

107TH CONGRESS
1ST SESSION

S. 805

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

IN THE SENATE OF THE UNITED STATES

MAY 1, 2001

Mr. WELLSTONE (for himself, Mr. COCHRAN, Ms. COLLINS, Mr. BENNETT, Mr. BREAUX, Mr. BUNNING, Mrs. CLINTON, Mr. CORZINE, Mr. DASCHLE, Mr. DAYTON, Mr. DORGAN, Mr. HUTCHINSON, Mr. JOHNSON, Mr. KERRY, Mr. KOHL, Ms. MIKULSKI, Mr. SARBANES, Mr. SCHUMER, Ms. SNOWE, Ms. STABENOW, and Mr. VOINOVICH) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Public Health Service Act to provide for research with respect to various forms of muscular dystrophy, including Duchenne, Becker, limb girdle, congenital, facioscapulohumeral, myotonic, oculopharyngeal, distal, and emery-dreifuss 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 “Muscular Dystrophy
3 Community Assistance, Research and Education Amend-
4 ments of 2001”, or the “MD–CARE Act”.

5 **SEC. 2. FINDINGS.**

6 Congress makes the following findings:

7 (1) Of the childhood muscular dystrophies,
8 Duchenne Muscular Dystrophy (DMD) is the
9 world’s most common and catastrophic form of ge-
10 netic childhood disease, and is characterized by a
11 rapidly progressive muscle weakness that almost al-
12 ways results in death, usually by 20 years of age.

13 (2) Duchenne muscular dystrophy is genetically
14 inherited, and mothers are the carriers in approxi-
15 mately 70 percent of all cases.

16 (3) If a female is a carrier of the dystrophin
17 gene, there is a 50 percent chance per birth that her
18 male offspring will have Duchenne muscular dys-
19 trophy, and a 50 percent chance per birth that her
20 female offspring will be carriers.

21 (4) Duchenne is the most common lethal ge-
22 netic disorder of childhood worldwide, affecting ap-
23 proximately 1 in every 3,500 boys worldwide.

24 (5) Children with muscular dystrophy exhibit
25 extreme symptoms of weakness, delay in walking,
26 waddling gait, difficulty in climbing stairs, and pro-

1 gressive mobility problems often in combination with
2 muscle hypertrophy.

3 (6) Other forms of muscular dystrophy affect-
4 ing children and adults include Becker, limb girdle,
5 congenital, facioscapulohumeral, myotonic,
6 oculopharyngeal, distal, and emery-dreifuss muscular
7 dystrophies.

8 (7) Myotonic muscular dystrophy (also known
9 as Steinert's disease and dystrophia myotonica) is
10 the second most prominent form of muscular dys-
11 trophy and the type most commonly found in adults.
12 Unlike any of the other muscular dystrophies, the
13 muscle weakness is accompanied by myotonia (de-
14 layed relaxation of muscles after contraction) and by
15 a variety of abnormalities in addition to those of
16 muscle.

17 (8) Facioscapulohumeral muscular dystrophy
18 (referred to in this section as "FSHD") is a neuro-
19 muscular disorder that is inherited genetically and
20 has an estimated frequency of 1 in 20,000. FSHD,
21 affecting between 15,000 to 40,000 persons, causes
22 a progressive and severe loss of skeletal muscle
23 gradually bringing weakness and reduced mobility.
24 Many persons with FSHD become severely phys-

1 ically disabled and spend many decades in a wheel-
2 chair.

3 (9) FSHD is regarded as a novel genetic phe-
4 nomenon resulting from a crossover of subtelomeric
5 DNA and may be the only human disease caused by
6 a deletion-mutation.

7 (10) Each of the muscular dystrophies, though
8 distinct in progressivity and severity of symptoms,
9 have a devastating impact on tens of thousands of
10 children and adults throughout the United States
11 and worldwide and impose severe physical and eco-
12 nomic burdens on those affected.

13 (11) Muscular dystrophies have a significant
14 impact on quality of life—not only for the individual
15 who experiences its painful symptoms and resulting
16 disability, but also for family members and care-
17 givers.

18 (12) Development of therapies for these dis-
19 orders, while realistic with recent advances in re-
20 search, is likely to require costly investments and in-
21 frastructure to support gene and other therapies.

