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COMMODITIES, RISK MANAGEMENT,
AND CREDIT

U.S. House of Representatives
Rep. Sharice L. Davids
Kansas Third District

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May 8, 2025

The Honorable Martin A. Makary
Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Ave
Building 32, Room 2356
Silver Spring, MD 20993

Dear Commissioner Makary,

I forward to you a letter my office received from Emily Milligan, Executive Director of Barth Syndrome Foundation (BSF), on April 30, 2025. BSF is the only worldwide volunteer organization dedicated to combatting Bath syndrome, an ultrarare and life threatening genetic disorder affecting one of my constituents.

Ms. Milligan expresses deep concern with the Food and Drug Administration's (FDA) failure to meet the April 29, 2025, decision date for elamipretide, a potential therapy for Barth syndrome. In October 2024, the FDA's Cardiovascular and Renal Drugs Advisory Committee voted in favor of the effectiveness of elamipretide, supporting its approval for the treatment of Barth syndrome. With no currently FDA-approved treatments for Barth syndrome, and no other therapies in late-stage clinical development, families living with this devastating disease – including my constituents in Kansas' Third District – cannot afford continued delays.

Therefore, I respectfully ask that the FDA quickly and publicly communicate a new approval decision date for elamipretide – preferably one that will occur as soon as possible given the urgency of this matter. I also ask that the FDA provide full and fair consideration of Ms. Milligan's request, on behalf of BSF, to approve this treatment for those living with Barth syndrome.

Sincerely,

Sharice L. Davids
Member of Congress

ENC: Original correspondence from Emily Milligan on behalf of BSF.

Barth Syndrome Foundation®

"Saving lives through education, advances in treatments, and finding a cure for Barth syndrome."

April 30, 2025

The Honorable Sharice L. Davids
U.S. House of Representatives
2435 Rayburn House Office Building
Washington, D.C. 20515

Dear Representative Davids,

On behalf of the Barth Syndrome Foundation (BSF) and the families we serve, I write to express deep concern regarding the U.S. Food and Drug Administration's failure to meet the April 29 decision date for elamipretide, a potential therapy for Barth syndrome.

Barth syndrome is an ultra-rare, life-threatening genetic disorder that primarily affects males. There are no FDA-approved treatments—and no other therapies in late-stage clinical development. Families living with this devastating disease cannot afford continued delays.

Last October, the FDA's Cardiovascular and Renal Drugs Advisory Committee voted 10–6 in favor of the effectiveness of elamipretide, supporting its approval for the treatment of Barth syndrome. Clinical studies have shown that elamipretide can improve mitochondrial function and increase muscle strength by 45% and heart function by 40%. Some patients have now taken this therapy for more than eight years and continue to benefit. Many others, including individuals in acute cardiac distress, have received emergency access through FDA-authorized expanded access programs.
We respectfully urge the FDA to:

- Complete its review as quickly as possible (exercising regulatory flexibility in recognition of the ultra-rare nature of our patient group and the extreme unmet clinical need);
- Communicate a new and definitive decision date; and
- Approve elamipretide for all ages of individuals living with Barth syndrome.

Individuals and families affected by Barth syndrome are not alone in this request. Leading rare disease organizations—including the United Mitochondrial Disease Foundation, MitoAction, Friedreich's Ataxia Research Foundation, PolG Foundation, and the Children's Cardiomyopathy Foundation—have joined us in urging the FDA to act swiftly and decisively.

Your continued leadership and compassion give our community strength. We would be grateful if you would consider communicating these concerns and requests to the FDA and reinforcing the urgency, clarity, and action our community needs.

With sincere thanks,

Emily Milligan
Executive Director
Barth Syndrome Foundation
emily.milligan@barthsyndrome.org