**Supplementary Tables**

Supplemental Table 1. Attrition tables for 2018 survey

|  |  |  |  |
| --- | --- | --- | --- |
|  | **Total Membership database 1996–March 2018** | **Sent survey invitation\*** | **Completed survey (for unique surveys only)** |
| **Number** | 7,211 | 3,542 | 760 |
| **Sex, *n* (%)**MaleFemaleUnknown | 3,480 (48.3)3,351 (46.5)380 (5.3) | 1,716 (48.5)1,719 (48.5)107 (3.0) | 345 (45.4)386 (50.8)29 (3.8) |
| **Deceased, *n* (%)** | 1,860 (25.8) | 841 (23.7) | 141 (18.6) |
| **Deceased, type I only, *n* (%)** | 1,516 (52.7) | 728 (49.6) | 111 (41.4) |
| **Average age at death, months type I only, *n* (%)** | 18.3 (44.9) | 19.1 (37.9) | 28.2 (54.1) |
| **Resides in US, *n* (%)** | 5,640 (80.1) | 2,587 (73.0) | 529 (85.6) |
| **No. US states represented** | 50 | 50 | 46 |
| **SMA type, *n* (%)**0IIIIIIIVUnknown | 11 (0.2)2,877 (39.9)2,133 (29.6)1,229 (17.0)230 (3.2)731 (10.1) | 9 (0.3)1,469 (41.5)1,146 (32.4)624 (17.6)67 (1.9)227 (6.4) | 12 (1.6)268 (35.2)290 (38.2)173 (22.8)5 (0.7)12 (1.6) |
| **Median (range) age by SMA type, years** 0IIIIIIIV | Data not collected7 (0–57)14 (0–98)24 (2–83)55 (20–89) | Data not collected 4 (0–53)9 (0–98)17 (2–81)52 (30–78) | 03 (0–43)11 (1–76)29 (2–78)67 (58–78) |
| **Median (range) age at symptom onset by SMA type, months**0IIIIIIIV | Data not collected2 (0–12)8 (0–18)26 (3–300)Data not collected  | 12 (0–12)8 (0–18)26 (3–300)Data not collected  | 0 (0–2)2 (0–49)9 (0–160)18 (0–680)37.5 (14–420) |
| **Median (range) age at diagnosis by SMA type, months**0IIIIIIIV | 1 (0–2)4 (-8 to 267)17 (-11 to 590)56 (-5 to 912)504 (14–988) | 0.5 (0–2)4 (-8 to 127)17 (-9 to 462)47 (-5 to 912)458 (14–812) | 2 (0–13)4 (-16 to 441)16 (-7 to 344)60 (-6 to 587)557.5 (343–664) |
| **Median (range) diagnostic delay, by SMA type, months†**0IIIIIIIV | 12 (0–9)7 (0–21)15 (1–115)No data | 12 (0–9)7 (0–21)15 (1–115)No data | 1 (0–13)1 (0–437)6 (0–329)26 (0–547)329 (35–619) |

SMA, spinal muscular atrophy; US, United States.

\*760 respondents who completed the survey were not all part of the 3,542 people sent a survey invitation.

†If diagnostic delay was negative (child started to show symptoms after receiving a diagnosis), the negative value was edited to “0” to indicate no diagnostic delay.

Supplemental Table 2. 2017 Member Update Survey

# Community Update Survey

If you have any questions, please feel free to contact research@curesma.org. As a thank you for your time in completing this survey, everyone who participates will be entered into a drawing for a trip to the 2017 Annual SMA Conference in Disney World, as well as other thank you gifts. Thank you again for your participation.

Information on person completing survey (Survey can only be completed by the affected individual or their parent, legal guardian, or primary caregiver):

1. Name:
2. Email:
3. Street 1:
4. City/State/ZIP:
5. Country:
6. Phone Number:
7. Date of Birth:
8. Your relationship to affected child or individual:

[ ] Self

[ ] Parent

[ ] Grand parent

[ ] Relative

[ ] Spouse

[ ] Friend

[ ] Other

1. Are you the primary caregiver (A person who takes primary responsibility for someone who cannot care fully for themselves. May be a family member, a trained professional or another individual)?