22 (13) There is a shortage of qualified research-
23 ers in the field of neuromuscular research.

24 (14) Many family physicians and health care
25 professionals lack the knowledge and resources to

1 detect and properly diagnose the disease as early as
2 possible, thus exacerbating the progressiveness of
3 symptoms in cases that go undetected or
4 misdiagnosed.

5 (15) There is a need for efficient mechanisms
6 to translate clinically relevant findings in muscular
7 dystrophy research from basic science to applied
8 work.

9 (16) Educating the public and health care com-
10 munity throughout the country about this dev-
11 astating disease is of paramount importance and is
12 in every respect in the public interest and to the
13 benefit of all communities.

14 **SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINA-**
15 **TION OF ACTIVITIES OF NATIONAL INSTI-**
16 **TUTES OF HEALTH WITH RESPECT TO RE-**
17 **SEARCH ON MUSCULAR DYSTROPHY.**

18 Part A of title IV of the Public Health Service Act
19 (42 U.S.C. 281 et seq.) is amended by adding at the end
20 the following:

21 **“SEC. 404E. MUSCULAR DYSTROPHY; INITIATIVE THROUGH**
22 **DIRECTOR OF NATIONAL INSTITUTES OF**
23 **HEALTH.**

24 **“(a) EXPANSION, INTENSIFICATION, AND COORDINA-**
25 **TION OF ACTIVITIES.—**

1 “(1) IN GENERAL.—The Director of NIH, in
2 coordination with the Directors of the National In-
3 stitute of Neurological Disorders and Stroke, the
4 National Institute of Arthritis and Musculoskeletal
5 and Skin Diseases, the National Institute of Child
6 Health and Human Development, and the other Na-
7 tional Institutes of Health Institutes as appropriate,
8 shall expand and intensify programs of such Insti-
9 tutes with respect to research and related activities
10 concerning various forms of muscular dystrophy, in-
11 cluding Duchenne, myotonic, Facioscapulohumeral
12 muscular dystrophy (referred to in this section as
13 ‘FSHD’) and other forms of muscular dystrophy.

14 “(2) COORDINATION.—The Directors referred
15 to in paragraph (1) shall jointly coordinate the pro-
16 grams referred to in such paragraph and consult
17 with the Muscular Dystrophy Interagency Coordi-
18 nating Committee established under section 6 of the
19 MD–CARE Act.

20 “(3) ALLOCATIONS BY DIRECTOR OF NIH.—The
21 Director of NIH shall allocate the amounts appro-
22 priated to carry out this section for each fiscal year
23 among the national research institutes referred to in
24 paragraph (1).

25 “(b) CENTERS OF EXCELLENCE.—

1 “(1) IN GENERAL.—The Director of NIH shall
2 award grants and contracts under subsection (a)(1)
3 to public or nonprofit private entities to pay all or
4 part of the cost of planning, establishing, improving,
5 and providing basic operating support for centers of
6 excellence regarding research on various forms of
7 muscular dystrophy.

8 “(2) RESEARCH.—Each center under para-
9 graph (1) shall supplement but not replace the es-
10 tablishment of a comprehensive research portfolio in
11 all the muscular dystrophies. As a whole, the centers
12 shall conduct basic and clinical research in all forms
13 of muscular dystrophy including early detection, di-
14 agnosis, prevention, and treatment, including the
15 fields of muscle biology, genetics, noninvasive imag-
16 ing, genetics, pharmacological and other therapies.

17 “(3) COORDINATION OF CENTERS; REPORTS.—
18 The Director of NIH—

19 “(A) shall, as appropriate, provide for the
20 coordination of information among centers
21 under paragraph (1) and ensure regular com-
22 munication between such centers; and

23 “(B) shall require the periodic preparation
24 of reports on the activities of the centers and
25 the submission of the reports to the Director.

1 “(4) ORGANIZATION OF CENTERS.—Each cen-
2 ter under paragraph (1) shall use the facilities of a
3 single institution, or be formed from a consortium of
4 cooperating institutions, meeting such requirements
5 as may be prescribed by the Director of NIH.

