

**Congress of the United States**  
**Washington, DC 20515**

September 16, 2025

The Honorable Martin A Makary, M.D., M.P.H.  
Commissioner  
U.S. Food and Drug Administration (FDA)  
10903 New Hampshire Ave  
Silver Spring, MD 20993

Dear Commissioner Makary:

We are writing regarding FDA's ongoing review of the use of elamipretide to treat Barth syndrome — a rare mitochondrial condition that can cause severe and debilitating muscle weakness, cardiomyopathy, and cardiac dysfunction which can lead to premature death, infections, and delayed growth.

Approval decisions are the FDA's to make. However, the FDA's most recent review of elamipretide ended in May 2025 with the FDA denying approval well after the statutorily required deadline for a priority review designated therapy. FDA then requested a complete, class 2 resubmission with a 6-month user fee goal date of February 25, 2026. Following previous delays, it is our understanding that FDA advised the sponsor that it would accelerate its planned action date to September 26, 2025.

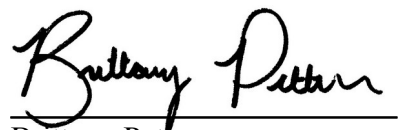
We would like to applaud the agency for its recent commitment to providing an accelerated goal date as there are no known cures or FDA-approved treatments for Barth syndrome, particularly for infants in acute cardiac distress. However, we remain concerned that continued or additional delays beyond the planned action date may prematurely shut off access to potentially life-saving drugs and leave patients and families in our districts with no viable alternatives.

As such, we request your continued full and fair consideration of potential treatments for Barth syndrome. Additionally, we urge your timely response and commitment to meeting goal dates for rare disease products in accordance with all existing rules, regulations, and ethical guidelines. We appreciate your consideration and engagement with our staff.

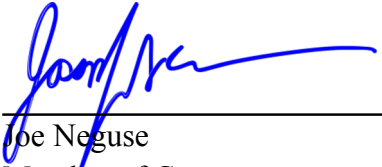
Sincerely,



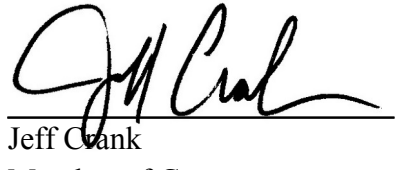
Jason Crow  
Member of Congress



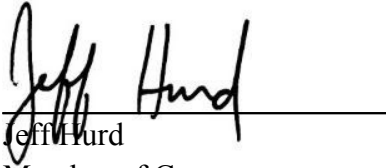
Brittany Pettersen  
Member of Congress



Joe Neguse  
Member of Congress



Jeff Crank  
Member of Congress



Jeff Hurd  
Member of Congress



Gabe Evans  
Member of Congress



Lauren Boebert  
Member of Congress

CC:

The Honorable Robert F. Kennedy, Jr., Secretary of Health and Human Services

The Honorable George Tidmarsh, M.D., Ph.D, Director of the Center for Drug Evaluation and Research