STATE OF GEORGIA

OFFICE OF THE GOVERNOR

ATLANTA 30334-0900

Brian P. Kemp

GOVERNOR

August 8, 2025

Secretary Robert F. Kennedy, Jr.
U.S. Department of Health and Human Services
200 Independence Avenue, S.W.
Washington, D.C. 20201

RE: FDA Action on Elamipretide treatment for Barth Syndrome

Dear Secretary Kennedy:

We write today on behalf of concerned Georgians, especially those families with small children who live daily with Barth syndrome and similar conditions. As you know, Barth syndrome is an extremely rare disease that leads to severe muscle weakness, fatigue, heart failure, infections, and stunted growth. Those who suffer from Barth syndrome have dramatically lower life expectancies, with many patients dying before they reach the age of 5.

As you also know, **there are currently no approved therapies for Barth syndrome**.

A family enduring this painful situation within our state has notified us that the Federal Drug Administration (FDA) first began to consider the treatment known as Elamipretide -- which was developed by a company known as Stealth BioTherapeutics to treat Barth syndrome, mitochondrial myopathy, and related conditions – in January 2024. Last October, **an FDA committee determined that Elamipretide is an effective treatment** for patients with Barth syndrome, reaching the same conclusion expressed by numerous physicians, scientists, patient

advocates, and our constituents -- the parents of a young girl named Hope Filchak who currently takes this medication and has responded positively. Along with her distressed family members, we were discouraged to learn that in May of this year the FDA issued a letter rejecting Stealth's new drug application for Elamipretide. Stealth sought reconsideration of that denial, and we understand that its request was denied earlier this week, despite good-faith efforts to address concerns that were expressed by the FDA in connection with the May denial.

The FDA's rejection of this treatment will deny access to patients like Hope, many of whom are small children currently using this potentially life-changing medication under emergency access requests or following initial treatments in clinical trials. This upsetting decision which seems to disregard the strong supporting vote of the committee last October also provokes questions around the integrity of the agency's review processes, the transparency and clarity with which it communicates with stakeholders, and its alignment with the expressed desire to promote treatments for rare diseases, including your own statements to that effect in an FDA panel discussion on biotechnology innovation this past June. See https://www.reuters.com/business/healthcare-pharmaceuticals/us-health-secretary-kennedy-looks-fast-tracking-approvals-rare-disease-drugs-2025-06-05/.

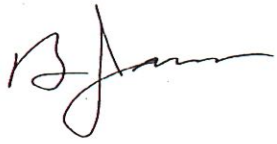
In short, this sudden denial cuts against broader goals President Trump and his administration have set in reforming the federal government's regulatory approach in a manner that fosters and encourages American innovation, especially in critical areas like healthcare and pharmaceutical research and development. It is our hope that your track record of removing barriers and streamlining such bureaucratic processes will help eliminate the slow, cumbersome, and overly complicated regulatory structure that is actively harming families like the Filchaks. In addition to the immediate consequences of this denial for patients like Hope, the FDA's action in this case will be discouraging to scientists and innovators who seek to develop effective treatments for rare diseases here in the United States.

Please note, Georgia's entire Congressional delegation sent a similar letter to Commissioner Martin Makary in June of this year to urge quick attention to this matter. Unfortunately, no action has been taken thus far.

For these reasons, we respectfully ask you to work with the FDA and encourage it to reconsider this action and allow Stealth to resubmit its application for approval on an expedited timeline. On behalf of a grateful state, thank you for your prompt attention to this matter and for your service to our country.

Sincerely,

Brian Kemp
83rd Governor of Georgia



Lt. Governor Burt Jones
President of the Georgia Senate

Speaker Jon Burns
Speaker of the Georgia House of Representatives

CC: Dr. Martin A. Makary
Commissioner of Food and Drugs
U.S. Food and Drug Administration
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