maximum | Median | Mean | Standard Deviation |
| 1.00             | 4.00    | 1.00   | 1.37 | 0.64               |

Q34 Were any of the following significant issues for you when visits were scheduled? Please select all that apply.

Answered: 46 Skipped: 24

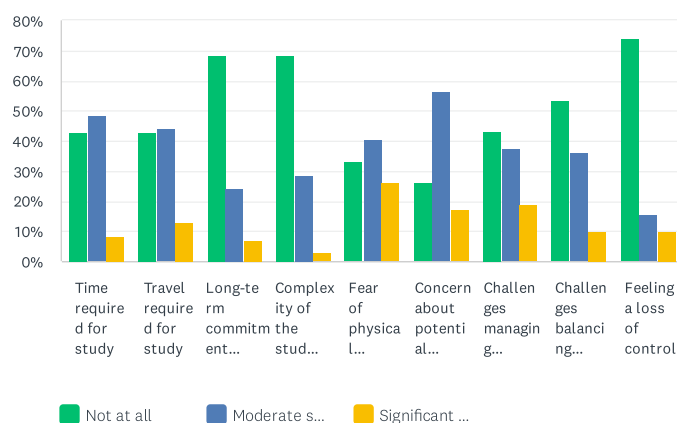


| ANSWER CHOICES                                       |                 |                |              | RESPONSES                  |    |
|------------------------------------------------------|-----------------|----------------|--------------|----------------------------|----|
| Visits scheduled last-minute, with little notice (1) |                 |                |              | 4.35%                      | 2  |
| Visits scheduled at inconvenient time of day (2)     |                 |                |              | 13.04%                     | 6  |
| Time required for travel (3)                         |                 |                |              | 43.48%                     | 20 |
| Conflicts with work and school (4)                   |                 |                |              | 47.83%                     | 22 |
| Conflicts between eating and visit schedules (5)     |                 |                |              | 23.91%                     | 11 |
| Other (please specify) (6)                           |                 |                |              | 19.57%                     | 9  |
| Total Respondents: 46                                |                 |                |              |                            |    |
| BASIC STATISTICS                                     |                 |                |              |                            |    |
| Minimum<br>1.00                                      | Maximum<br>6.00 | Median<br>4.00 | Mean<br>3.87 | Standard Deviation<br>1.24 |    |



## Q35 Were any of the following sources of stress during the trial?

Answered: 70 Skipped: 0

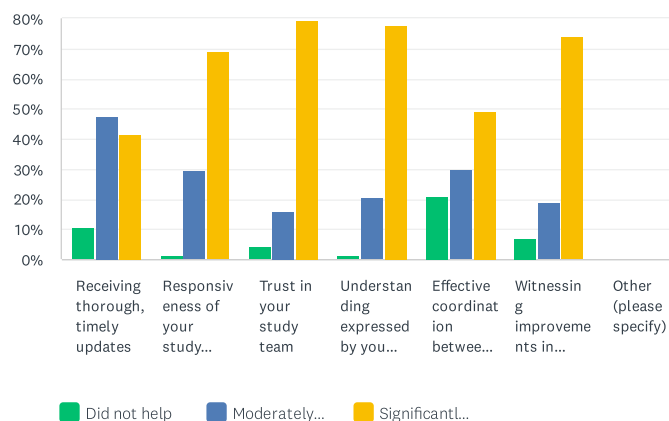


|                                                                         | NOT AT ALL (1) | MODERATE SOURCE (2) | SIGNIFICANT SOURCE (3) | TOTAL | WEIGHTED AVERAGE |
|-------------------------------------------------------------------------|----------------|---------------------|------------------------|-------|------------------|
| Time required for study                                                 | 42.86%<br>30   | 48.57%<br>34        | 8.57%<br>6             | 70    | 1.66             |
| Travel required for study                                               | 42.86%<br>30   | 44.29%<br>31        | 12.86%<br>9            | 70    | 1.70             |
| Long-term commitment required                                           | 68.57%<br>48   | 24.29%<br>17        | 7.14%<br>5             | 70    | 1.39             |
| Complexity of the study logistics                                       | 68.57%<br>48   | 28.57%<br>20        | 2.86%<br>2             | 70    | 1.34             |
| Fear of physical and/or mental pain that could accompany required tests | 33.33%<br>23   | 40.58%<br>28        | 26.09%<br>18           | 69    | 1.93             |
| Concern about potential adverse events                                  | 26.09%<br>18   | 56.52%<br>39        | 17.39%<br>12           | 69    | 1.91             |
| Challenges managing medical complications related to SMA                | 43.48%<br>30   | 37.68%<br>26        | 18.84%<br>13           | 69    | 1.75             |
| Challenges balancing daily life with study requirements                 | 53.62%<br>37   | 36.23%<br>25        | 10.14%<br>7            | 69    | 1.57             |
| Feeling a loss of control                                               | 74.29%<br>52   | 15.71%<br>11        | 10.00%<br>7            | 70    | 1.36             |

| BASIC STATISTICS                                                        |         |         |        |      |                    |
|-------------------------------------------------------------------------|---------|---------|--------|------|--------------------|
|                                                                         | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| Time required for study                                                 | 1.00    | 3.00    | 2.00   | 1.66 | 0.63               |
| Travel required for study                                               | 1.00    | 3.00    | 2.00   | 1.70 | 0.68               |
| Long-term commitment required                                           | 1.00    | 3.00    | 1.00   | 1.39 | 0.62               |
| Complexity of the study logistics                                       | 1.00    | 3.00    | 1.00   | 1.34 | 0.53               |
| Fear of physical and/or mental pain that could accompany required tests | 1.00    | 3.00    | 2.00   | 1.93 | 0.77               |
| Concern about potential adverse events                                  | 1.00    | 3.00    | 2.00   | 1.91 | 0.65               |
| Challenges managing medical complications related to SMA                | 1.00    | 3.00    | 2.00   | 1.75 | 0.75               |
| Challenges balancing daily life with study requirements                 | 1.00    | 3.00    | 1.00   | 1.57 | 0.67               |
| Feeling a loss of control                                               | 1.00    | 3.00    | 1.00   | 1.36 | 0.66               |