[ ] Yes

[ ] No

For each affected child or affected individual, please complete the following information through the viewpoint of the affected individual

## **Demographics**

1. First Name:
2. Last Name:
3. Street Address:
4. City:
5. State/Province:
6. Zip Code:
7. Country:
8. Email:
9. Primary Phone Number:
10. Birthdate: *mm/dd/yyyy*
11. Marital Status

[ ] Married

[ ] Common Law (permanent living arrangement with a partner, but not married)

[ ] Widowed

[ ] Divorced

[ ] Single

1. Gender

[ ] Male

[ ] Female

1. What is your Ethnicity origin (or Race)? If mixed, please choose *other*

[ ] White

[ ] Black or African American

[ ] Native American or American Indian

[ ] Hispanic or Latino

[ ] Asian/Pacific Islander

[ ] Other

If *Other,* please specify:

1. What is the highest degree or level of school you have completed? If currently enrolled, highest degree received. If homeschooled, chose the grade equivalent.

[ ] No schooling completed

[ ] Pre-school

[ ] Kindergarten

[ ] First grade

[ ] Second grade

[ ] Third grade

[ ] Fourth grade

[ ] Fifth Grade

[ ] Sixth Grade

[ ] Seventh Grade

[ ] Eighth Grade

[ ] Some high school, no diploma

[ ] High school graduate, diploma or the equivalent (for example: GED)

[ ] Some college credit, no degree

[ ] Trade/technical/vocational training

[ ] Associate degree

[ ] Bachelor’s degree

[ ] Master’s degree

[ ] Professional degree

[ ] Doctorate degree

1. Type of SMA

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

1. How many SMN2 Copies do you have?

[ ] 1

[ ] 2

[ ] 3

[ ] 4

[ ] 5 or more

[ ] Don’t know

1. On what date were you diagnosed? *mm/dd/yyyy*

If diagnosis date is estimated, check here: [ ]

1. If you are completing this survey on behalf of an individual that has passed away, please provide the deceased date: *mm/dd/yyy*
2. If the deceased date is estimated, check here: [ ]
3. How did you first hear of Cure SMA?

[ ] Doctor or other healthcare provider

[ ] Family/Friend

[ ] Online/Website Search

[ ] Other

## **Physician Information**

1. Primary Health Care Provider - Who is your primary health care provider?

 Primary Health Care Provider's Full Name:

1. Primary Health Care Provider's Full address: (ie: Street, Suite, City, State, Zip)
2. Primary Health Care Provider's Email address if available:
3. What is your Primary Health Care Provider’s specialty?

[ ] Family practice

[ ] Internal medicine

[ ] Pediatrics

[ ] Neurology

[ ] Pulmonology

[ ] Ob/Gyn

[ ] Other

If *Other*, please specify:

1. Please identify all the specialists in your care team (Check all that apply):

☐Neurologist

☐Pulmonologist (Lung doctor)

☐Rehab Medicine/physiatrist

☐Genetics

☐Palliative Care

☐Orthopedics

☐Cardiologist (Heart doctor)

☐Physical therapist

☐Speech therapist

☐Occupational therapist

☐Nutritionist

☐Orthotist

☐Vocational Rehabilitation Counselor

☐Psychologist/Therapist

☐Gastroenterologist (Stomach Doctor)

☐Nephrologist (Kidney Doctor)

☐Endocrinologist (Metabolic Doctor)

☐Social Worker

☐Other

If Other, please specify

## **Health Information**

1. How many times in the past year (last 12 months) have you been admitted to the hospital for a reason related to SMA?
2. What type of surgeries have you EVER had related to SMA?

