117th CONGRESS

1st Session

S. RES. XXX

Designating September 15, 2021 as "International Myotonic Dystrophy Awareness Day" and supporting the goals and ideals of International Myotonic Dystrophy Awareness Day.

IN THE SENATE OF THE UNITED STATES

INSERT DATE

SENATE RESOLUTION SPONSOR NAME (for herself/himself, SENATOR XX, SENATOR XX) submitted the following resolution; which was considered and agreed to

RESOLUTION

Designating September 15, 2021 as "International Myotonic Dystrophy Awareness Day" and supporting the goals and ideals of International Myotonic Dystrophy Awareness Day.

Whereas myotonic dystrophy is a rare, multi-systemic, inherited disease that affects **approximately 1 in 2,100 people or 150,000 individuals in the United States.** [1] Whereas there are well over a million people living with DM globally, yet thousands of people do not know they have the disease and are in need of care.

Whereas **myotonic dystrophy is the most common form of adult muscular dystrophy and considered the most variable of all known conditions**. [2] The symptoms become more severe with each generation (known as anticipation), yet there is currently no cure and there are no approved treatments. [74-89]

Whereas the disease is caused by a mutation in the **DMPK gene, resulting in myotonic dystrophy type one, and the CNBP gene, resulting in myotonic dystrophy type two**. These mutations **prevent the genes from carrying out their functions properly**, impacting multiple body systems. [3-31]

Whereas the genetic mutation is an autosomal dominant mutation, where one copy of the altered gene is sufficient to cause the disorder. As a result, affected individuals have a 50% chance of passing on the

mutated gene to their children. A child is equally likely to have inherited the mutated gene from either parent. If both parents do not have the disease, their children cannot inherit it. [8]

Whereas through this inherited genetic anomaly, **individuals with myotonic dystrophy experience varied and complex symptoms, from skeletal muscle problems [91-112], to heart [113-126], breathing [127-150], digestive [151-166], hormonal [198-219], speech and swallowing [246], diabetic [247], immune [220-237], excessive daytime sleepiness [248], early cataracts and vision [238-245], and cognitive difficulties [167-197].**

Whereas myotonic dystrophy is a highly variable and complicated disorder. The systems affected, the severity of symptoms, and the age of onset of those symptoms vary greatly between individuals, even in the same family. In general, the younger an individual is when symptoms first appear, the more severe symptoms are likely to be.

Whereas a complete diagnostic evaluation, which includes family history, physical examination, and medical tests, is typically required for a presumptive diagnosis of myotonic dystrophy. The presence of the disorder can then be confirmed by genetic testing. Prenatal testing, where the DNA of the fetus is checked for the presence of the myotonic dystrophy mutation, is also available. Despite the availability of simple genetic tests, misdiagnoses persist for decades. [249-250]

Whereas delays in diagnosing myotonic dystrophy are common. This is usually because of the lack of familiarity with the disease on the part of clinicians and that more common diseases with symptoms that mimic myotonic dystrophy must typically first be ruled out before this disorder is considered.

Whereas there are currently no Food and Drug Administration approved treatments for myotonic dystrophy, however, many biopharmaceutical companies are leading promising trials which may lead to treatments for the disease. [251]

Whereas the Myotonic Dystrophy Foundation was founded in 2007 with a mission to enhance the quality of life of people living with myotonic dystrophy and accelerate research focused on finding treatments and a cure. It is the leading global advocate helping patients and families navigate the myotonic dystrophy disease process and is often the first resource contacted by newly diagnosed patients, their families, their social workers and their physicians around the world.

Whereas in 2014, Congress reauthorized the MD-CARE Act which has increased muscular dystrophy research funding and public health surveillance activities including for myotonic dystrophy. Further this law funds the University of Rochester Medical Center's Paul D. Wellstone Muscular Dystrophy Cooperative Research Center which is an internationally recognized research program.

Whereas on September 15, 2016, the Myotonic Dystrophy Foundation hosted the first ever myotonic dystrophy focused Patient-Focused Drug Development meeting with FDA senior leadership that raised awareness of patient and caregiver perspectives as part of an initiative to drive biopharmaceutical discovery. This led to the publication and delivery to the FDA of the *Myotonic Dystrophy Voice of the Patient* report on April 14, 2017.[252]

Whereas in September 2017, recognizing the seriousness of the disease and its especially disabling impact on persons with congenital myotonic dystrophy, the Social Security Administration added the congenital form of the disease to the Compassionate Allowance Program which allows individuals to quickly qualify for disability benefits including health insurance coverage.

Whereas the United States Senate added myotonic dystrophy to list of eligible conditions for research funding under the Department of Defense Peer Reviewed Medical Research Program in 2018, which has resulted in over $6 million in new research awards.

Whereas myotonic dystrophy research funding supported by the National Institutes of Health has remained flat over the past decade with the agency estimating awarding $11 million in research grants in fiscal year 2021.

Whereas increased federal funding for myotonic dystrophy research will improve health outcomes, reduce disability, and increase life expectancy for individuals living with disease, and holds great promise for helping individuals with similar genetic diseases like Fragile X syndrome and Huntington's disease.

Resolved, That the Senate—

(1) designates September 15, 2021 as "International Myotonic Dystrophy Awareness Day";

(2) supports the goals and ideals of International Myotonic Dystrophy Awareness Day including;

(3) committing to promoting and advancing the health, well-being, and inherent dignity of all children and adults with myotonic dystrophy;

(4) supporting the advancement of scientific and medical myotonic dystrophy research at the National Institutes of Health and as part of the Department of Defense Peer Reviewed Medical Research Program;

(5) fostering biopharmaceutical innovation that will lead to FDA approved treatments and eventually a cure for myotonic dystrophy;

(6) advancing programs and policies that assist individuals disabled by myotonic dystrophy and their caregivers; and

(6) encouraging awareness and education regarding myotonic dystrophy among patients, caregivers, clinicians, and researchers.

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