## Q36 Did any of the following help to reduce your stress during the trial?

Answered: 69 Skipped: 1

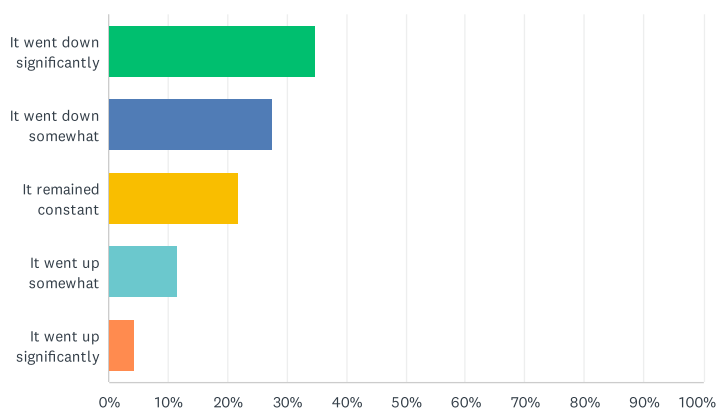


|                                                                          | DID NOT HELP (1) | MODERATELY HELPED (2) | SIGNIFICANTLY HELPED (3) | TOTAL | WEIGHTED AVERAGE |
|--------------------------------------------------------------------------|------------------|-----------------------|--------------------------|-------|------------------|
| Receiving thorough, timely updates                                       | 10.45%<br>7      | 47.76%<br>32          | 41.79%<br>28             | 67    | 2.31             |
| Responsiveness of your study team                                        | 1.47%<br>1       | 29.41%<br>20          | 69.12%<br>47             | 68    | 2.68             |
| Trust in your study team                                                 | 4.35%<br>3       | 15.94%<br>11          | 79.71%<br>55             | 69    | 2.75             |
| Understanding expressed by your study team                               | 1.47%<br>1       | 20.59%<br>14          | 77.94%<br>53             | 68    | 2.76             |
| Effective coordination between your study team and primary care provider | 20.90%<br>14     | 29.85%<br>20          | 49.25%<br>33             | 67    | 2.28             |
| Witnessing improvements in me/my child(ren) during the trial             | 7.25%<br>5       | 18.84%<br>13          | 73.91%<br>51             | 69    | 2.67             |
| Other (please specify)                                                   | 0.00%<br>0       | 0.00%<br>0            | 0.00%<br>0               | 0     | 0.00             |

| BASIC STATISTICS                                                         |         |         |        |      |                    |
|--------------------------------------------------------------------------|---------|---------|--------|------|--------------------|
|                                                                          | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| Receiving thorough, timely updates                                       | 1.00    | 3.00    | 2.00   | 2.31 | 0.65               |
| Responsiveness of your study team                                        | 1.00    | 3.00    | 3.00   | 2.68 | 0.50               |
| Trust in your study team                                                 | 1.00    | 3.00    | 3.00   | 2.75 | 0.52               |
| Understanding expressed by your study team                               | 1.00    | 3.00    | 3.00   | 2.76 | 0.46               |
| Effective coordination between your study team and primary care provider | 1.00    | 3.00    | 2.00   | 2.28 | 0.79               |
| Witnessing improvements in me/my child(ren) during the trial             | 1.00    | 3.00    | 3.00   | 2.67 | 0.61               |
| Other (please specify)                                                   | 0.00    | 0.00    | 0.00   | 0.00 | 0.00               |

## Q37 How did your overall stress level change throughout the trial?

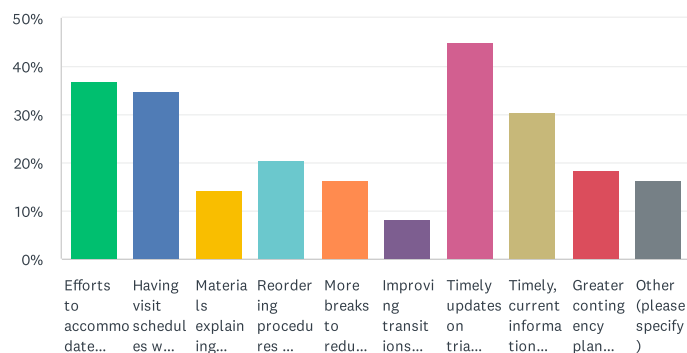
Answered: 69 Skipped: 1



| ANSWER CHOICES                 |                 | RESPONSES                  |              |
|--------------------------------|-----------------|----------------------------|--------------|
| It went down significantly (1) |                 | 34.78%                     | 24           |
| It went down somewhat (2)      |                 | 27.54%                     | 19           |
| It remained constant (3)       |                 | 21.74%                     | 15           |
| It went up somewhat (4)        |                 | 11.59%                     | 8            |
| It went up significantly (5)   |                 | 4.35%                      | 3            |
| TOTAL                          |                 |                            | 69           |
| BASIC STATISTICS               |                 |                            |              |
| Minimum<br>1.00                | Maximum<br>5.00 | Median<br>2.00             | Mean<br>2.23 |
|                                |                 | Standard Deviation<br>1.17 |              |

### Q39 Would any of the following have significantly improved your experience during the trial? Please select all that apply.