 [ ] Scoliosis

 [ ] Hip surgery

 [ ] Ankle/Foot surgery

 [ ] Eye surgery

 [ ] Gastrostomy

 [ ] Nissen Fundoplication (stomach wrap to prevent vomiting)

 [ ] Ileostomy

 [ ] Colostomy

 [ ] Dental Surgery

 [ ] Tonsils

 [ ] Joint contracture

 [ ] G-tubes

 [ ] Tracheotomy

1. Have you EVER fractured or broken a bone? (Check all that apply)

 [ ] Yes, I have fallen and broken a bone

 [ ] Yes, I had a stress fracture

 [ ] Yes, I broke or fractured a bone due to other reasons

 [ ] No, I have never fractured or broken a bone

 [ ] Don’t know

1. Do you currently have pain in your back, hip, groin, and/or feet?

[ ] All the time

[ ] Some of the time

[ ] Rarely

[ ] Never

[ ] Not applicable (N/A)

5. Have you ever participated in a clinical trial for SMA?

[ ] Yes

 [ ] No

 [ ] Don’t Know

## **Motor Function**

1. What is the maximum motor function you have ever achieved?

☐Head control

☐Roll over completely

☐Maintain seated position supported

☐Maintain seated position unsupported

☐Crawl combat style

☐Crawl 4 point

☐Stand with support

☐Cruise along furniture

☐Stand without support

☐Walk independently

☐None of the above

1. What is your current maximum motor function?

[ ] Head control

[ ] Roll over completely

[ ] Maintain seated position supported

[ ] Maintain seated position unsupported

[ ] Crawl combat style

[ ] Crawl 4 point

[ ] Stand with support

[ ] Cruise along furniture

[ ] Stand without support

[ ] Walk independently

[ ] None of the above

☐Not applicable (N/A)

## **Nutrition**

1. Do you currently receive any of the following for nutrition? (Check all that apply)

[ ] Standard/intact (e.g. Enfamil, Pediasure),

[ ] Hydrolyzed (e.g. Nutramigen, Peptamen Jr, Pediasure Peptide)

[ ] Elemental (e.g. Elecare, Pediatric Vivonex, Tolerex)

[ ] Commercial blenderized formula (e.g. Compleat pediatric, Nourish)

[ ] Home blenderized formula

[ ] Breast Milk

[ ] I’m not on any type of formula

☐Not applicable (N/A)

1. Do you currently have a feeding tube (e.g. G-tube, J-tube, GJ-tube, NG-tube, NJ-tube)?

☐Yes, I’m fed by a gastrostomy tube into the stomach

[ ] Yes, I’m fed by Jejunostomy tube into the small intestine

[ ] Yes, I’m fed by a nasogastric tube into the stomach

☐No, I don’t have a feeding tube

☐Unknown

☐Not applicable (N/A)

## **Breathing**

1. Do you currently use any of the following? (Choose all that apply)

[ ] Oxygen

[ ] BiPAP machine

[ ] CPAP machine

[ ] Cough Machine

[ ] Ventilator

[ ] Tracheostomy with breathing machine

[ ] None, I don’t use any breathing machines

☐Not applicable (N/A)

[ ] Other

If *Other,* please specify:

1. How many hours per day do you use oxygen or a breathing machine?

[ ] Less than 8 hours per day

[ ] 8-16 hours per day

[ ] More than 16 hours per day

[ ] I don’t use oxygen or a breathing machine

[ ] Not applicable (N/A)

## **Family/Home Life** (please remember to fill in the following questions through the viewpoint of the SMA affected individual)

1. Do you have any siblings (not including step-brothers/step-sisters)? If yes, please write down their name, age and if they are affected or not affected with SMA.

Sibling 1 Name (First and Last):

Sibling 1 birthdate: *mm/dd/yyyy*

Sibling 1 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 1 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 2 Name (First and Last):

Sibling 2 birthdate: *mm/dd/yyyy*

Sibling 2 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 2 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 3 Name (First and Last):

Sibling 3 birthdate: *mm/dd/yyyy*

Sibling 3 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 3 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 4 Name (First and Last):

Sibling 4 birthdate: *mm/dd/yyyy*

Sibling 4 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 4 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 5 Name (First and Last):

Sibling 5 birthdate: *mm/dd/yyyy*

Sibling 5 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 5 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 6 Name (First and Last):

Sibling 6 birthdate: *mm/dd/yyyy*

Sibling 6 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 6 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[x] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

1. What type of caretakers (other family members, nurses, personal care worker, attendant, in-home aide) care for you? Choose all that apply.

