

Externally-Led Patient-Focused Drug Development Meeting

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

EI-PFDD Polling Questions - for Mitochondrial Disease Affected Adults with Myopathies

Demographics

1. Which of the following best describes you?
 - a. I am an affected adult with a mitochondrial myopathy.
 - b. I am a caregiver for an affected adult with mitochondrial myopathy.
 - c. I have lost a loved one who was an adult patient with mitochondrial myopathy.
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. 18-20 years old
 - b. 21-30 years old
 - c. 31-40 years old
 - d. 41-50 years old
 - e. > 50 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. 31-40 years old
 - f. > 40 years old

Mitochondrial Disease Adult Patients with Myopathies – Symptoms and Daily Impact

1. Please select the answer that best describes the stage of disability for you or the person for whom you care.
 - 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 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 a service dog. Abilities vary from day to day.
 - 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 your daily quality of life. Select up to 5.
 - a. Chronic Fatigue
 - b. Muscle Weakness
 - c. Gastrointestinal Problems
 - d. Exercise Intolerance
 - e. Sleep Difficulties
 - f. Dysautonomia
 - g. Headaches or Migraines
 - h. Peripheral Neuropathy
 - i. Eye Muscle Problem and/or Vision Impairment
 - j. Mood Disorder
 - k. Other

Mitochondrial Disease Adult Patients with Myopathies Continues – Symptoms and Daily Impact

3. As mitochondrial disease progresses, development or progression of which of the following symptoms worries you the most? Select up to 5.
 - a. Chronic Fatigue
 - b. Muscle Weakness
 - c. Gastrointestinal Problems
 - d. Exercise Intolerance
 - e. Sleep Difficulties
 - f. Dysautonomia
 - g. Headaches or Migraines
 - h. Peripheral Neuropathy
 - i. Eye Muscle Problem and/or Vision Impairment
 - j. Mood Disorder
 - k. Other
4. What specific activities of daily life are most important to you that you (or the person for whom you care) are NOT able to do because of mitochondrial disease? Select TOP 3.
 - a. Speaking with others, being understood (especially in noisy settings)
 - b. Driving
 - c. Personal hygiene, taking a shower, dressing independently, etc.
 - d. Walking, moving around independently and safely
 - e. Writing and typing
 - f. Feeding oneself, cutting food and handling utensils
 - g. Manipulating small objects (e.g., a key, picking up items)
 - h. Reading books, seeing a computer screen or phone
 - i. Other
5. As a result of living with mitochondrial disease, which of the following social, emotional or economic consequences are most significant to you? Select up to 4.
 - a. Loss of hobbies or activities
 - b. Social isolation
 - c. Frustration
 - d. Depression and/or anxiety
 - e. Financial difficulties
 - f. Loss of job or inability to get a job

- g. Trouble building or maintaining relationships
- h. Lack of hope for the future
- i. Loss of independence
- j. Modified work/school hours
- k. Communication Issues
- l. Other

Mitochondrial Disease Adult Patients with Myopathies – Current and Future Approaches to Treatment

1. What PRESCRIPTION MEDICATIONS do you take now to treat symptoms of your 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 do you take now to treat symptoms of your mitochondrial disease? Select ALL that apply.
 - a. CoQ10
 - b. Carnitine
 - c. Riboflavin
 - 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. Choice of diet
 - b. Modifications/accommodations at work/in school/at home

Mitochondrial Disease Adult Patients with Myopathies Continues – Current and Future Approaches to Treatment

- c. Physical therapy, including aqua or hippo therapy
 - d. Stretching
 - e. Use of adaptive devices
 - f. Exercise (cardio or strength training)
 - g. Mental health services
 - h. Occupational therapy
 - i. Speech therapy
 - j. Nothing
 - k. Other
4. In general, how much do the medications, therapies or lifestyle changes used improve your 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 Chronic Fatigue
 - b. Reduced Muscle Weakness
 - c. Reduced Gastrointestinal Problems
 - d. Reduced Exercise Intolerance
 - e. Reduced Sleep Difficulties
 - f. Reduced Dysautonomia
 - g. Reduced Headaches or Migraines
 - h. Reduced Peripheral Neuropathy
 - i. Reduced Mood Disorder
 - j. Reduced Eye Muscle Problems and/or Improved Vision
 - k. Other
7. Which of the following factors would influence your decision to 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. Cost and/or travel
 - c. The burden of administration (need for anesthesia, radiation exposure, surgical procedure, etc.)
 - d. Common side effects of the treatment (nausea, loss of appetite, headache, etc.)
 - e. Length of treatment, requires hospitalization, requires numerous doctors visits, etc.
 - f. Changing my current treatment or management plan (stopping a medication, supplements or exercise)
 - g. The way that treatment is administered (orally, intravenously, subcutaneous)
 - h. None of these
 - i. Other