

MICHAEL GUEST  
3RD DISTRICT, MISSISSIPPI

450 CANNON HOUSE OFFICE BUILDING  
WASHINGTON, DC 20515  
(202) 225-5031

<https://guest.house.gov>

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**Congress of the United States**  
**House of Representatives**  
**Washington, DC 20515-2403**

July 24, 2025

The Honorable Martin Makary  
Commissioner of Food and Drugs  
US Food and Drug Administration  
10903 New Hampshire Ave  
Silver Spring, MD 20993-0002

Dear Commissioner Makary,

I am writing to respectfully request your attention to the ongoing review of elamipretide, a first-of-its-kind experimental medication aimed at treating Barth Syndrome.

As you are aware, elamipretide is the first and only treatment in clinical development for Barth Syndrome, an ultra-rare, progressive genetic disorder with no FDA-approved treatment option. Elamipretide is also provided to patients with rare mitochondrial disorders, such as Microphthalmia with Linear Skin Defects Syndrome (MLS Syndrome), 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, primarily males, have a shortened life expectancy, with 85% of early deaths occurring by age five, and most survivors of early childhood passing away before their 40s.

On behalf of my constituents living with Barth Syndrome, I respectfully request that the FDA clarify the regulatory path forward for elamipretide. Given the early-onset nature of this disease and the promising research on the effectiveness of elamipretide, the FDA's approval could be transformative and lifesaving. I understand that safety and efficacy are paramount, and I trust the agency's thorough and rigorous review process. However, I strongly urge you to consider elamipretide fairly and promptly at the earliest opportunity, in accordance with the FDA's safety guidelines and processes.

In accordance with all existing rules, regulations, and ethical guidelines, I appreciate your consideration and your continued engagement with my respective staff.

Sincerely,



Michael Guest  
Member of Congress

BRANDON  
308 B EAST GOVERNMENT STREET  
BRANDON, MS 39208  
(769) 241-6120

MERIDIAN  
200 22ND AVENUE  
MERIDIAN, MS 39301  
(601) 693-6681

STARKVILLE  
301 EAST MAIN STREET, SUITE 300  
STARKVILLE, MS 39759  
(662) 324-0007

BROOKHAVEN  
230 SOUTH WHITWORTH STREET  
BROOKHAVEN, MS 39601  
(601) 823-3400