6 “(5) NUMBER OF CENTERS; DURATION OF SUP-
7 PORT.—

8 “(A) IN GENERAL.—The Director of NIH
9 shall provide for the establishment of not less
10 than 5 centers under paragraph (1).

11 “(B) DURATION.—Support for a center es-
12 tablished under paragraph (1) may be provided
13 under this section for a period of not to exceed
14 5 years. Such period may be extended for 1 or
15 more additional periods not exceeding 5 years if
16 the operations of such center have been re-
17 viewed by an appropriate technical and sci-
18 entific peer review group established by the Di-
19 rector of NIH and if such group has rec-
20 ommended to the Director that such period
21 should be extended.

22 “(c) FACILITATION OF RESEARCH.—The Director of
23 NIH shall provide for a program under subsection (a)(1)
24 under which samples of tissues and genetic materials that
25 are of use in research on muscular dystrophy are donated,

1 collected, preserved, and made available for such research.
2 The program shall be carried out in accordance with ac-
3 cepted scientific and medical standards for the donation,
4 collection, and preservation of such samples.

5 “(d) COORDINATING COMMITTEE.—

6 “(1) IN GENERAL.—The Secretary shall estab-
7 lish the Muscular Dystrophy Coordinating Com-
8 mittee (referred to in this section as the ‘Coordi-
9 nating Committee’) to coordinate activities across
10 the National Institutes and with other Federal
11 health programs and activities relating to the var-
12 ious forms of muscular dystrophy.

13 “(2) COMPOSITION.—The Coordinating Com-
14 mittee shall consist of not more than 15 members to
15 be appointed by the Secretary, of which—

16 “(A) $\frac{2}{3}$ of such members shall represent
17 governmental agencies, including the directors
18 or their designees of each of the national re-
19 search institutes involved in research with re-
20 spect to muscular dystrophy and representatives
21 of all other Federal departments and agencies
22 whose programs involve health functions or re-
23 sponsibilities relevant to such diseases, includ-
24 ing the Centers for Disease Control and Pre-
25 vention, the Health Resources and Services Ad-

1 ministration and the Food and Drug Adminis-
2 tration, and representatives of other govern-
3 mental agencies that serve children with mus-
4 cular dystrophy such as the Department of
5 Education and

6 “(B) $\frac{1}{3}$ of such members shall be public
7 members, including a broad cross section of
8 persons affected with muscular dystrophies in-
9 cluding parents or legal guardians, affected in-
10 dividuals, researchers, and clinicians.

11 Members appointed under subparagraph (B) shall
12 serve for a term of 3 years, and may serve for an
13 unlimited number of terms if reappointed.

14 “(3) CHAIR.—

15 “(A) IN GENERAL.—With respect to mus-
16 cular dystrophy, the Chair of the Coordinating
17 Committee shall serve as the principal advisor
18 to the Secretary, the Assistant Secretary for
19 Health, and the Director of NIH, and shall pro-
20 vide advice to the Director of the Centers for
21 Disease Control and Prevention, the Commis-
22 sioner of Food and Drugs, and to the heads of
23 other relevant agencies. The Coordinating Com-
24 mittee shall select the Chair for a term not to
25 exceed 2 years.

1 “(B) APPOINTMENT.—The Chair of the
2 Committee shall be appointed by and be directly
3 responsible to the Secretary.

4 “(4) ADMINISTRATIVE SUPPORT; TERMS OF
5 SERVICE; OTHER PROVISIONS.—The following shall
6 apply with respect to the Coordinating Committee:

7 “(A) The Coordinating Committee shall re-
8 ceive necessary and appropriate administrative
9 support from the Department of Health and
10 Human Services.

11 “(B) The Coordinating Committee shall
12 meet as appropriate as determined by the Sec-
13 retary, in consultation with the chair.

14 “(e) PLAN FOR HHS ACTIVITIES.—

15 “(1) IN GENERAL.—Not later than 1 year after
16 the date of enactment of this section, the Coordi-
17 nating Committee shall develop a plan for con-
18 ducting and supporting research and education on
19 muscular dystrophy through the national research
20 institutes and shall periodically review and revise the
21 plan. The plan shall—

22 “(A) provide for a broad range of research
23 and education activities relating to biomedical,
24 epidemiological, psychosocial, and rehabilitative

1 issues, including studies of the impact of such
2 diseases in rural and underserved communities;

3 “(B) identify priorities among the pro-
4 grams and activities of the National Institutes
5 of Health regarding such diseases; and

6 “(C) reflect input from a broad range of
7 scientists, patients, and advocacy groups.

