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Polling Questions

***Only a person with SMA and parents/caregivers answering on behalf of a person with SMA should answer these questions. If the person with SMA is answering for her/himself, the parent, if also present/participating, should not answer on that person's behalf. If a parent/caregiver cares for more than one child/person with SMA, he/she should only answer for one of them.**

Demographic Questions

1. **Where do you live?**
 - a. Within Washington, D.C. metropolitan area (including the Virginia and Maryland suburbs)
 - b. East Coast (outside the Washington D.C area)
 - c. Midwest
 - d. West Coast
 - e. Northwest
 - f. Outside of the U.S.(International participants)

2. **Do you live in:**
 - a. A city
 - b. A rural area
 - c. A suburban area

3. **Which of the following best describes you?**
 - a. I have SMA (Type 1, 2, 3, 4)
 - b. I am the parent or caregiver with someone with SMA (Type 0,1, 2, 3, 4)

4. **Have you/your loved one been diagnosed with any of the following SMA types?**
 - a. SMA type 0
 - b. SMA type 1
 - c. SMA type 2
 - d. SMA type 3
 - e. SMA type 4
 - f. Unknown

5. **What is the length of time since your/your loved one's diagnosis?**
 - a. Less than 1 year ago
 - b. 1 year ago to less than 2 years ago
 - c. 2 years ago to less than 5 years ago
 - d. 5 or more years ago
 - e. I'm not sure



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6. **What is your/your loved one's age?**
 - a. ≤ 2 years
 - b. 3-12 years
 - c. 13-17 years
 - d. 18 – 34
 - e. 35 - 49
 - f. 50 – 65
 - g. older than 65

7. **You are/your loved one is:**
 - a. Male
 - b. Female

8. **On a day to day basis, *primary* caregiving for me/my loved one is provided by:**
 - a. Parent(s)
 - b. Partner/spouse
 - c. Grandparent(s)
 - d. Sibling/other family member
 - e. Nurse or other professional caregiver
 - f. Other (friend, nanny, other relative)

Topic 1 Polling Questions

9. **In the past year, how often have you/your loved one had to go to the hospital, for emergency care or inpatient hospitalization due to your/your loved one's SMA?**
 - a. None in the past year
 - b. 1 – 2 times
 - c. 3 – 5 times
 - d. 6 – 10 times
 - e. More than 10 times

10. **In the past year, how often have you/your loved one had to go to a doctor or a specialty provider for routine care, or follow up of your/your loved one's SMA (*this may include routine doctor's appointments that are in a hospital setting*)**
 - a. None in the past year
 - b. 1 – 2 times
 - c. 3 – 5 times
 - d. 6 – 10 times
 - e. More than 10 times



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11. Which of the following symptoms currently has the most significant impact on you/your loved one's life? Select TOP 4

- a. Breathing difficulties (shallow, rapid, depressed breathing, etc.)
- b. Breathing/lung infections (e.g. pneumonia, viral infections, etc.)
- c. Bone fractures/Hip dislocation
- d. Communication difficulties
- e. Feeding/swallowing difficulties
- f. Falls
- g. Fatigue
- h. Inability to cough/clear lung secretions
- i. Joint contractures (tight muscles and tendons) / severe scoliosis
- j. Muscle weakness (facial, neck, arms, forearms, hips, legs)
- k. Paralysis
- l. Respiratory Failure requiring assistive devices (BiPAP, Ventilator, etc.)
- m. Sleep problems
- n. Other

12. What specific activities that are most important to you/your loved one are you/your loved one not able to do because of SMA? Select TOP 4

- a. Feed oneself
- b. Dress oneself
- c. Going to restroom by oneself
- d. Transferring (from wheelchair/scooter to bed, toilet, etc.)
- e. Turning in bed
- f. Attend to personal hygiene independently
- g. Independence in mobility (around the house, to work, to school)
- h. Attend work or school
- i. Engage in social activities and building relationships (playdates, dining out, dating, hugging my partner)
- j. Engage in physical activities (playing sports, going to the gym)
- k. Ability to spend time alone / be independent
- l. Other

13. Which of the following have you experienced as a result of coping with your/your loved ones SMA? Select ALL that apply

- a. Depression
- b. Anxiety
- c. Social isolation
- d. Loss of job
- e. Troubled relationships
- f. Other



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14. What is the estimated annual SMA-related expenses/costs that you and your family pay directly including copays, deductibles, prescriptions, medical supplies, adaptive vehicles, and mobility devices? Select **ONE** option
- a. <\$500
 - b. \$500-\$999
 - c. \$1,000-\$1,999
 - d. \$2,000-\$2,999
 - e. \$3,000-\$4,999
 - f. \$5,000-\$14,999
 - g. \$15,000-\$19,999
 - h. \$20,000-\$29,999
 - i. \$30,000-\$39,999
 - j. \$40,000-\$49,999
 - k. \$50,000-\$79,999
 - l. \$80,000-\$100,000
 - m. >\$100,000
 - n. Unknown
 - o. Not applicable (N/A)

