

Externally-Led Patient-Focused Drug Development Meeting

March 29, 2019 – College Park Marriott – Hyattsville, MD

EI-PFDD Polling Questions – Pediatric Mitochondrial Disease with Neurologic Manifestations

Demographics

1. Which of the following best describes you?
 - a. I am a parent/caregiver of a child who has a neurologic manifestation of mitochondrial disease.
 - b. I have lost a child who had a neurologic manifestation of mitochondrial disease.
2. Where do you currently reside?
 - a. US Pacific (including California)
 - b. US West and Mountain
 - c. US Midwest
 - d. US South (including Texas)
 - e. US Northeast and New England
 - f. Canada
 - g. Mexico
 - h. Outside of North America
 - i. Other
3. Do you live in
 - a. a city
 - b. a rural area
 - c. a suburban area
4. How old is the patient now?
 - a. 0-10 years old
 - b. 11-17 years old
 - c. 18-20 years old
 - d. 21-30 years old
 - e. > 30 years old
5. At what age was the patient diagnosed with mitochondrial disease?
 - a. 0-10 years old
 - b. 11-17 years old
 - c. 18-20 years old
 - d. 21-30 years old
 - e. > 30 years old

Pediatric Mitochondrial Disease Patients (Neuro) – Symptoms and Daily Impact

1. Please select the answer that best describes the patient's stage of disability.
 - a. Minimal disability, able to run or jump.
 - b. Symptoms present but mild, able to walk and capable of leading independent life.
 - c. Symptoms are overt and significant. Require regular or periodic holding on to wall or another person for stability and walking.
 - d. Walking requires a walker or other aid such as service dog. Can perform several activities of daily living.
 - e. Not able to walk, confined to wheelchair. Can perform some activities of daily living that do not require standing or walking.
 - f. Severe disability, dependency on others for assistance with all activities of daily living.
2. Select the mitochondrial disease symptoms that most impact the patient's daily quality of life. Select up to 5.
 - a. Muscle Weakness
 - b. Speech Problems
 - c. Chronic Fatigue
 - d. Gastrointestinal Problems
 - e. Balance Problems
 - f. Sleep Difficulties
 - g. Learning Disability
 - h. Movement Disorders (chorea, tremors, dystonia)
 - i. Delayed Milestones
 - j. Decreased Vision

Pediatric Mitochondrial Disease Patients (Neuro) with Continues – Symptoms and Daily Impact

- k. Exercise Intolerance
 - l. Seizures
 - m. Headache
 - n. Other
3. As mitochondrial disease progresses, development or progression of which of the following symptoms worries you the most? Select up to 5.
- a. Muscle Weakness
 - b. Speech Problems
 - c. Chronic Fatigue
 - d. Gastrointestinal Problems
 - e. Balance Problems
 - f. Sleep Difficulties
 - g. Learning Disability
 - h. Movement Disorders (chorea, tremors, dystonia)
 - i. Delayed Milestones
 - j. Impaired Vision
 - k. Exercise Intolerance
 - l. Seizures
 - m. Headache
 - n. Other
4. What specific activities of daily life are most important to your child and they are NOT able to do because of mitochondrial disease? Select TOP 3.
- a. Moving around independently and safely, walking and standing
 - b. Speaking with others, being understood (especially in noisy settings)
 - c. Personal hygiene, taking a shower, dressing independently, etc.
 - d. Feeding oneself, cutting food and handling utensils
 - e. Going to school or work
 - f. Writing and typing
 - g. Reading books, seeing a computer screen or phone
 - h. Manipulating small objects (e.g., a key, picking up items)

- i. Other
5. As a result of living with mitochondrial disease, which of the following social, emotional or economic consequences are most significant to your child? Select up to 4.
- a. Frustration
 - b. Social isolation
 - c. Loss of independence
 - d. Communication issues
 - e. Lack of hope for the future
 - f. Depression and/or anxiety
 - g. Trouble building or maintaining relationships
 - h. Modified school hours
 - i. Loss of hobbies or activities
 - j. Other

Pediatric Mitochondrial Disease Patients (Neuro) – Current and Future Approaches to Treatment

1. What PRESCRIPTION MEDICATIONS does the patient take now to treat symptoms for mitochondrial disease? Select ALL that apply.
- a. Pain medications
 - b. Heart medications
 - c. Antidepressants or anti-anxiety medications
 - d. Muscle relaxants
 - e. Intravenous Immunoglobulin therapy (IVIg)
 - f. Diabetes medications
 - g. Experimental medications as a part of a clinical trial
 - h. Other prescription medications not listed
 - i. Nothing
2. What VITAMINS or SUPPLEMENTS does the patient take now to treat symptoms for mitochondrial disease? Select ALL that apply.
- a. CoQ10
 - b. Carnitine
 - c. Riboflavin

Pediatric Mitochondrial Disease Patients (Neuro) – Current and Future Approaches to Treatment

- d. Creatine
 - e. Vitamin E
 - f. Alpha lipoic acid
 - g. Vitamin B3, Nicotinamide or Niacin
 - h. Idebenone
 - i. Other supplements or vitamins not listed
 - j. Nothing
3. What are you currently doing to help manage mitochondrial disease or mitochondrial disease symptoms? Select ALL that apply.
- a. Physical therapy, including aqua or hippo therapy
 - b. Modifications/accommodations at school/home
 - c. Occupational therapy
 - d. Use of adaptive devices
 - e. Speech therapy
 - f. Choice of diet
 - g. Stretching
 - h. Exercise (cardio or strength training)
 - i. Mental health services
 - j. Nothing
 - k. Other
4. In general, how much do the medications, therapies or lifestyle changes used improve the patient's quality of life?
- a. No benefit
 - b. Help somewhat
 - c. Help a lot
 - d. Significant benefit
 - e. Not sure
5. Which outcome is MOST important for a possible drug treatment?
- a. Slowing/stopping of progression (even if no gain in function, symptoms won't get worse)
 - b. Gain in function (e.g., energy, strength, mobility, dexterity, cardiac function, speech)
 - c. Prolong life
 - d. Other
6. Which ability or symptom would you rank as most important for a possible drug treatment today? Select up to 3.
- a. Reduced Muscle Weakness
 - b. Improved Speech
 - c. Reduced Chronic Fatigue
 - d. Reduced Gastrointestinal Problems
 - e. Improved Balance
 - f. Reduced Sleep Difficulties
 - g. Reduced Learning Disability
 - h. Improved Movement Disorders (chorea, tremors, dystonia)
 - i. Improved Meeting Milestones
 - j. Improved Vision
 - k. Reduced Exercise Intolerance
 - l. Reduced Seizures
 - m. Reduced Headache
 - n. Other
7. Which of the following factors would influence your decision to have your child take a new medication or participate in a clinical trial or research study? Select ALL that apply.
- a. Significant risks of serious side effects (cardiac, kidney issues, etc.)
 - b. Common side effects of the treatment (nausea, headaches, etc.)
 - c. The way that treatment is administered (orally, intravenously, subcutaneous)
 - d. Length of treatment, requires hospitalization, frequent doctor visits, etc.
 - e. Burden of administration (need for anesthesia, radiation exposure, surgery, etc.)
 - f. Changing my current treatment or management plan (stopping a medication, supplement, or exercise)
 - g. Cost and/or travel
 - h. None of these
 - i. Other