Answered: 49 Skipped: 21

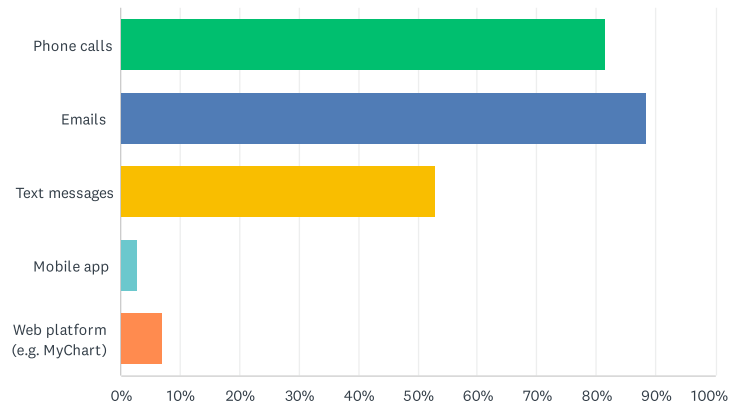


| ANSWER CHOICES                                                               | RESPONSES |    |
|------------------------------------------------------------------------------|-----------|----|
| Efforts to accommodate your availability when scheduling research visits (1) | 36.73%    | 18 |
| Having visit schedules well in advance of research visits (2)                | 34.69%    | 17 |
| Materials explaining trial instructions in layman's terms (3)                | 14.29%    | 7  |
| Reordering procedures to reduce fatigue (4)                                  | 20.41%    | 10 |
| More breaks to reduce fatigue (5)                                            | 16.33%    | 8  |
| Improving transitions between procedures (6)                                 | 8.16%     | 4  |
| Timely updates on trial results as they are released (7)                     | 44.90%    | 22 |
| Timely, current information on adverse events (8)                            | 30.61%    | 15 |
| Greater contingency planning (9)                                             | 18.37%    | 9  |
| Other (please specify) (10)                                                  | 16.33%    | 8  |
| Total Respondents: 49                                                        |           |    |

| BASIC STATISTICS |                  |                |              |                            |
|------------------|------------------|----------------|--------------|----------------------------|
| Minimum<br>1.00  | Maximum<br>10.00 | Median<br>5.00 | Mean<br>5.19 | Standard Deviation<br>2.98 |

**Q40 Which methods of communication did you and your primary contact use? Please select all that apply.**

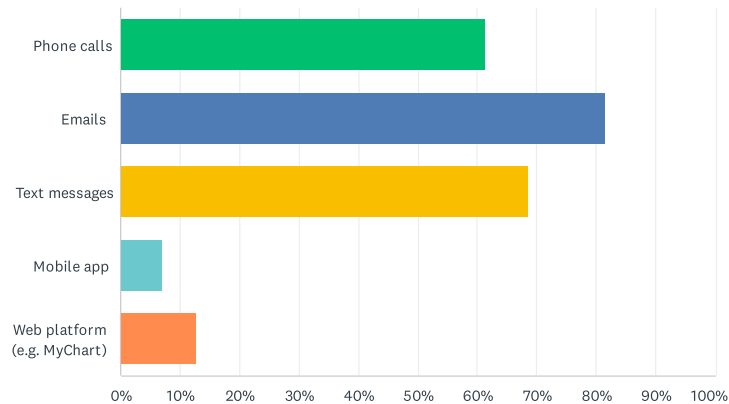
Answered: 70 Skipped: 0



| ANSWER CHOICES                  |                 |                |              | RESPONSES                  |    |
|---------------------------------|-----------------|----------------|--------------|----------------------------|----|
| Phone calls (1)                 |                 |                |              | 81.43%                     | 57 |
| Emails (2)                      |                 |                |              | 88.57%                     | 62 |
| Text messages (3)               |                 |                |              | 52.86%                     | 37 |
| Mobile app (4)                  |                 |                |              | 2.86%                      | 2  |
| Web platform (e.g. MyChart) (5) |                 |                |              | 7.14%                      | 5  |
| Total Respondents: 70           |                 |                |              |                            |    |
| BASIC STATISTICS                |                 |                |              |                            |    |
| Minimum<br>1.00                 | Maximum<br>5.00 | Median<br>2.00 | Mean<br>1.99 | Standard Deviation<br>0.95 |    |

