# Congress of the United States
Washington, DC 20510

June 25, 2025

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

Dear Commissioner Makary:

On behalf of our constituents in the state of South Carolina impacted by Barth syndrome and other rare mitochondrial conditions, we write to request the FDA's 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 may be aware, elamipretide is the first and only treatment in clinical development for Barth syndrome, an ultra-rare, lethal, progressive genetic disorder with no known cure or FDA-approved treatment options. Elamipretide is also provided to patients with rare mitochondrial disorders under the Expanded Access Program (EAP). Barth syndrome is characterized by symptoms including severe and debilitating muscle weakness, exercise intolerance and fatigue, cardiomyopathy and cardiac dysfunction commonly leading to premature death, recurrent infections, feeding issues, and delayed growth. Those affected have a shortened life expectancy, with 85 percent of early deaths occurring by age five and most survivors of early childhood passing away before their forties.

Despite the exceedingly rare nature of Barth syndrome (1:1,000,000 male births), which impacts fewer than 150 known individuals in the United States, we are aware of individuals in the state of South Carolina living with Barth syndrome. Beyond those impacted by Barth syndrome, there are a number of individuals affected by rare mitochondrial disorders in South Carolina who are also impacted by a lack of access to elamipretide.

Our constituents have raised concerns about the recent announcement that the FDA did not approve elamipretide in this review cycle. We respectfully request the FDA provide our offices with clarity around elamipretide's review pathway moving forward, and we encourage the FDA to consider patient and physician testimony as part of its review process in accordance with existing rules and regulations. Given the early-onset nature of this

disease, as well as the successful research conducted on the effectiveness of this treatment, we strongly urge full, fair, and prompt consideration of elamipretide at the earliest opportunity that aligns with the safety guidelines and processes of the FDA.

Thank you for your attention to this important matter.

Sincerely,

Ralph Norman
Member of Congress

Sheri Biggs
Member of Congress

Joe Wilson
Member of Congress

Russell Fry
Member of Congress

Nancy Mace
Member of Congress

William R. Timmons, IV
Member of Congress

James E. Clyburn
Member of Congress