

**FOOD AND DRUG ADMINISTRATION (FDA)**  
Center for Drug Evaluation and Research (CDER)

*Cardiovascular and Renal Drugs Advisory Committee (CRDAC) Meeting*  
October 10, 2024

**QUESTIONS**

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1. **DISCUSSION:** Discuss whether SPIBA-201, Part 2 demonstrates that elamipretide is effective for the treatment of Barth syndrome (BTHS). Include in your discussion the interpretability of the single-arm, open label study design and the findings on:
  - a. 6-minute walk distance
  - b. Other functional outcomes: hand-held dynamometry, 5 times sit-stand-test, SWAY application balance.
  - c. Echocardiography
  - d. Patient reported outcomes (e.g., BTHS-Symptom Assessment total fatigue score, patient- and caregiver global impression scales)
  - e. Monolysocardiolipin (MLCL) to tetralinoleoyl cardiolipin (CL) ratio (MLCL:CL ratio)
  
2. **DISCUSSION:** Discuss whether SPIBA-001 demonstrates that elamipretide is effective for the treatment of Barth syndrome. Include in your discussion the interpretability of the externally-controlled study design and the findings on:
  - a. 6-minute walk distance
  - b. Other functional outcomes: hand-held dynamometry, 5 times sit-stand-test, SWAY application balance.
  - c. Echocardiography
  
3. **DISCUSSION:** Discuss the extent to which other data (e.g., nonclinical data or other clinical study results) support the effectiveness of elamipretide.
  
4. **VOTE:** Based on available evidence, do you conclude that elamipretide is effective for the treatment of Barth syndrome?

Provide rationale for your vote.

If you voted **yes**, specify the evidence of elamipretide's effectiveness.

If you voted **no**, provide recommendations for additional data that may support a conclusion that elamipretide is effective.