**Q41 What are your preferred methods of communication? Please select all that apply.**

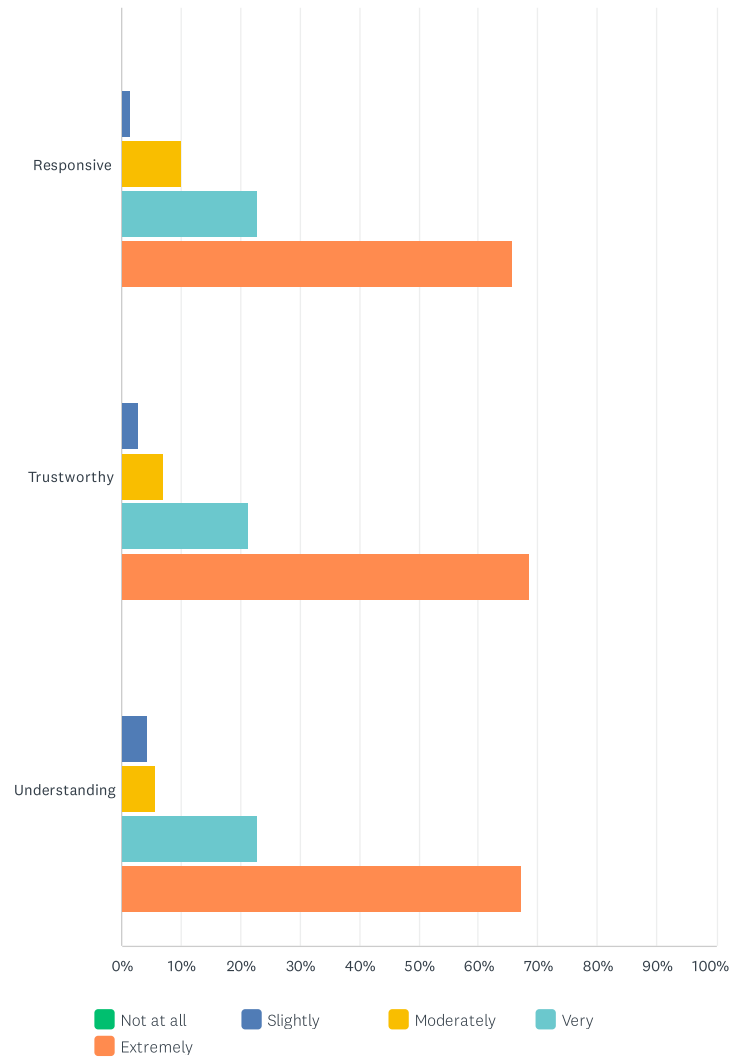
Answered: 70 Skipped: 0



| ANSWER CHOICES                  |                 |                |              | RESPONSES                  |    |
|---------------------------------|-----------------|----------------|--------------|----------------------------|----|
| Phone calls (1)                 |                 |                |              | 61.43%                     | 43 |
| Emails (2)                      |                 |                |              | 81.43%                     | 57 |
| Text messages (3)               |                 |                |              | 68.57%                     | 48 |
| Mobile app (4)                  |                 |                |              | 7.14%                      | 5  |
| Web platform (e.g. MyChart) (5) |                 |                |              | 12.86%                     | 9  |
| Total Respondents: 70           |                 |                |              |                            |    |
| BASIC STATISTICS                |                 |                |              |                            |    |
| Minimum<br>1.00                 | Maximum<br>5.00 | Median<br>2.00 | Mean<br>2.26 | Standard Deviation<br>1.06 |    |

Q42 How responsive, understanding, and trustworthy was your study coordinator? If you had multiple study coordinators, please indicate how responsive, understanding, and trustworthy the coordinators were on average.

Answered: 70 Skipped: 0

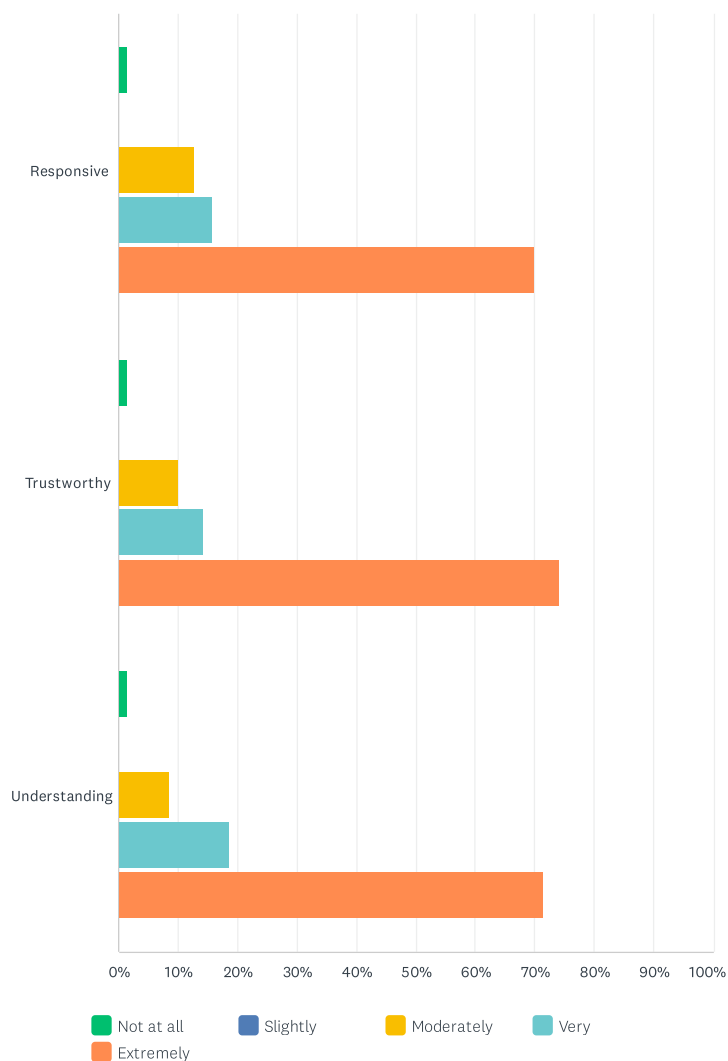


|               | NOT AT ALL<br>(1) | SLIGHTLY<br>(2) | MODERATELY<br>(3) | VERY<br>(4)  | EXTREMELY<br>(5) | TOTAL | WEIGHTED<br>AVERAGE |
|---------------|-------------------|-----------------|-------------------|--------------|------------------|-------|---------------------|
| Responsive    | 0.00%<br>0        | 1.43%<br>1      | 10.00%<br>7       | 22.86%<br>16 | 65.71%<br>46     | 70    | 4.53                |
| Trustworthy   | 0.00%<br>0        | 2.86%<br>2      | 7.14%<br>5        | 21.43%<br>15 | 68.57%<br>48     | 70    | 4.56                |
| Understanding | 0.00%<br>0        | 4.29%<br>3      | 5.71%<br>4        | 22.86%<br>16 | 67.14%<br>47     | 70    | 4.53                |

| BASIC STATISTICS |         |         |        |      |                    |  |
|------------------|---------|---------|--------|------|--------------------|--|
|                  | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |  |
| Responsive       | 2.00    | 5.00    | 5.00   | 4.53 | 0.73               |  |
| Trustworthy      | 2.00    | 5.00    | 5.00   | 4.56 | 0.75               |  |
| Understanding    | 2.00    | 5.00    | 5.00   | 4.53 | 0.79               |  |

