110TH CONGRESS
2D SESSION

H. R. 5265

IN THE SENATE OF THE UNITED STATES

SEPTEMBER 25 (legislative day, SEPTEMBER 17), 2008
Received

AN ACT

To amend the Public Health Service Act to provide for research with respect to various forms of muscular dystrophy, including Becker, congenital, distal, Duchenne, Emery-Dreifuss facioscapulohumeral, limb-girdle, myotonic, and oculopharyngeal, muscular dystrophies.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2008''.

SEC. 2. FINDINGS.

The Congress finds as follows:

(1) The muscular dystrophies are devastating diseases that have a significant impact on quality of life—not only for the individual who experiences its painful symptoms and resulting disability, but also for family members and caregivers.

(2) DMD is the most common lethal genetic disorder of childhood worldwide, affecting approximately 1 in every 3,500 boys born each year around the globe. It is characterized by a rapidly progressive muscle weakness that almost always results in death from respiratory or cardiac failure, typically in the late teens or twenties.

(3) Myotonic muscular dystrophy is the second most prominent form of muscular dystrophy and the type most commonly found in adults affecting an estimated 1 in 8,000 people. However, it can affect people of any age—from birth to old age. Described as the most variable disease known in medicine, it is multi-systemic and can cause not only muscle atrophy and myotonia, but also serious cardiac, res-

piratory, endocrine, gastrointestinal, skeletal and central nervous system complications, as well as problems with the eyes, teeth and hair. As it passes from one generation to the next, it generally worsens with earlier onset. Congenital myotonic muscular dystrophy is the most severe form of myotonic muscular dystrophy affecting infants and causing severe cognitive delays. It often causes sudden death; however, others can live for many years with this slowly degenerative disorder.

(4) Facioscapulohumeral muscular dystrophy (referred to in this section as ''FSHD'') is the second most prevalent adult muscular dystrophy and the third most prevalent muscular dystrophy of men, women and children. It is inherited genetically and has an estimated incidence of 1 in 20,000 persons. Many leading FSHD scientists note that the prevalence may be three times higher due to undiagnosed and misdiagnosed cases. FSHD, affecting between 15,000 to 40,000 persons, causes a lifelong progressive and severe loss of all skeletal muscles gradually bringing weakness and reduced mobility. It is genetically transmitted to children, can occur spontaneously, and may affect entire families. Persons with FSHD may also experience hearing loss, vision prob-

lems and respiratory insufficiency; some may become severely physically disabled and spend decades in a wheelchair and on a ventilator. FSHD is caused by a novel epigenetic phenomenon not found in other forms of muscular dystrophy and is caused by a contraction of repetitive DNA previously thought to be ''junk DNA''. The unique epigenetic structure of FSHD is unprecedented in other muscular dystrophies and genetic disorders and demands novel approaches and new research groups. Understanding this mechanism will have great benefit to other areas of biomedical research including cancer and other disease of epigenetic origin.

(5) Congenital muscular dystrophies represent a group of distinct diseases, which begin at birth, with varying severity and involvement of both muscle strength and brain. These diseases often lead to premature infant death, or severely disabled young children who require 24-hour care given their developmental delay compounded by muscle weakness. Other children live to young adulthood and typically require the use of a wheelchair for mobility.

(6) Forms of muscular dystrophy affecting children and adults include Becker, congenital, distal, Duchenne, Emery-Dreifuss, facioscapulohumeral,

limb-girdle, myotonic, and oculopharyngeal muscular dystrophies. The limb-girdle muscular dystrophies are of 15 known different types.

(7) Each of the muscular dystrophies, though distinct in progressivity and severity of symptoms, has a devastating impact on hundreds of thousands of children and adults throughout the United States and worldwide, as well as imposes severe physical and economic burdens on those affected. In many of the muscular dystrophies, there are associated medical problems arising from pulmonary issues, respiratory insufficiency, cardiomyopathy, which in many cases is the cause of death for persons with muscular dystrophy.

(8) In the 5 years since enactment of the Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001 (MD–CARE Act) and due directly to the momentum established by the MD–CARE Act, progress has been made in the battle against the Muscular Dystrophies.

(9) Investments made by the Federal Government as a result of the MD–CARE Act include the creation of the MD Coordinating Committee (MDCC), the development of the MDCC Action Plan, establishment of 6 Paul D. Wellstone Mus-

cular Dystrophy Cooperative Research Centers (co-funded, in part, by a national non-profit health organization), development of the Muscular Dystrophy Surveillance, Tracking and Research Network (MD STARnet), and the launch of a comprehensive education and outreach initiative.

(10) In the past few years, the NIH program in translational research in muscular dystrophy has grown significantly and funded a number of large-scale projects to further the development of therapies for muscular dystrophy. As part of this program, the National Institute of Neurological Disorders and Stroke (NINDS) and the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) of the National Institutes of Health (NIH) awarded a $15.4 million, five-year cooperative agreement to develop new small molecule drugs for the treatment of Duchenne muscular dystrophy (DMD) and potentially other forms of muscular dystrophy as well. The project is a unique research collaboration between private, public, and non-profit partners to build upon previous research and discovery work originally initiated by non-profit partners to identify new treatments for muscular dystrophy. Also through the translational program,

three other major cooperative agreements have been awarded for highly targeted therapy development projects in the muscular dystrophies.

