Additional File 2: 2023 Survey to Assess Clinical Trial Readiness and Capacity at SMA Clinical Trial Sites

INFORMATION ABOUT YOU AND YOUR SITE

1) Please provide the information below.*

Site Name: _____

Name of Respondent (First and Last Name):
_____

Role of Respondent within Organization: (drop down menu)
() Principal Investigator (PI)
() Physical Therapist (PT)
() Clinical Research Coordinator (CRC)
If your role is not listed above, please specify: ____
Site Location (City) : _____
Site Location (State) : (drop down menu, see below))
Your Email Address: _____
Phone Number: _____

Site location (State)

( ) Alabama ( ) Alaska

( ) Arizona
( ) Arkansas
( ) California
( ) Colorado
( ) Connecticut
( ) Delaware
( ) District of Columbia
( ) Florida
( ) Georgia
( ) Hawaii
( ) Idaho
( ) Illinois
( ) Indiana
( ) Iowa
( ) Kansas
( ) Kentucky
( ) Louisiana
( ) Maine
( ) Maryland
( ) Massachusetts
( ) Michigan
( ) Minnesota
( ) Mississippi
( ) Missouri
( ) Montana
( ) Nebraska
( ) Nevada
( ) New Hampshire
( ) New Jersey
( ) New Mexico
( ) New York
( ) North Carolina
( ) North Dakota
( ) Ohio
( ) Oklahoma
( ) Oregon
( ) Pennsylvania
( ) Rhode Island
( ) South Carolina
( ) South Dakota
( ) Tennessee
( ) Texas
( ) Utah
( ) Vermont
( ) Virginia
( ) Washington
( ) West Virginia
( ) Wisconsin
( ) Wyoming

GENERAL INFORMATION ABOUT YOUR SITE

This section asks for general information about the number, age, and source of referrals for SMA patients seen at your site.

2) How many SMA patients (all types) were seen at your site for care and or research (investigational and non-interventional) within the last year?

Seen for care only: _____

Seen for research only: _____

Seen for both care and research: _____

3) What ages of patients do you see? Please answer in years.

	Youngest Age	Oldest Age
Children for care		
Adults for care		
Children for research		
Adults for research		

4) What percentage of your SMA patients were referred to your site within the last year?

	0% - 25%	26% - 50%	51% - 75%	76% - 100%
For Care				
For Research				

5) In the last year, how important have the following factors been in driving referrals of SMA patients to your site for research (investigational and non-interventional) and care?

	Not important	Somewhat important	Important	Very important	Extremely important
Lack of specialty care at referring site	( )	( )	( )	( )	( )
SMA expertise at your site	( )	( )	( )	( )	( )
Capacity at referring site	( )	( )	( )	( )	( )
Clinical trial(s) recruiting at your site	( )	( )	( )	( )	( )
Social media	( )	( )	( )	( )	( )
Patient previous trial experience at your site	( )	( )	( )	( )	( )
Other (write in)	( )	( )	( )	( )	( )

STUDIES UNDERWAY AT YOUR SITE

This section asks for general information about the number of clinical trials for investigational drugs and other studies underway at your site.

6) **Do you have a dedicated clinical research unit or clinical trials office?**

( ) Yes

( ) No

7) **How many clinical trials for investigational drugs do you currently have underway, both overall, and in SMA specifically?**

# of neurology trials (total) _____

# of neuromuscular trials (total): _____

# of SMA trials: _____

8) **<u>For SMA pediatric (0 years to < 18 years) studies only,</u> how many, if any, of the following types of studies are currently being conducted at your site? If none, please enter zero (0).**

Phase I trials: _____

Phase II trials: _____

Phase III trials: _____

Open label extension trials: _____

Observational / Natural history studies:

_____

Investigator-initiated studies: _____

9) **<u>For SMA adult (18 years and older) studies only,</u> how many, if any, of the following types of studies are currently being conducted at your site? If none, please enter zero (0).**

Phase I trials: _____

Phase II trials: _____

Phase III trials: _____

Open label extension trials: _____

Observational / Natural history studies:

_____

Investigator-initiated studies: _____

INFORMATION ABOUT SITE STAFF

This next section asks for information about research and clinical staff at your site, and their experience with clinical trials and outcome measures in SMA.