### Q43 How responsive, understanding, and trustworthy was your principal investigator?

Answered: 70 Skipped: 0



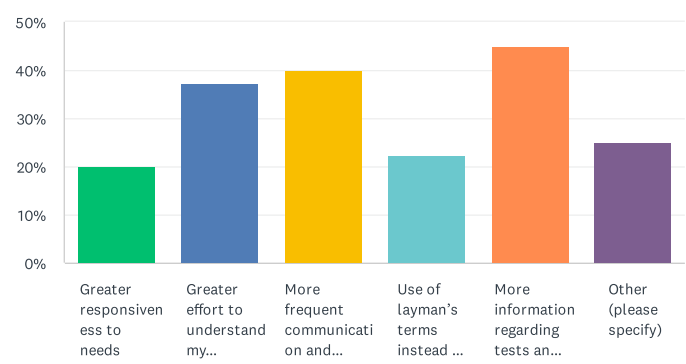
|               | NOT AT ALL<br>(1) | SLIGHTLY<br>(2) | MODERATELY<br>(3) | VERY<br>(4)  | EXTREMELY<br>(5) | TOTAL | WEIGHTED<br>AVERAGE |
|---------------|-------------------|-----------------|-------------------|--------------|------------------|-------|---------------------|
| Responsive    | 1.43%<br>1        | 0.00%<br>0      | 12.86%<br>9       | 15.71%<br>11 | 70.00%<br>49     | 70    | 4.53                |
| Trustworthy   | 1.43%<br>1        | 0.00%<br>0      | 10.00%<br>7       | 14.29%<br>10 | 74.29%<br>52     | 70    | 4.60                |
| Understanding | 1.43%<br>1        | 0.00%<br>0      | 8.57%<br>6        | 18.57%<br>13 | 71.43%<br>50     | 70    | 4.59                |

| BASIC STATISTICS |         |         |        |      |                    |  |
|------------------|---------|---------|--------|------|--------------------|--|
|                  | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |  |
| Responsive       | 1.00    | 5.00    | 5.00   | 4.53 | 0.82               |  |
| Trustworthy      | 1.00    | 5.00    | 5.00   | 4.60 | 0.78               |  |
| Understanding    | 1.00    | 5.00    | 5.00   | 4.59 | 0.77               |  |



Q44 Do you have any suggestions for how clinical trial sites and sponsors could strengthen communication and trust?

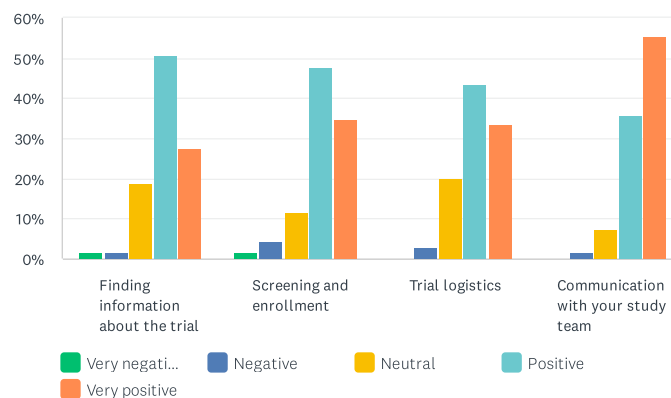
Answered: 40 Skipped: 30



| ANSWER CHOICES                                                             |         | RESPONSES          |      |
|----------------------------------------------------------------------------|---------|--------------------|------|
| Greater responsiveness to needs (1)                                        |         | 20.00%             | 8    |
| Greater effort to understand my experience as a person affected by SMA (2) |         | 37.50%             | 15   |
| More frequent communication and check-ins (3)                              |         | 40.00%             | 16   |
| Use of layman's terms instead of medical jargon (4)                        |         | 22.50%             | 9    |
| More information regarding tests and the "why" behind testing (5)          |         | 45.00%             | 18   |
| Other (please specify) (6)                                                 |         | 25.00%             | 10   |
| Total Respondents: 40                                                      |         |                    |      |
| BASIC STATISTICS                                                           |         |                    |      |
| Minimum                                                                    | Maximum | Median             | Mean |
| 1.00                                                                       | 6.00    | 3.00               | 3.58 |
|                                                                            |         | Standard Deviation |      |
|                                                                            |         | 1.59               |      |

### Q45 Overall, how did you feel about your experience with each of these aspects of the clinical trial?

Answered: 70 Skipped: 0

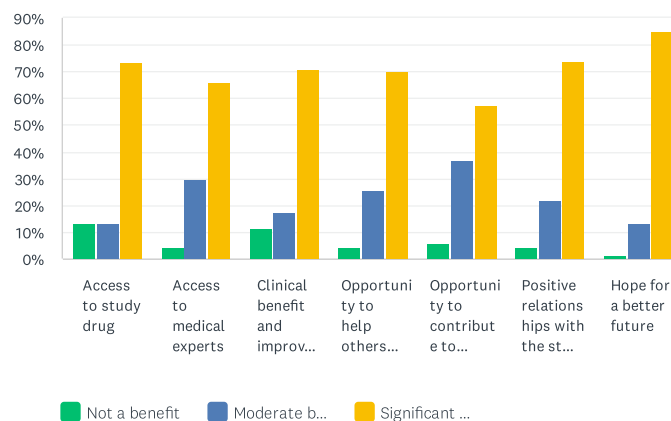


|                                     | VERY NEGATIVE (1) | NEGATIVE (2) | NEUTRAL (3)  | POSITIVE (4) | VERY POSITIVE (5) | TOTAL | WEIGHTED AVERAGE |
|-------------------------------------|-------------------|--------------|--------------|--------------|-------------------|-------|------------------|
| Finding information about the trial | 1.45%<br>1        | 1.45%<br>1   | 18.84%<br>13 | 50.72%<br>35 | 27.54%<br>19      | 69    | 4.01             |
| Screening and enrollment            | 1.45%<br>1        | 4.35%<br>3   | 11.59%<br>8  | 47.83%<br>33 | 34.78%<br>24      | 69    | 4.10             |
| Trial logistics                     | 0.00%<br>0        | 2.90%<br>2   | 20.29%<br>14 | 43.48%<br>30 | 33.33%<br>23      | 69    | 4.07             |
| Communication with your study team  | 0.00%<br>0        | 1.43%<br>1   | 7.14%<br>5   | 35.71%<br>25 | 55.71%<br>39      | 70    | 4.46             |

