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Congress of the United States
House of Representatives
Washington, DC 20515

March 4, 2025

The Honorable Sara Brenner
Acting Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993

Dear Acting Commissioner Brenner:

I am writing you today to respectfully request your prompt attention to the review and final approval process for elamipretide—a first of its kind, experimental medication aimed at treating Barth Syndrome.

As you know, Barth Syndrome is an ultra-rare genetic mitochondrial disorder that effects 1 in 400,000 individuals worldwide. In most cases Barth Syndrome is found in males and is usually prevalent in a patients' body during early infancy or childhood—with 85% of early deaths occurring by age five. According to recent research published by the Indiana University School of Medicine, there are fewer than 400 cases of known Barth Syndrome globally. One of these brave carriers is a one-year-old boy located in Omaha, Nebraska, Jaylen Karle. Jaylen and his mother, Jordan, have been fighting since Jaylen was diagnosed with Barth Syndrome to see that elamipretide is fully approved by the FDA as soon as safely possible to do so.

Unfortunately, these efforts have received consistent delays, as the initial preclinical studies for this life-altering treatment began in 2014. After receiving a priority review designation by FDA, this review process is now going on 15 months—while typically, priority review designation is a six-month process. This includes yet another delay, caused by an extension for the Prescription Drug User Fee Act (PDUFA) action date for the New Drug Application (NDA) for elamipretide. Despite having not received any safety concerns or requests for new pre-marketing studies from FDA, as well as an October 10, 2024 vote by the FDA advisory committee in favor of approving elamipretide, the deadline for full approval has now been extended to April 29, 2025.

Given the early-onset nature of this disease, as well as the successful research conducted on the effectiveness of this treatment, priority for full approval by the FDA for elamipretide must be of paramount importance. I strongly urge you to consider full approval of elamipretide at the earliest opportunity that aligns with the safety guidelines and processes of FDA.

I appreciate your attention to this matter. If you have any questions, please contact me or my team at 202-225-4806, or my Legislative Assistant, Joshua McGuire, at Joshua.McGuire@mail.house.gov.

Yours very truly,

Mike Flood
Member of Congress