[ ] My parent(s)/legal guardian(s) are my full-time caretakers

[ ] My spouse is my full-time caretakers

[ ] My sibling is my full-time caretakers

[ ] Other family members

[ ] Nurses

[ ] Personal Care Worker

[ ] Attendant

[ ] In-home aid

[ ] Other

If other, please specify:

[ ] None

1. About how many hours per week do you have a caretaker, other than a family member?

[ ] 0-5 hours per week

[ ] 6-10 hours per week

[ ] 11-20 hours per week

[ ] 21-40 hours per week

[ ] 41-60 hours per week

[ ] 61-80 hours per week

[ ] 81-100 hours per week

[ ] >100 hours per week

[ ] Not applicable (N/A)

1. Are you currently employed or attending school?

[ ] Yes, I am employed full-time

[ ] Yes, I am employed part-time

[ ] Yes, I attend school full-time

[ ] Yes, I attend school and am employed

[ ] No, I am not employed nor attending school

[ ] Not applicable (N/A)

1. What is the estimated annual SMA-related expenses/costs that your family pays directly including copays, deductibles, prescriptions, medical supplies, adapted vehicles, and mobility devices.

[ ] Less than $1,000

[ ] $1,000-$1,999

[ ] $2,000-$2,999

[ ] $3,000-$4,999

[ ] $5,000-$14,999

[ ] $15,000-$19,999

[ ] $20,000-$29,999

[ ] $30,000-$39,999

[ ] $40,000-$49,999

[ ] $50,000-$79,999

[ ] $80,000-$100,000

[ ] Greater than $100,000

☐Unknown

[ ] Not applicable (N/A)

Supplemental Table 3.2018 Member Update Survey

**Community Update Survey – Year 2**

If you have any questions, please feel free to contact research@curesma.org.

**Information on person completing survey (Survey can only be completed by the affected individual or their parent, legal guardian, or primary caregiver):**

1. Name:
2. Email:
3. Street 1:
4. City/State/ZIP:
5. Country:
6. Phone Number:
7. Date of Birth:
8. Your relationship to affected child or individual:

[ ] Self

[ ] Parent

[ ] Grand parent

[ ] Relative

[ ] Spouse

[ ] Friend

[ ] Other

1. Are you the primary caregiver (A person who takes primary responsibility for someone who cannot care fully for themselves. May be a family member, a trained professional or another individual)?

[ ] Yes

[ ] No

For each affected child or affected individual, please complete the following information through the viewpoint of the affected individual

**Demographics**

1. First Name:
2. Last Name:
3. Street Address:
4. City:
5. State/Province:
6. Zip Code:
7. Country:
8. Email:
9. Primary Phone Number:
10. Birthdate: *mm/dd/yyyy*
11. Marital Status

[ ] Married

[ ] Common Law (permanent living arrangement with a partner, but not married)

[ ] Widowed

[ ] Divorced

[ ] Single

1. Gender

[ ] Male

[ ] Female

1. What is your Ethnicity origin (or Race)? If mixed, please choose *other*

[ ] White

[ ] Black or African American

[ ] Native American or American Indian

[ ] Hispanic or Latino

[ ] Asian/Pacific Islander

[ ] Other

If *Other,* please specify:

1. What is the highest degree or level of school you have completed? If currently enrolled, highest degree received. If homeschooled, chose the grade equivalent.

[ ] No schooling completed

[ ] Pre-school

[ ] Kindergarten

[ ] First grade

[ ] Second grade

[ ] Third grade

[ ] Fourth grade

[ ] Fifth Grade

[ ] Sixth Grade

[ ] Seventh Grade

[ ] Eighth Grade

[ ] Some high school, no diploma

[ ] High school graduate, diploma or the equivalent (for example: GED)

[ ] Some college credit, no degree

[ ] Trade/technical/vocational training

[ ] Associate degree

[ ] Bachelor’s degree

[ ] Master’s degree

[ ] Professional degree

[ ] Doctorate degree

1. What is your current height (in inches) and weight (in pounds)? Leave blank if not sure.

Height (inches):

Weight (pounds):

1. Type of SMA

[ ] Type 0

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

1. How many SMN2 Copies do you have?

[ ] 1

[ ] 2

[ ] 3

[ ] 4

[ ] 5 or more

[ ] Don’t know

19. At what age (in months) did you first notice something was wrong? If less than 1 month, please enter 0:

1. On what date were you diagnosed? *mm/dd/yyyy*

If diagnosis date is estimated, check here: [ ]

1. If you are completing this survey on behalf of an individual that has passed away, please provide the deceased date: *mm/dd/yyyy*
2. If the deceased date is estimated, check here: [ ]
3. How did you first hear of Cure SMA?

