

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.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

1 **SECTION 1. SHORT TITLE.**

2 This Act may be cited as the “Paul D. Wellstone
3 Muscular Dystrophy Community Assistance, Research,
4 and Education Amendments of 2008”.

5 **SEC. 2. FINDINGS.**

6 The Congress finds as follows:

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

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

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

1 piratory, endocrine, gastrointestinal, skeletal and
2 central nervous system complications, as well as
3 problems with the eyes, teeth and hair. As it passes
4 from one generation to the next, it generally worsens
5 with earlier onset. Congenital myotonic muscular
6 dystrophy is the most severe form of myotonic mus-
7 cular dystrophy affecting infants and causing severe
8 cognitive delays. It often causes sudden death; how-
9 ever, others can live for many years with this slowly
10 degenerative disorder.

11 (4) Facioscapulohumeral muscular dystrophy
12 (referred to in this section as “FSHD”) is the sec-
13 ond most prevalent adult muscular dystrophy and
14 the third most prevalent muscular dystrophy of men,
15 women and children. It is inherited genetically and
16 has an estimated incidence of 1 in 20,000 persons.
17 Many leading FSHD scientists note that the preva-
18 lence may be three times higher due to undiagnosed
19 and misdiagnosed cases. FSHD, affecting between
20 15,000 to 40,000 persons, causes a lifelong progres-
21 sive and severe loss of all skeletal muscles gradually
22 bringing weakness and reduced mobility. It is geneti-
23 cally transmitted to children, can occur spontane-
24 ously, and may affect entire families. Persons with
25 FSHD may also experience hearing loss, vision prob-

1 lems and respiratory insufficiency; some may become
2 severely physically disabled and spend decades in a
3 wheelchair and on a ventilator. FSHD is caused by
4 a novel epigenetic phenomenon not found in other
5 forms of muscular dystrophy and is caused by a con-
6 traction of repetitive DNA previously thought to be
7 “junk DNA”. The unique epigenetic structure of
8 FSHD is unprecedented in other muscular dys-
9 trophies and genetic disorders and demands novel
10 approaches and new research groups. Understanding
11 this mechanism will have great benefit to other areas
12 of biomedical research including cancer and other
13 disease of epigenetic origin.

14 (5) Congenital muscular dystrophies represent a
15 group of distinct diseases, which begin at birth, with
16 varying severity and involvement of both muscle
17 strength and brain. These diseases often lead to pre-
18 mature infant death, or severely disabled young chil-
19 dren who require 24-hour care given their develop-
20 mental delay compounded by muscle weakness.
21 Other children live to young adulthood and typically
22 require the use of a wheelchair for mobility.

23 (6) Forms of muscular dystrophy affecting chil-
24 dren and adults include Becker, congenital, distal,
25 Duchenne, Emery-Dreifuss, facioscapulohumeral,

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

4 (7) Each of the muscular dystrophies, though
5 distinct in progressivity and severity of symptoms,
6 has a devastating impact on hundreds of thousands
7 of children and adults throughout the United States
8 and worldwide, as well as imposes severe physical
9 and economic burdens on those affected. In many of
10 the muscular dystrophies, there are associated med-
11 ical problems arising from pulmonary issues, res-
12 piratory insufficiency, cardiomyopathy, which in
13 many cases is the cause of death for persons with
14 muscular dystrophy.

15 (8) In the 5 years since enactment of the Mus-
16 cular Dystrophy Community Assistance, Research
17 and Education Amendments of 2001 (MD-CARE
18 Act) and due directly to the momentum established
19 by the MD-CARE Act, progress has been made in
20 the battle against the Muscular Dystrophies.

21 (9) Investments made by the Federal Govern-
22 ment as a result of the MD-CARE Act include the
23 creation of the MD Coordinating Committee
24 (MDCC), the development of the MDCC Action
25 Plan, establishment of 6 Paul D. Wellstone Mus-

1 cular Dystrophy Cooperative Research Centers (co-
2 funded, in part, by a national non-profit health orga-
3 nization), development of the Muscular Dystrophy
4 Surveillance, Tracking and Research Network (MD
5 STARnet), and the launch of a comprehensive edu-
6 cation and outreach initiative.

7 (10) In the past few years, the NIH program
8 in translational research in muscular dystrophy has
9 grown significantly and funded a number of large-
10 scale projects to further the development of thera-
11 pies for muscular dystrophy. As part of this pro-
12 gram, the National Institute of Neurological Dis-
13 orders and Stroke (NINDS) and the National Insti-
14 tute of Arthritis and Musculoskeletal and Skin Dis-
15 eases (NIAMS) of the National Institutes of Health
16 (NIH) awarded a \$15.4 million, five-year cooperative
17 agreement to develop new small molecule drugs for
18 the treatment of Duchenne muscular dystrophy
19 (DMD) and potentially other forms of muscular dys-
20 trophy as well. The project is a unique research col-
21 laboration between private, public, and non-profit
22 partners to build upon previous research and dis-
23 covery work originally initiated by non-profit part-
24 ners to identify new treatments for muscular dys-
25 trophy. Also through the translational program,

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

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

7 (12) There remains a shortage of qualified re-
8 searchers in the field of muscular dystrophy re-
9 search. Many family physicians and health care pro-
10 fessionals still lack the knowledge and resources to
11 detect and properly diagnose muscular dystrophy as
12 early as possible, thus delaying management of
13 symptoms in cases that go undetected or
14 misdiagnosed.