Principal Investigator(s)

10) How many principal investigators (PIs) with experience conducting clinical trials for investigational drugs (<u>in any disease</u>) do you have on staff?

_____

11) Of the PIs you have on staff, how many have experience conducting clinical trials <u>specifically in SMA?</u>

_____

12) On average, how many years of experience do PIs at your site have conducting clinical trials, both in general and in SMA specifically?

years of trial experience overall: _____

years of experience with SMA trials:

_____

Clinical Research Coordinators/Study Coordinators

13) How many clinical research coordinators do you have on staff?

_____

14) Of the coordinators that you have on staff, how many have experience conducting clinical trials <u>specifically in SMA?</u>

_____

15) On average, how many years of experience do the clinical research coordinators at your site have conducting clinical trials, both in general and in SMA specifically?

years of trial experience overall: _____

years of SMA-specific trial experience:

_____

Physical Therapists

16) How many physical therapists with experience conducting SMA-specific outcome measures in a clinical setting do you have on staff?

_____

17) On average, how many years of experience do these physical therapists have conducting SMA-specific outcome measures <u>in SMA clinical trials?</u>

_____

18) How many of your physical therapists have had <u>reliability training</u> for conducting SMA-specific outcome measures for SMA clinical trials?

Received reliability training from sponsors: _____

of PTs received reliability training from institutions:

_____

Clinical Staff

19) Do you have professionals on staff who could support SMA Clinical Trials? (please check all that apply)

[ ] Neurologists

[ ] Nurse Practitioners (NPs)

[ ] Pulmonologists

[ ] GI specialist/Nutritionist/ Dieticians

[ ] Physical Therapists

[ ] Occupational Therapists (OTs)

[ ] Electrophysiologists

[ ] Genetic Counselors

[ ] Social Workers

[ ] Principal Investigators

[ ] Clinical Research Coordinators

FACTORS AFFECTING REFERRALS AND SITE CAPACITY FOR CLINICAL TRIALS

This final section asks for information about factors affecting patient referrals, your site's ability to successfully run trials, and your site's ability to take on additional trials.

20) Does your site have the capacity to conduct additional SMA trials at this time?

( ) Yes

( ) No

21) How important have the following factors been in enabling you to successfully run clinical trials in SMA?

	Not important	**Somewhat important**	**Important**	**Very important**	**Extremely important**
Staff availability/bandwidth in general	( )	( )	( )	( )	( )
Strong staff coordination in general	( )	( )	( )	( )	( )
High-performing clinical trials coordinator	( )	( )	( )	( )	( )
Staff expertise in SMA	( )	( )	( )	( )	( )
Ability to streamline patient visits (e.g. by coordinating appointments to	( )	( )	( )	( )	( )

occur in a single day)					
Robust site infrastructure (e.g. facilities well-suited to SMA patients/trials)	( )	( )	( )	( )	( )
Adequate funding available to conduct trial	( )	( )	( )	( )	( )
Adequate funding available for patient reimbursement	( )	( )	( )	( )	( )
External infrastructure (i.e.. hotels nearby for traveling families)	( )	( )	( )	( )	( )
Support from patient advocacy groups and SMA registries for recruitment	( )	( )	( )	( )	( )
Other (write in)	( )	( )	( )	( )	( )

22) Are any of the following <u>team and site factors</u> currently limiting your site's ability to take on additional SMA trials? Please indicate how significant of a barrier each factor is, if at all.

	Not significant	**Somewhat significant**	**Significant**	**Very significant**	**Extremely significant**
Bandwidth of clinical staff in general	( )	( )	( )	( )	( )

Bandwidth of clinical trial (research) staff specifically	( )	( )	( )	( )	( )
Bandwidth of clinical trials staff with SMA expertise	( )	( )	( )	( )	( )
Physical therapist bandwidth to conduct required training for new SMA trials	( )	( )	( )	( )	( )
Staff shortages	( )	( )	( )	( )	( )
Staff turnover	( )	( )	( )	( )	( )
No prior experience in SMA trials	( )	( )	( )	( )	( )
Limited Infrastructure (e.g. size or number of facilities)	( )	( )	( )	( )	( )
Other (write in)	( )	( )	( )	( )	( )

23) Are any of the following <u>patient-related factors</u> currently limiting your site's ability to take on additional SMA trials? Please indicate how significant of a barrier each factor is, if at all.

