INTRODUCTION
• Barth syndrome (BTHS) is a rare, X-linked disease caused by defects in TAZ, the talassemia encoding gene, responsible for the final maturation step to mature cardiolipin, critical for mitochondrial function
• The inability to produce mature cardiolipin leads to clinical manifestations of BTHS, including cardiac and skeletal myopathy, neuropathy, and growth abnormalities
• Elamipretide localizes to the inner mitochondrial membrane, where it is believed to associate with cardiolipin, improving membrane stability and ATP production and reducing pathogenic ROS production
• The efficacy and safety of elamipretide are being studied in TAZPOWER, which is the first clinical trial to evaluate a therapeutic agent in patients with BTHS

OBJECTIVE
• To measure efficacy through functional, patient-reported outcome (PRO) assessments, and cardiac parameters and safety/tolerability through adverse events (AEs) and laboratory tests

METHODS

RESULTS

Patient Demographics
• A total of 12 patients were randomized into the trial

Table 1. Patient Demographics (N=12)

Table 2. Treatment-Emergent Adverse Events

Cardiac Findings
• At the end of the double-blind phase of the TAZPOWER trial, statistical significance was not achieved in the ITT population on the primary endpoint

Open-label Extension
• A total of 10 patients elected to continue into the TAZPOWER open-label extension (OLE)

RESULTS

Figure 4. Summary of Treatment Effect Changes from Baseline to Week 36 OLE (n=6)

CONCLUSIONS
• TAZPOWER is the first clinical trial to evaluate the tolerability and efficacy of a potential therapeutic agent in patients with BTHS
• Blinded Phase of the TAZPOWER Trial
  - Statistical significance was not achieved in the ITT population on the primary endpoint; elamipretide provided clinically meaningful improvements in individual functional and PROs
  - Elamipretide was generally well tolerated; most adverse events were mild to moderate in severity, with the most commonly reported adverse events including injection site reactions
• Open-Label Extension Phase of the TAZPOWER Trial
  - At 36-week OLE, elamipretide therapy was associated with improvements in 6MWT, BTHS-SA Total Fatigue Scores, and PRO assessments
  - Safety and tolerability of elamipretide was consistent with blinded phase observations
• Cardiac Findings at Open-Label Extension
  - At 36-week OLE, elamipretide showed a statistically significant improvement in indexed stroke volume

Safety and Tolerability
• There were 91 TEAEs reported, with 1 serious AE deemed not related to elamipretide; injection site reactions occurred in 100% of patients while on elamipretide

Table 3. Efficacy and Tolerability of Elamipretide in Patients with Barth Syndrome: Results from TAZPOWER, a Randomized, Double-Blind, Placebo-Controlled, Crossover and Open-Label Extension Trial

Figure 5. Summary of Efficacy and Tolerability of Elamipretide in Patients with Barth Syndrome: Results from TAZPOWER, a Randomized, Double-Blind, Placebo-Controlled, Crossover and Open-Label Extension Trial

Figure 6. Cardiac Assessment

Average BSA Indexed Stroke Volume

Baseline OLE Week 12 OLE Week 24 OLE Week 36

p=0.025

p<0.05

p=0.004

Table 4. Summary of Treatment Effect Changes from Baseline to Week 36 OLE (n=6)

Figure 1. Study Design

Figure 2. Functional Assessments

Figure 3. Patient-reported Outcomes Assessments

Figure 5. Summary of Efficacy and Tolerability of Elamipretide in Patients with Barth Syndrome: Results from TAZPOWER, a Randomized, Double-Blind, Placebo-Controlled, Crossover and Open-Label Extension Trial

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