| BASIC STATISTICS                    |         |         |        |      |                    |  |
|-------------------------------------|---------|---------|--------|------|--------------------|--|
|                                     | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |  |
| Finding information about the trial | 1.00    | 5.00    | 4.00   | 4.01 | 0.81               |  |
| Screening and enrollment            | 1.00    | 5.00    | 4.00   | 4.10 | 0.87               |  |
| Trial logistics                     | 2.00    | 5.00    | 4.00   | 4.07 | 0.80               |  |
| Communication with your study team  | 2.00    | 5.00    | 5.00   | 4.46 | 0.69               |  |

## Q46 What benefits have come from participation in this trial, and how significant have they been?

Answered: 70 Skipped: 0



|                                               | NOT A BENEFIT (1) | MODERATE BENEFIT (2) | SIGNIFICANT BENEFIT (3) | TOTAL | WEIGHTED AVERAGE |
|-----------------------------------------------|-------------------|----------------------|-------------------------|-------|------------------|
| Access to study drug                          | 13.24%<br>9       | 13.24%<br>9          | 73.53%<br>50            | 68    | 2.60             |
| Access to medical experts                     | 4.29%<br>3        | 30.00%<br>21         | 65.71%<br>46            | 70    | 2.61             |
| Clinical benefit and improved quality of life | 11.59%<br>8       | 17.39%<br>12         | 71.01%<br>49            | 69    | 2.59             |
| Opportunity to help others with SMA           | 4.29%<br>3        | 25.71%<br>18         | 70.00%<br>49            | 70    | 2.66             |
| Opportunity to contribute to science          | 5.71%<br>4        | 37.14%<br>26         | 57.14%<br>40            | 70    | 2.51             |
| Positive relationships with the study team    | 4.35%<br>3        | 21.74%<br>15         | 73.91%<br>51            | 69    | 2.70             |
| Hope for a better future                      | 1.47%<br>1        | 13.24%<br>9          | 85.29%<br>58            | 68    | 2.84             |

| BASIC STATISTICS                              |         |         |        |      |                    |
|-----------------------------------------------|---------|---------|--------|------|--------------------|
|                                               | MINIMUM | MAXIMUM | MEDIAN | MEAN | STANDARD DEVIATION |
| Access to study drug                          | 1.00    | 3.00    | 3.00   | 2.60 | 0.71               |
| Access to medical experts                     | 1.00    | 3.00    | 3.00   | 2.61 | 0.57               |
| Clinical benefit and improved quality of life | 1.00    | 3.00    | 3.00   | 2.59 | 0.69               |
| Opportunity to help others with SMA           | 1.00    | 3.00    | 3.00   | 2.66 | 0.56               |
| Opportunity to contribute to science          | 1.00    | 3.00    | 3.00   | 2.51 | 0.60               |
| Positive relationships with the study team    | 1.00    | 3.00    | 3.00   | 2.70 | 0.55               |
| Hope for a better future                      | 1.00    | 3.00    | 3.00   | 2.84 | 0.41               |

## Supplement 3

### Representativeness of Study Sample

The Cure SMA Clinical Trial Experience Survey: A Study of Trial Participant Perspectives on Clinical Trial Management and Patient-Centric Management Practices

## Representativeness of the Study Sample

Recognizing the relatively small sample size of the survey population, multiple analyses were conducted to evaluate the representativeness of the study sample. Cure SMA's patient-reported database is the largest SMA database, encompassing contact information for over 9000 individuals with SMA (Belter et al, 2018). These analyses looked at the characteristics of the study population relative to the population included in Cure SMA's membership database, as well as how the selection of sites represented in the survey compared with the representation that would have been expected based on publicly available information about clinical trial site participation in trials.