[ ] Doctor or other healthcare provider

[ ] Family/Friend

[ ] Online/Website Search

[ ] Other

**Physician Information**

1. Primary Health Care Provider - Who is your primary health care provider?

 Primary Health Care Provider's Full Name:

1. Primary Health Care Provider's Full address: (ie: Street, Suite, City, State, Zip)
2. Primary Health Care Provider's Email address if available:
3. What is your Primary Health Care Provider’s specialty?

[ ] Family practice

[ ] Internal medicine

[ ] Pediatrics

[ ] Neurology

[ ] Pulmonology

[ ] Ob/Gyn

[ ] Other

If *Other*, please specify:

1. Please identify all the specialists in your care team (Check all that apply):

☐Neurologist

☐Pulmonologist (Lung doctor)

☐Rehab Medicine/Physiatrist

☐Genetics

☐Palliative Care

☐Orthopedics

☐Cardiologist (Heart doctor)

☐Physical therapist

☐Speech therapist

☐Occupational therapist

☐Nutritionist

☐Orthotist

☐Vocational Rehabilitation Counselor

☐Psychologist/Therapist

☐Gastroenterologist (Stomach Doctor)

☐Nephrologist (Kidney Doctor)

☐Endocrinologist (Metabolic Doctor)

☐Social Worker

[ ] Interventional Radiologist

☐Other

If Other, please specify:

**Health Information**

1. What type of surgeries have you EVER had related to SMA?

 [ ] Spinal fusion for scoliosis

 [ ] Spinal rods for scoliosis

 [ ] MAGEC rods for scoliosis

☐ Vertical expandable prosthetic titanium rib (VEPTR) surgery

 [ ] Hip surgery

 [ ] Ankle/Foot surgery

 [ ] Eye surgery

 [ ] Gastrostomy

 [ ] Nissen Fundoplication (stomach wrap to prevent vomiting/aspiration)

 [ ] Ileostomy

 [ ] Colostomy

 [ ] Dental Surgery

 [ ] Tonsils

 [ ] Joint contracture

 [ ] G-tubes

 [ ] Tracheotomy

 [ ] Ear tubes placement (myringotomy/tympanostomy)

 [ ] I have never had surgery related to SMA

 [ ] Don’t Know

1. If you had scoliosis surgery, at what age did you have the surgery done?
2. How many times in the past year (last 12 months) have you been admitted to the hospital for a reason related to SMA?
3. Why were you hospitalized over the last 12 months? (Check all that apply)

[ ] Respiratory distress

[ ] Pneumonia

[ ] Infection other than pneumonia

[ ] Failure to thrive

[ ] Dehydration or malnutrition

[ ] Feeding tube problems

[ ] Abdominal pain

[ ] Cardiomyopathy or arrhythmia

[ ] Trauma, fracture or external injury

[ ] Seizure

[ ] Headache

[ ] Surgery

[ ] Rash

[ ] I was not hospitalized over the last 12 months

[ ] Other

If Other, please specify:

1. Have you EVER fractured or broken a bone? (Check all that apply)

 [ ] Yes, I have fallen and broken a bone

 [ ] Yes, I had a stress fracture

 [ ] Yes, I broke or fractured a bone due to other reasons

 [ ] No, I have never fractured or broken a bone

 [ ] Don’t know

1. Do you currently have pain in your back, hip, groin, and/or feet?

[ ] All the time

[ ] Some of the time

[ ] Rarely

[ ] Never

[ ] Not applicable (N/A)