15 (13) As new understandings of the genetic basis
16 for disease and potential treatment has emerged, the
17 public and health care communities are in urgent
18 need of education and outreach to ensure competent,
19 informed engagement in genetic testing and coun-
20 seling and appropriate patient characterization so
21 that patients are able to participate in new avenues
22 of research and clinical trials.

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

1 search infrastructure necessary to bring these thera-
2 peutic leads to human trials; these infrastructure
3 needs include validated endpoints, current natural
4 history studies, biomarkers, clinical research net-
5 works, patient registries and databases.

6 (15) In order to improve lives and develop effec-
7 tive treatments for individuals with muscular dys-
8 trophy, there must be improved communications and
9 partnerships between patients, patient advocacy, re-
10 searchers, and clinical care providers. To that end,
11 renewed effort to work together by all parties is a
12 critical element for successful outcomes in the years
13 to come.

14 (16) Continued focus and investment are re-
15 quired to build on the current momentum, respond
16 to public need, and ensure that research and other
17 innovation is translated to therapeutic targets as
18 quickly as possible.

19 **SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINA-**
20 **TION OF ACTIVITIES OF NIH WITH RESPECT**
21 **TO RESEARCH ON MUSCULAR DYSTROPHY.**

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

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

3 (1) in subsection (a)(1), by inserting “the Na-
 4 tional Heart, Lung, and Blood Institute,” after “the
 5 Eunice Kennedy Shriver National Institute of Child
 6 Health and Human Development,”;

7 (2) in subsection (b)(1), by adding at the end
 8 of the following: “Such centers of excellence shall be
 9 known as the ‘Paul D. Wellstone Muscular Dys-
 10 trophy Cooperative Research Centers’.”; and

11 (3) by adding at the end the following:

12 “(g) CLINICAL RESEARCH.—The Coordinating Com-
 13 mittee may evaluate the potential need to enhance the clin-
 14 ical research infrastructure required to test emerging
 15 therapies for the various forms of muscular dystrophy by
 16 prioritizing the achievement of the goals related to this
 17 topic in the plan under subsection (e)(1).”.

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

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

23 (1) by redesignating subsection (d) as sub-
 24 section (f); and

1 (2) by inserting after subsection (c) the fol-
2 lowing:

3 “(d) DATA.—In carrying out this section, the Sec-
4 retary shall ensure that any data on patients that is col-
5 lected as part of the Muscular Dystrophy STARnet (under
6 a grant under this section) is regularly updated to reflect
7 changes in patient condition over time.

8 “(e) REPORTS AND STUDY.—

9 “(1) ANNUAL REPORT.—Not later than 18
10 months after the date of the enactment of the Paul
11 D. Wellstone Muscular Dystrophy Community As-
12 sistance, Research, and Education Amendments of
13 2008, and annually thereafter, the Director of the
14 Centers for Disease Control and Prevention shall
15 submit to the appropriate committees of the Con-
16 gress a report—

17 “(A) concerning the activities carried out
18 by MD STARnet site funded under this section
19 during the year for which the report is pre-
20 pared;

21 “(B) containing the data collected and
22 findings derived from the MD STARnet sites
23 each fiscal year (as funded under a grant under
24 this section during fiscal years 2008 through
25 2012); and

1 “(C) that every 2 years outlines prospec-
2 tive data collection objectives and strategies.

3 “(2) TRACKING HEALTH OUTCOMES.—The Di-
4 rector of the Centers for Disease Control and Pre-
5 vention shall provide health outcome data on the
6 health and survival of people with muscular dys-
7 trophy.”.

8 **SEC. 5. INFORMATION AND EDUCATION.**

9 Section 5 of the Muscular Dystrophy Community As-
10 sistance, Research and Education Amendments of 2001
11 (42 U.S.C. 247b–19) is amended—

12 (1) by redesignating subsection (c) as sub-
13 section (d); and

14 (2) by inserting after subsection (b) the fol-
15 lowing:

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

19 “(1) partner with leaders in the muscular dys-
20 trophy patient community; and

21 “(2) widely disseminate the Duchenne-Becker
22 muscular dystrophy care considerations as broadly
23 as possible, including through partnership opportuni-
24 ties with the muscular dystrophy patient commu-
25 nity.”.

1 **SEC. 6. STANDARDS OF CARE.**

2 Part A of title IX of the Public Health Service Act
3 (42 U.S.C. 299 et seq.) is amended by adding at the end
4 the following:

5 **“SEC. 904. STANDARDS OF CARE RELATING TO MUSCULAR**
6 **DYSTROPHY.**

7 “The Director—

8 “(1) shall evaluate the available scientific evi-
9 dence for the appropriate medical or patient organi-
10 zations for purposes of the development and
11 issuance of an initial set of care considerations for
12 Duchenne-Becker muscular dystrophy and provide
13 periodic review and updates where appropriate; and

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

Passed the House of Representatives September 24,
2008.

Attest: LORRAINE C. MILLER,
Clerk.