### Characteristics of Survey Population Relative to Cure SMA's Membership Database

| Supplemental Table 3-1. Comparison of Sample to Clinical Trials Population in Cure SMA's Database using Chi Square Goodness of Fit tests |                                                   |                                                                |         |
|------------------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------|----------------------------------------------------------------|---------|
|                                                                                                                                          | Completed survey<br>(unique surveys only)<br>n=70 | Clinical trial participants<br>in Cure SMA's database<br>n=311 | p-value |
| <b>Sex, n (%)</b>                                                                                                                        |                                                   |                                                                |         |
| Male                                                                                                                                     | 35 (50)                                           | 144 (46.3)                                                     | 0.22    |
| <b>Race/Ethnicity, n(%)<sup>1</sup></b>                                                                                                  |                                                   |                                                                |         |
| White                                                                                                                                    | 53 (77.9)                                         | 192 (81.0)                                                     | 0.11    |
| Other                                                                                                                                    | 15 (22.1)                                         | 40 (16.9)                                                      |         |
| Unknown                                                                                                                                  | 0                                                 | 5 (2.1)                                                        |         |
| <b>Resides in US, n (%)</b>                                                                                                              | 69 (98.6)                                         | 285 (91.6)                                                     | <0.0001 |
| <b>Deceased, n (%)</b>                                                                                                                   | 1 (1.4)                                           | 37 (11.9)                                                      | <0.0001 |
| <b>SMA type, n (%)</b>                                                                                                                   |                                                   |                                                                |         |
| I                                                                                                                                        | 25 (35.7)                                         | 111 (35.7)                                                     | 0.26    |
| II                                                                                                                                       | 25 (35.7)                                         | 121 (38.9)                                                     |         |
| III                                                                                                                                      | 19 (27.1)                                         | 69 (22.2)                                                      |         |
| Unknown                                                                                                                                  | 1 (1.4)                                           | 6 (1.9)                                                        |         |
| Other**                                                                                                                                  | 0                                                 | 4 (1.3)                                                        |         |
| <b>SMN Copy Number, n (%)<sup>1</sup></b>                                                                                                |                                                   |                                                                |         |
| 1                                                                                                                                        | 0                                                 | 11 (3.5)                                                       | <0.0001 |
| 2                                                                                                                                        | 30 (42.9)                                         | 88 (28.3)                                                      |         |
| 3                                                                                                                                        | 22 (31.4)                                         | 90 (28.9)                                                      |         |
| 4 or more                                                                                                                                | 12 (17.1)                                         | 17 (5.5)                                                       |         |
| Unknown                                                                                                                                  | 6 (8.6)                                           | 31 (33.8)                                                      |         |
| <b>Mean (sd) age by SMA type at time of survey, years</b>                                                                                |                                                   |                                                                |         |
| I                                                                                                                                        | 4.0 (1.8)                                         | 5.4 (4.3)                                                      | n/a     |
| II                                                                                                                                       | 10.8 (8.1)                                        | 14.3 (10.4)                                                    |         |
| III                                                                                                                                      | 37.4 (18.6)                                       | 33.2 (19.5)                                                    |         |
| Unknown                                                                                                                                  | 3.7 (n/a)                                         | 15.5 (14.9)                                                    |         |
| Other**                                                                                                                                  | n/a                                               | 5 (5.7)                                                        |         |
| <b>Age at diagnosis, n (%)</b>                                                                                                           |                                                   |                                                                |         |
| Prenatally                                                                                                                               | 8 (11.4)                                          | 0                                                              | <0.0001 |
| Within the first month of life                                                                                                           | 1 (1.4)                                           | 8 (2.6)                                                        |         |
| Between 1-6 months                                                                                                                       | 18 (25.7)                                         | 92 (29.6)                                                      |         |
| Between 7-12 months                                                                                                                      | 7 (10.0)                                          | 71 (22.8)                                                      |         |
| Between 1-5 years                                                                                                                        | 28 (40.0)                                         | 130 (41.8)                                                     |         |
| Between 6-17 years                                                                                                                       | 6 (8.6)                                           | 7 (2.3)                                                        |         |
| 18 or older                                                                                                                              | 2 (2.9)                                           | 0                                                              |         |
| Unknown                                                                                                                                  | 0                                                 | 3 (1.0)                                                        |         |

<sup>1</sup> Data from 237 clinical trial participants in Cure SMA's database

\*\*Includes Distal SMA, SMARD, Kennedy's, SMA Type 0, SMA Type IV, SMA Type II-III, and SMA Type I-II.

| Supplemental Table 3-2. Comparison of Sample to Total Membership in Cure SMA's Database using Chi Square Goodness of Fit tests |                                                   |                                                                        |         |
|--------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------|------------------------------------------------------------------------|---------|
|                                                                                                                                | Completed survey<br>(unique surveys only)<br>n=70 | Total Cure SMA<br>membership database<br>1996–November 2019<br>n=8,234 | p-value |
| <b>Sex, n (%)</b>                                                                                                              |                                                   |                                                                        |         |
| Male                                                                                                                           | 35 (50)                                           | 3,948 (48.0)                                                           | 0.68    |
| <b>Race/Ethnicity, n(%)<sup>1</sup></b>                                                                                        |                                                   |                                                                        |         |
| White                                                                                                                          | 53 (77.9)                                         | 1,303 (75.4)                                                           | 0.51    |
| Other                                                                                                                          | 15 (22.1)                                         | 382 (22.1)                                                             |         |
| Unknown                                                                                                                        | 0                                                 | 42 (2.4)                                                               |         |
| <b>Resides in US, n (%)</b>                                                                                                    | 69 (98.6)                                         | 6,404 (77.8)                                                           | <0.0001 |
| <b>Deceased, n (%)</b>                                                                                                         | 1 (1.4)                                           | 2,117 (25.7)                                                           | <0.0001 |
| <b>SMA type, n (%)</b>                                                                                                         |                                                   |                                                                        |         |
| I                                                                                                                              | 25 (35.7)                                         | 3,216 (39.1)                                                           | <0.0001 |
| II                                                                                                                             | 25 (35.7)                                         | 2,569 (31.2)                                                           |         |
| III                                                                                                                            | 19 (27.1)                                         | 1,468 (17.8)                                                           |         |
| Unknown                                                                                                                        | 1 (1.4)                                           | 627 (7.6)                                                              |         |
| Other**                                                                                                                        | 0                                                 | 354 (4.3)                                                              |         |
| <b>SMN Copy Number, n (%)<sup>2</sup></b>                                                                                      |                                                   |                                                                        |         |
| 1                                                                                                                              | 0                                                 | 84 (4.7)                                                               | <0.0001 |
| 2                                                                                                                              | 30 (42.9)                                         | 562 (31.1)                                                             |         |
| 3                                                                                                                              | 22 (31.4)                                         | 615 (34.1)                                                             |         |
| 4 or more                                                                                                                      | 12 (17.1)                                         | 184 (10.2)                                                             |         |
| Unknown                                                                                                                        | 6 (8.6)                                           | 360 (19.9)                                                             |         |
| <b>Mean (sd) age by SMA type at time of survey, years</b>                                                                      |                                                   |                                                                        |         |
| I                                                                                                                              | 4.0 (1.8)                                         | 8.8 (8.0)                                                              | n/a     |
| II                                                                                                                             | 10.8 (8.1)                                        | 18.8 (13.3)                                                            |         |
| III                                                                                                                            | 37.4 (18.6)                                       | 29.3 (17.8)                                                            |         |
| Unknown                                                                                                                        | 3.7 (n/a)                                         | 20.2 (18.3)                                                            |         |
| Other**                                                                                                                        | n/a                                               | 46.4 (23.7)                                                            |         |
| <b>Age at diagnosis, n (%)</b>                                                                                                 |                                                   |                                                                        |         |
| Prenatally                                                                                                                     | 8 (11.4)                                          | 75 (0.9)                                                               | <0.0001 |
| Within the first month of life                                                                                                 | 1 (1.4)                                           | 173 (2.1)                                                              |         |
| Between 1-6 months                                                                                                             | 18 (25.7)                                         | 1,764 (21.4)                                                           |         |
| Between 7-12 months                                                                                                            | 7 (10.0)                                          | 965 (11.7)                                                             |         |
| Between 1-5 years                                                                                                              | 28 (40.0)                                         | 2,129 (25.9)                                                           |         |
| Between 6-17 years                                                                                                             | 6 (8.6)                                           | 438 (5.3)                                                              |         |
| 18 or older                                                                                                                    | 2 (2.9)                                           | 448 (5.4)                                                              |         |
| Unknown                                                                                                                        | 0                                                 | 2,242 (27.2)                                                           |         |
| <sup>1</sup> Data from Cure SMA membership database is from 1,727 individuals.                                                 |                                                   |                                                                        |         |
| <sup>2</sup> Data from Cure SMA membership database is from 1,805 individuals.                                                 |                                                   |                                                                        |         |
| **'Other' Type of SMA includes Distal SMA, SMARD, Kennedy's, SMA Type 0, SMA Type IV, SMA Type II-III, and SMA Type I-II.      |                                                   |                                                                        |         |