1. Have you EVER participated in a clinical trial for SMA?

[ ] Yes

[ ] No

[ ] Don’t Know

1. Have you EVER been treated with the drug, Spinraza (nusinersen)?

[ ] Yes, I was in the Spinraza clinical trial or early access program (EAP)

[ ] Yes, I am currently being treated with Spinraza that my doctor has prescribed for me

[ ] Yes, I am currently being treated with Spinraza and was also in the clinical trial/EAP

[ ] Yes, I was previously (but not currently) treated with Spinraza that my doctor prescribed for me

[ ] Yes, I was previously (but not currently) treated with Spinraza that my doctor prescribed for me and was also in the clinical trial/EAP

 [ ] No, I have never been treated with Spinraza

 [ ] Don’t know

1. If you have not been treated with the drug, Spinraza (nusinersen), what are the reasons? (Check all that apply)

 [ ] I’m not familiar with Spinraza (nusinersen)

[ ] It’s too expensive

[ ] My doctor/healthcare center does not administer Spinraza

[ ] I’m currently waiting for my insurance to cover Spinraza

[ ] I am unable to receive Spinraza due to my scoliosis surgery

☐I don’t want to be treated with Spinraza right now

☐I was told I was not a candidate for Spinraza

[ ] Other

If other, please specify

1. Have you EVER been diagnosed or been told by a doctor that you have any of the following conditions? (Check all that apply)

[ ] Osteoporosis

 [ ] Asthma

 [ ] Tonsillitis

 [ ] Contractures

☐No, I have never been diagnosed with any of the above conditions

**Motor Function**

1. What was your maximum motor function 24 months ago?

☐Head control

☐Roll over completely

☐Maintain seated position supported

☐Maintain seated position unsupported

☐Crawl combat style

☐Crawl 4 point

☐Stand with support

☐Cruise along furniture

☐Stand without support

☐Walk independently

☐None of the above

1. What is the maximum motor function you have EVER achieved?

☐Head control

☐Roll over completely

☐Maintain seated position supported

☐Maintain seated position unsupported

☐Crawl combat style

☐Crawl 4 point

☐Stand with support

☐Cruise along furniture

☐Stand without support

☐Walk independently

☐None of the above

1. What is your current maximum motor function?

[ ] Head control

[ ] Roll over completely

[ ] Maintain seated position supported

[ ] Maintain seated position unsupported

[ ] Crawl combat style

[ ] Crawl 4 point

[ ] Stand with support

[ ] Cruise along furniture

[ ] Stand without support

[ ] Walk independently

[ ] None of the above

☐Not applicable (N/A)

**Nutrition**

1. Do you currently receive any of the following for nutrition? (Check all that apply)

[ ] Standard/intact (e.g. Enfamil, Pediasure),

[ ] Hydrolyzed (e.g. Nutramigen, Peptamen Jr, Pediasure Peptide)

[ ] Elemental (e.g. Elecare, Pediatric Vivonex, Tolerex)

[ ] Commercial blenderized formula (e.g. Compleat pediatric, Nourish)

[ ] Home blenderized formula

[ ] Breast Milk

[ ] I’m not on any type of formula

☐Not applicable (N/A)

1. Do you currently have a feeding tube (e.g. G-tube, J-tube, GJ-tube, NG-tube, NJ-tube)?

☐Yes, I’m fed by a gastrostomy tube into the stomach

[ ] Yes, I’m fed by Jejunostomy tube into the small intestine

[ ] Yes, I’m fed by a nasogastric tube into the stomach

☐No, I don’t have a feeding tube

☐Unknown

☐Not applicable (N/A)

**Breathing**

1. Do you currently use any of the following? (Check all that apply)

[ ] Oxygen

[ ] BiPAP machine

[ ] CPAP machine

[ ] Cough Machine

[ ] Ventilator

[ ] Tracheostomy with breathing machine

[ ] None, I don’t use any breathing machines

☐Not applicable (N/A)

[ ] Other

If *Other,* please specify:

1. How many hours per day do you use oxygen or a breathing machine?

[ ] Less than 8 hours per day

[ ] 8-16 hours per day

[ ] More than 16 hours per day

[ ] I don’t use oxygen or a breathing machine

[ ] Not applicable (N/A)

**Family/Home Life** (please remember to fill in the following questions through the viewpoint of the SMA affected individual)

1. Do you have any siblings (not including step-brothers/step-sisters)? If yes, please write down their name, age and if they are affected or not affected with SMA.

