118TH CONGRESS
1ST SESSION

H. RES. 276

Expressing support for the designation of April 5, 2023, as ''Barth Syndrome Awareness Day''.

IN THE HOUSE OF REPRESENTATIVES

APRIL 3, 2023

Mr. TONKO (for himself, Mr. NORMAN, and Mr. BILIRAKIS) submitted the following resolution; which was referred to the Committee on Energy and Commerce

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RESOLUTION

Expressing support for the designation of April 5, 2023, as ''Barth Syndrome Awareness Day''.

Whereas Barth syndrome is a rare, life-threatening, genetic disorder which primarily affects males;

Whereas Barth syndrome is caused by a mutation in the tafazzin gene (TAFAZZIN, also called G4.5), resulting in an inborn error of phospholipid metabolism, affecting many systems of the body;

Whereas Barth syndrome is a multisystem disorder with characteristics including abnormalities of heart and skeletal muscle, low levels of certain white blood cells called neutrophils that help to fight bacterial infections, and delays in growth;

Whereas with probably fewer than 10 new Barth infants identified each year in the United States, the incidence of Barth syndrome is likely only 1 in every 300,000 to 400,000 births and could be as few as 1.5 in 1 million;

Whereas globally there are approximately 300 individuals diagnosed with Barth syndrome, and, in the United States, there are fewer than 150 individuals diagnosed with Barth syndrome;

Whereas Barth syndrome can be fatal in childhood due to heart failure or uncontrollable infection, with approximately 50 percent of deaths due to Barth syndrome occurring within the first year of life and 85 percent before the fifth year of life;

Whereas individuals with Barth syndrome who survive to adulthood often have a severely limited life expectancy;

Whereas individuals with Barth syndrome, like many other rare disorders, experience challenges with obtaining a diagnosis, limited treatment options, and difficulty finding and accessing treatment centers and physicians with expertise in Barth syndrome;

Whereas because the disorder affects multiple systems of the body, a patient with Barth syndrome often requires access to care from experts across a range of medical specialties;

Whereas the Barth Syndrome Clinic at Kennedy Krieger Institute is the only interdisciplinary clinic dedicated to the diagnosis and treatment of Barth syndrome in the United States;

Whereas, to date, there is no treatment for Barth syndrome that is approved by the Food and Drug Administration (FDA);

Whereas there is a critical need for research and development to advance treatments for Barth syndrome;

Whereas, as a result of the Orphan Drug Act, there have been important advances in research on and treatment for rare diseases, including development efforts in Barth syndrome;

Whereas the FDA established the Accelerated Approval Pathway in 1992 and Congress codified the pathway in 2012;

Whereas the Accelerated Approval Pathway is an important pathway for rare and ultrarare diseases as it allows for ''earlier approval of drugs that treat serious conditions, and fill an unmet medical need. . .'';

Whereas Congress and the FDA have affirmed the importance of incorporating the patient perspective throughout the drug review process through the FDA's Patient-Focused Drug Development program;

Whereas the Barth Syndrome Foundation, a nonprofit organization established in 2000 to accelerate progress through collaboration between families and scientists, is dedicated to educating, advancing treatments, and finding a cure for Barth syndrome;

Whereas the Barth Syndrome Foundation sponsors ''Barth Syndrome Awareness Day'' in the United States to increase public awareness and generate additional support for Barth syndrome; and

Whereas ''Barth Syndrome Awareness Day'' is expected to be observed in the United States for years to come, providing hope and information for patients, caregivers, and families around the country: Now, therefore, be it

Resolved, That the House of Representatives—

(1) supports the designation of ''Barth Syndrome Awareness Day''; and

(2) recognizes the importance of, with respect to Barth syndrome—

(A) improving awareness;

(B) encouraging accurate and early diagnosis;

(C) advancing research;

(D) developing new treatments, diagnostics, and cures; and

(E) identifying regulatory pathways for drug development of ultrarare diseases like Barth syndrome.

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