## Representation of Clinical Trial Sites

When developing the Cure SMA Clinical Trial Readiness Program, Peterson et al. (2020) created a model of clinical trial recruitment expected across trials and sites based upon information available via ClinicalTrials.org. This model was used to estimate expected proportions of trial participants that would have been recruited across clinical trial sites in the US during the approximate period of time survey respondents participated in clinical trials. Expected proportions were compared with the survey distribution to assess how well this sample represented the various sites that have been active in SMA clinical trials in the United States. For the 19 sites that were included in the Clinical Trial Recruitment model, only four sites were more than 5% over or under what would have been expected.

| Supplemental Table 3-3. Representation of Clinical Trial Sites in Survey Responses, Sorted by Absolute Difference |                       |                  |                                                             |            |                     |                |
|-------------------------------------------------------------------------------------------------------------------|-----------------------|------------------|-------------------------------------------------------------|------------|---------------------|----------------|
| Site                                                                                                              | Respondents from Site | % of Respondents | % of Expected Based on Trial Model                          | Difference | Absolute Difference | Representation |
| 1                                                                                                                 | 2                     | 2.9              | 2.4                                                         | 0.4        | 0.4                 | Over           |
| 2                                                                                                                 | 3                     | 4.3              | 3.9                                                         | 0.3        | 0.3                 | Over           |
| 3                                                                                                                 | 1                     | 1.4              | 1.5                                                         | -0.1       | 0.1                 | Under          |
| 4                                                                                                                 | 5                     | 7.1              | 6.1                                                         | 1.1        | 1.1                 | Over           |
| 5                                                                                                                 | 1                     | 1.4              | 0.6                                                         | 0.8        | 0.8                 | Over           |
| 6                                                                                                                 | 1                     | 1.4              | 0.6                                                         | 0.8        | 0.8                 | Over           |
| 7                                                                                                                 | 3                     | 4.3              | 4.5                                                         | -0.3       | 0.3                 | Under          |
| 8                                                                                                                 | 0                     | 0.0              | 0.9                                                         | -0.9       | 0.9                 | Under          |
| 9                                                                                                                 | 0                     | 0.0              | 1.2                                                         | -1.2       | 1.2                 | Under          |
| 10                                                                                                                | 0                     | 0.0              | 2.4                                                         | -2.4       | 2.4                 | Under          |
| 11                                                                                                                | 5                     | 7.1              | 8.8                                                         | -1.6       | 1.6                 | Under          |
| 12                                                                                                                | 7                     | 10.0             | 6.4                                                         | 3.6        | 3.6                 | Over           |
| 13                                                                                                                | 3                     | 4.3              | 6.4                                                         | -2.1       | 2.1                 | Under          |
| 14                                                                                                                | 2                     | 2.9              | 5.5                                                         | -2.6       | 2.6                 | Under          |
| 15                                                                                                                | 6                     | 8.6              | 4.5                                                         | 4.0        | 4.0                 | Over           |
| 16                                                                                                                | 3                     | 4.3              | 9.1                                                         | -4.8       | 4.8                 | Under          |
| 17                                                                                                                | 4                     | 5.7              | 12.4                                                        | -6.7       | 6.7                 | Under          |
| 18                                                                                                                | 15                    | 21.4             | 13.6                                                        | 7.8        | 7.8                 | Over           |
| 19                                                                                                                | 0                     | 0.0              | 9.1                                                         | -9.1       | 9.1                 | Under          |
| 20                                                                                                                | 4                     | 5.7              | *Not included in original clinical trial recruitment model. |            |                     |                |
| 21                                                                                                                | 1                     | 1.4              |                                                             |            |                     |                |
| 22                                                                                                                | 2                     | 2.9              |                                                             |            |                     |                |
| 23                                                                                                                | 1                     | 1.4              |                                                             |            |                     |                |
| 24                                                                                                                | 1                     | 1.4              |                                                             |            |                     |                |
| Total                                                                                                             | 70                    | 100              | 100                                                         |            |                     |                |