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

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

DRAFT 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.