Elamipretide Effects in Adults with Primary Mitochondrial Myopathy: Phase 2 Double-Blind, Randomized, Placebo-Controlled Crossover Trial (MPMPOWER-2)

INTRODUCTION

• Primary mitochondrial myopathies (PMMs) are genetic disorders that impair normal mitochondrial function, affecting skeletal muscle, 
  • Decreased tolerance to physical exercise because of skeletal muscle respiratory chain dysfunction
  • Mitochondrial dysfunction
  • Tiredness and muscle weakness
  • Fatigue
  • Reduced exercise capacity
  • Symptoms of fatigue

RESULTS

Demographic and Other Baseline Characteristics

Table 1. MPMPOWER-2 Patient Baseline Characteristics

Table 2. Treatment-emergent Adverse Events (AEs) (≥2 Participants)

METHODS

Clinical Procedures and Outcomes

• Primary objective: To evaluate the effect of 4 weeks of a once-daily dose of SC ELAM on the distance walked in the 6MWT at Baseline and end of each treatment period values recorded (in meters)

Safety Evaluation

• Injection site reactions were the most commonly reported AEs with ELAM (60%) (Table 2)

Efficacy Findings

• ELAM resulted in an improvement of 13.8 meters (6MWT - end of treatment period) compared with placebo (95% confidence interval [CI], -2.8, 42.5; P = 0.0056 Figure 5)

CONCLUSIONS

• ELAM treatment was well tolerated by most participants

REFERENCES


Acknowledgments: Patients and families for their participation. MitoAction and the United Mitochondrial Disease Foundation for helping with recruitment. We also thank Harvard Catalyst (http://catalyst.harvard.edu/) for critical intellectual content. Jeffrey S. Fenster, PhD,iju Pari Pharma Consult, LLC for statistical consultation.

Study funding: Trial funded by Biotherapies, Thorpeter, MA