	Not significant	**Somewhat significant**	**Significant**	**Very significant**	**Extremely significant**
Not enough patients interested in new trials	( )	( )	( )	( )	( )
Patients interested in clinical trials but unable to participate due to distance from site	( )	( )	( )	( )	( )
Patients interested in clinical trials but unable to participate due to eligibility criteria (i.e. age, prior treatment exposure, etc)	( )	( )	( )	( )	( )
Patient saturation given existing number of patients being followed as part of open label extension trials	( )	( )	( )	( )	( )
Increase in number of clinical patients being	( )	( )	( )	( )	( )

followed as new treatment options become available					
Patient preference for approved drugs over investigational drugs	( )	( )	( )	( )	( )
Patient need for increased funding for participation	( )	( )	( )	( )	( )
Other (write in)	( )	( )	( )	( )	( )

24) In the past year, did any of the following present significant barriers to study start-up?

	Not all	**Somewhat**	**A great deal**
Contract negotiations	( )	( )	( )
Regulatory/IRB submissions and requirements	( )	( )	( )
Use of local IRBs	( )	( )	( )
Protocol Amendments	( )	( )	( )
Administrative errors	( )	( )	( )
Budget constraints	( )	( )	( )
Inexperienced staff	( )	( )	( )
Inexperienced clinical trials coordinator	( )	( )	( )
Staff turnover	( )	( )	( )
Staff bandwidth	( )	( )	( )
Amount of required training for PTs	( )	( )	( )
Lack of clinical supplies	( )	( )	( )
Lack of communication with study sponsor	( )	( )	( )

Other (write in)	( )	( )	( )	( ) (

25) How helpful would the following be in increasing your site's ability to take on additional SMA trials?

	Not helpful	**Somewhat helpful**	**Helpful**	**Very helpful**	**Extremely helpful**
Increasing the number of clinical staff in general	( )	( )	( )	( )	( )
Increasing the number of clinical trial staff, specifically	( )	( )	( )	( )	( )
Having more clinical research coordinators	( )	( )	( )	( )	( )
Having more PIs	( )	( )	( )	( )	( )
Having more PTs	( )	( )	( )	( )	( )
PT training in SMA outcome measures	( )	( )	( )	( )	( )
Streamlined PT training for trials	( )	( )	( )	( )	( )

Increased interest from patients in clinical trials	( )	( )	( )	( )	( )
Increased awareness about your site as a SMA clinical trial site	( )	( )	( )	( )	( )
Other (write in)	( )	( )	( )	( )	( )

26) Have you reviewed or utilized our Cure SMA Trial Readiness Program resources (e.g. toolkits)?

( ) Yes

( ) No

27) What are your suggestions on additional topics that Cure SMA could add to its existing resources? (show if Yes to 26)

_____

_____

28) What other resources could Cure SMA develop to support trial sites as they prepare for or conduct clinical trials in SMA?

_____

_____

29) Is there anything Cure SMA might be able to do to help with the management of existing patients at your site?

_____

_____

30) Is there anything Cure SMA might be able to do to help your site take on new clinical trials?

_____

_____

FOLLOW-UP

May we reach out to you with follow-up questions (e.g., to request clarifying information related to responses above)?

( ) Yes

( ) No

Thank You!

Thank you for taking our survey. In appreciation for your time, Cure SMA is offering a $40 gift card to all eligible individuals from clinical trial sites who have completed this survey. Payments issued in connection with your participation in this survey will be reported as transfer of value from Biogen, Scholar Rock, Novartis Gene Therapies, Biohaven Pharmaceuticals, Epirium Bio, Genentech/Roche Pharmaceuticals, the current members of the Cure SMA Industry Collaboration, which funded this research.

Please check the appropriate box below to let us know if you're interested in receiving a gift card.

[ ] I do not have any restrictions that prevents me from accepting gifts and I would like to receive the $40 Amazon gift card incentive from Cure SMA for completing this survey.

[ ] I would NOT like to receive the $40 Amazon gift card incentive from Cure SMA for completing this survey.

Please also provide the preferred email address for receipt of your gift card:

_____

You have reached the end of the survey.

After you click "Submit survey", your responses are locked, finalized and submitted.