Topic 2 Polling Questions

15. Have you/ your loved one ever been prescribed (either by your doctor or through a clinical trial) and taken the following medications? Select **ALL** that apply
- a. Albuterol (inhaled)
 - b. Albuterol (liquid)
 - c. Albuterol (tablet)
 - d. Carnitine
 - e. Creatine
 - f. Hydroxyurea
 - g. Steroids
 - h. Valproic Acid (VPA)
 - i. Sodium Phenylbutyrate
 - j. Riluzole
 - k. Nusinersen/Spinraza
 - l. Other



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16. **What are you currently doing to help treat your/your loved one's SMA/SMA symptoms? Select ALL that apply**
- a. Respiratory Maintenance (may include Chest PT, suctioning, airway clearance, cough assistance, breathing support, devices, etc.)
 - b. Nutritional support (nasogastric tube, nasojejunal (NJ) tube, gastrostomy (G) tube)
 - c. Physiotherapy /Physical Therapy (PT)
 - d. Aqua Therapy
 - e. Occupational Therapy (OT)
 - f. Speech Therapy
 - g. Mobility equipment (adaptive strollers, wheelchair, scooters, adaptive tricycles, crutches, walkers)
 - h. Orthotics' support (Braces [AFOs, KAFOs, TLSO], neck collar, splints, etc.)
 - i. Orthopedic support (Stander)
 - j. Approved therapy (Spinraza)
 - k. Investigational product (clinical trial)
 - l. Other
17. **Which of these do you/your loved one use for respiratory assistance? Select ALL that apply**
- a. Chest physiotherapy (CPT) for clearance/comfort
 - b. Postural Drainage
 - c. High frequency chest wall oscillation (VEST ©)
 - d. Cough Assist Device
 - e. Suction to remove secretions
 - f. Non-invasive ventilation (NIV), such as BiPAP
 - g. Invasive ventilation / Mechanical ventilator (with tracheotomy)
 - h. Other
18. **Have you/your loved one undergone Scoliosis surgery (growing rods or spinal fusion)?**
- a. Yes
 - b. No



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19. Which outcome below would you rank as most important for a *possible* drug treatment? Select **ONE option**

- a. The treatment will provide gains in function (e.g., increased strength, energy, doing something I was unable to do before)
- b. The treatment will lessen symptoms that would improve my/my loved one's current quality of life and /or allow for enhanced activities of daily living
- c. The treatment will stop or slow down disease progression (even if does not provide lessening of symptoms that would improve my/my loved one's current quality of life and /or allow for enhanced activities of daily living)
- d. The treatment will prolong life span
- e. Other

20. Which of the following factors would influence your decision to not use or stop a given treatment? Select **ALL that apply**

- a. The significant risks of serious side effects such as cardiac or kidney issues
- b. The common side effects of the treatment, such as nausea, loss of appetite, etc.
- c. The way that treatment is administered (for example, orally, intravenously, intrathecally),
- d. How long the treatment takes, whether it requires hospitalization, required doctors visits, etc.
- e. The time that it would take away from my daily activities, job, school, etc.
- f. The burden of administration, such as the need for anesthesia, radiation exposure, surgical procedure, etc.
- g. Cost
- h. Other



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On Clinical Trials

21. Have you /your loved one ever participated in any type of clinical trial studying experimental treatments for SMA? Select ALL that apply

- a. Yes
- b. No
- c. Tried to enroll in a clinical trial, but did not qualify
- d. Tried to enroll but trial enrolment was closed
- e. Did not want to enroll due to burden of trial (travel, potential risks, time missed from work, etc.)
- f. Did not want to enroll for other reasons
- g. I've received access to an experimental drug through an Expanded Access / Compassionate Use Program but did not participate in a clinical trial for this drug
- h. I'm not sure

22. Which of the following factors would you rank as most important to your decision about whether to participate in a clinical trial to study an experimental treatment? Select TOP 4

- a. Reputation of study site PI (Doctor)
- b. Common side effects (headache, back-pain, skin rashes)
- c. The risk of rare but serious side effects (life-threatening allergic reaction)
- d. How the treatment might prevent further disease progression or improve my/my loved one's health
- e. How the trial might affect my/my loved one's current treatment plan
- f. Promise of receiving open label therapy at the end of the study
- g. Proximity of the study site
- h. Frequency of visits
- i. Duration of visits
- j. Availability of safety data
- k. Availability of preclinical or animal model efficacy data

23. What type of insurance do you or your loved one have? Select ALL that apply

- a. Private/commercial health insurance (e.g. Aetna, Blue Cross Blue Shield, etc.)
- b. Medicare
- c. Medicaid
- d. TRICARE health insurance
- e. VA Care health insurance