[ ] Check here if you do not have any siblings and move on to Question 2.

Sibling 1 Name (First and Last):

Sibling 1 birthdate: *mm/dd/yyyy*

Sibling 1 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 1 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 2 Name (First and Last):

Sibling 2 birthdate: *mm/dd/yyyy*

Sibling 2 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 2 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 3 Name (First and Last):

Sibling 3 birthdate: *mm/dd/yyyy*

Sibling 3 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 3 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 4 Name (First and Last):

Sibling 4 birthdate: *mm/dd/yyyy*

Sibling 4 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 4 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 5 Name (First and Last):

Sibling 5 birthdate: *mm/dd/yyyy*

Sibling 5 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 5 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

Sibling 6 Name (First and Last):

Sibling 6 birthdate: *mm/dd/yyyy*

Sibling 6 SMA Status:

[ ] Not Affected

[ ] Known Carrier of SMA

[ ] Affected with SMA

[ ] Unknown

If Sibling 6 affected with SMA, please indicate Type of SMA:

[ ] Type I

[ ] Type II

[ ] Type III

[ ] Type IV

[ ] Distal

[ ] Kennedy's

[ ] SMARD

[ ] Unknown

1. What type of caretakers (other family members, nurses, personal care worker, attendant, in-home aide) care for you? Check all that apply.

[ ] My parent(s)/legal guardian(s) are my full-time caretakers

[ ] My spouse is my full-time caretakers

[ ] My sibling is my full-time caretakers

[ ] Other family members

[ ] Nurses

[ ] Personal Care Worker

[ ] Attendant

[ ] In-home aid

[ ] None

[ ] Other

If other, please specify:

1. About how many hours per week do you have a caretaker, other than a family member?

[ ] 1-5 hours per week

[ ] 6-10 hours per week

[ ] 11-20 hours per week

[ ] 21-40 hours per week

[ ] 41-60 hours per week

[ ] 61-80 hours per week

[ ] 81-100 hours per week

[ ] >100 hours per week

[ ] Not applicable (N/A)

1. Are you currently employed or attending school?

[ ] Yes, I am employed full-time

[ ] Yes, I am employed part-time

[ ] Yes, I attend school full-time

[ ] Yes, I attend school and am employed

[ ] No, I am not employed nor attending school

[ ] Not applicable (N/A)

1. What are the estimated SMA-related expenses/costs that your family paid out of pocket including copays, deductibles, prescriptions, medical supplies, adapted vehicles, and mobility devices over the past 12 months? These expenses/costs are not what your insurance or a third party pays.

[ ] Less than $1,000

[ ] $1,000-$1,999

[ ] $2,000-$2,999

[ ] $3,000-$4,999

[ ] $5,000-$9,999

[ ] $10,000-$14,999

[ ] $15,000-$19,999

[ ] $20,000-$29,999

[ ] $30,000-$39,999

[ ] $40,000-$49,999

[ ] $50,000-$79,999

[ ] $80,000-$100,000

[ ] Greater than $100,000

☐Unknown

[ ] Not applicable (N/A)

1. If you live in the United States, what type of health insurance do you have? Check all that apply.

[ ] Cigna

[ ] Humana

[ ] Blue Cross/Blue Shield

[ ] Aetna

[ ] Kaiser Permanente

[ ] UnitedHealthcare

[ ] Wellpoint

[ ] Medicaid

[ ] Medicare

[ ] Tricare

☐Don’t Know

[ ] I don’t have health insurance

[ ] I don’t live in the United States

[ ] Other

If *Other*, please specify:

1. How long have you had the above insurance?

[ ] Less than 1 year

[ ] 1-2 years

[ ] 2-5 years

[ ] 5-10 years

[ ] More than 10 years

[ ] Don’t Know

[ ] I don’t have health insurance