(11) Advancements in care have helped prolong life and quality of life for patients with muscular dystrophy.

(12) There remains a shortage of qualified researchers in the field of muscular dystrophy research. Many family physicians and health care professionals still lack the knowledge and resources to detect and properly diagnose muscular dystrophy as early as possible, thus delaying management of symptoms in cases that go undetected or misdiagnosed.

(13) As new understandings of the genetic basis for disease and potential treatment has emerged, the public and health care communities are in urgent need of education and outreach to ensure competent, informed engagement in genetic testing and counseling and appropriate patient characterization so that patients are able to participate in new avenues of research and clinical trials.

(14) As basic research into the muscular dystrophies points the way to new therapeutic targets, there is an urgent need to support the clinical re-

search infrastructure necessary to bring these therapeutic leads to human trials; these infrastructure needs include validated endpoints, current natural history studies, biomarkers, clinical research networks, patient registries and databases.

(15) In order to improve lives and develop effective treatments for individuals with muscular dystrophy, there must be improved communications and partnerships between patients, patient advocacy, researchers, and clinical care providers. To that end, renewed effort to work together by all parties is a critical element for successful outcomes in the years to come.

(16) Continued focus and investment are required to build on the current momentum, respond to public need, and ensure that research and other innovation is translated to therapeutic targets as quickly as possible.

SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINATION OF ACTIVITIES OF NIH WITH RESPECT TO RESEARCH ON MUSCULAR DYSTROPHY.

(a) TECHNICAL CORRECTION.—Section 404E of the Public Health Service Act (42 U.S.C. 283g) is amended by striking subsection (f) (relating to reports to Congress) and redesignating subsection (g) as subsection (f).

(b) AMENDMENTS.—Section 404E of the Public Health Service Act (42 U.S.C. 283g) is amended—

(1) in subsection (a)(1), by inserting ''the National Heart, Lung, and Blood Institute,'' after ''the Eunice Kennedy Shriver National Institute of Child Health and Human Development,'';

(2) in subsection (b)(1), by adding at the end of the following: ''Such centers of excellence shall be known as the 'Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers'.''; and

(3) by adding at the end the following:

''(g) CLINICAL RESEARCH.—The Coordinating Committee may evaluate the potential need to enhance the clinical research infrastructure required to test emerging therapies for the various forms of muscular dystrophy by prioritizing the achievement of the goals related to this topic in the plan under subsection (e)(1).''.

SEC. 4. DEVELOPMENT AND EXPANSION OF ACTIVITIES OF CDC WITH RESPECT TO EPIDEMIOLOGICAL RESEARCH ON MUSCULAR DYSTROPHY.

Section 317Q of the Public Health Service Act (42 U.S.C. 247b–18) is amended—

(1) by redesignating subsection (d) as subsection (f); and

(2) by inserting after subsection (c) the following:

''(d) DATA.—In carrying out this section, the Secretary shall ensure that any data on patients that is collected as part of the Muscular Dystrophy STARnet (under a grant under this section) is regularly updated to reflect changes in patient condition over time.

''(e) REPORTS AND STUDY.—

''(1) ANNUAL REPORT.—Not later than 18 months after the date of the enactment of the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2008, and annually thereafter, the Director of the Centers for Disease Control and Prevention shall submit to the appropriate committees of the Congress a report—

''(A) concerning the activities carried out by MD STARnet site funded under this section during the year for which the report is prepared;

''(B) containing the data collected and findings derived from the MD STARnet sites each fiscal year (as funded under a grant under this section during fiscal years 2008 through 2012); and

''(C) that every 2 years outlines prospective data collection objectives and strategies.

''(2) TRACKING HEALTH OUTCOMES.—The Director of the Centers for Disease Control and Prevention shall provide health outcome data on the health and survival of people with muscular dystrophy.''.

SEC. 5. INFORMATION AND EDUCATION.

Section 5 of the Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001 (42 U.S.C. 247b–19) is amended—

(1) by redesignating subsection (c) as subsection (d); and

(2) by inserting after subsection (b) the following:

''(c) REQUIREMENTS OF CDC.—In carrying out this section, the Director of the Centers for Disease Control and Prevention shall—

''(1) partner with leaders in the muscular dystrophy patient community; and

''(2) widely disseminate the Duchenne-Becker muscular dystrophy care considerations as broadly as possible, including through partnership opportunities with the muscular dystrophy patient community.''.

SEC. 6. STANDARDS OF CARE.

Part A of title IX of the Public Health Service Act (42 U.S.C. 299 et seq.) is amended by adding at the end the following:

"SEC. 904. STANDARDS OF CARE RELATING TO MUSCULAR DYSTROPHY.

''The Director—

''(1) shall evaluate the available scientific evidence for the appropriate medical or patient organizations for purposes of the development and issuance of an initial set of care considerations for Duchenne-Becker muscular dystrophy and provide periodic review and updates where appropriate; and

''(2) may replicate the same methodology used to develop the Duchenne-Becker muscular dystrophy care considerations developed under paragraph (1) as a model for other muscular dystrophies.''.

Passed the House of Representatives September 24, 2008.

Attest: LORRAINE C. MILLER,

Clerk.