8 “(2) CERTAIN ELEMENTS OF PLAN.—The plan
9 under paragraph (1) shall, with respect to each form
10 of muscular dystrophy, provide for the following as
11 appropriate:

12 “(A) Research to determine the reasons
13 underlying the incidence and prevalence of var-
14 ious forms of muscular dystrophy.

15 “(B) Basic research concerning the eti-
16 ology and genetic links of the disease and po-
17 tential causes of mutations.

18 “(C) The development of improved screen-
19 ing techniques.

20 “(D) Basic and clinical research for the
21 development and evaluation of new treatments,
22 including new biological agents.

23 “(E) Information and education programs
24 for health care professionals and the public.

1 “(f) REPORTS TO CONGRESS.—The Coordinating
2 Committee shall biennially submit to the Committee on
3 Commerce of the House of Representatives, and the Com-
4 mittee on Health, Education, Labor, and Pensions of the
5 Senate, a report that describes the research, education,
6 and other activities on muscular dystrophy being con-
7 ducted or supported through the Department of Health
8 and Human Services. Each such report shall include the
9 following:

10 “(1) The plan under subsection (e)(1) (or revi-
11 sions to the plan, as the case may be).

12 “(2) Provisions specifying the amounts ex-
13 pended by the Department of Health and Human
14 Services with respect to various forms of muscular
15 dystrophy, including Duchenne, myotonic, FSHD
16 and other forms of muscular dystrophy.

17 “(3) Provisions identifying particular projects
18 or types of projects that should in the future be con-
19 sidered by the national research institutes or other
20 entities in the field of research on all muscular dys-
21 trophies.

22 “(g) PUBLIC INPUT.—The Secretary shall, under
23 subsection (a)(1), provide for a means through which the
24 public can obtain information on the existing and planned
25 programs and activities of the Department of Health and

1 Human Services with respect to various forms of muscular
 2 dystrophy and through which the Secretary can receive
 3 comments from the public regarding such programs and
 4 activities.

5 “(h) AUTHORIZATION OF APPROPRIATIONS.—For the
 6 purpose of carrying out this section, there are authorized
 7 to be appropriated such sums as may be necessary for
 8 each of fiscal years 2002 through 2006. The authorization
 9 of appropriations established in the preceding sentence is
 10 in addition to any other authorization of appropriations
 11 that is available for conducting or supporting through the
 12 National Institutes of Health research and other activities
 13 with respect to muscular dystrophy.”.

14 **SEC. 4. DEVELOPMENT AND EXPANSION OF ACTIVITIES OF**
 15 **CENTERS FOR DISEASE CONTROL AND PRE-**
 16 **VENTION WITH RESPECT TO EPIDEMIOLOG-**
 17 **ICAL RESEARCH ON MUSCULAR DYSTROPHY.**

18 Part B of title III of the Public Health Service Act
 19 (42 U.S.C. 243 et seq.) is amended by inserting after sec-
 20 tion 317P the following:

21 **“SEC. 317Q. SURVEILLANCE AND RESEARCH REGARDING**
 22 **MUSCULAR DYSTROPHY.**

23 “(a) IN GENERAL.—The Secretary, acting through
 24 the Director of the Centers for Disease Control and Pre-
 25 vention, may award grants and cooperative agreements to

1 public or nonprofit private entities (including health de-
2 partments of States and political subdivisions of States,
3 and including universities and other educational entities)
4 for the collection, analysis, and reporting of data on
5 Duchenne and other forms of muscular dystrophy. In
6 making such awards, the Secretary may provide direct
7 technical assistance in lieu of cash.

8 “(b) NATIONAL MUSCULAR DYSTROPHY SURVEIL-
9 LANCE PROGRAM.—The Secretary, acting through the Di-
10 rector of the Centers for Disease Control and Prevention,
11 may award grants to public or nonprofit private entities
12 (including health departments of States and political sub-
13 divisions of States, and including universities and other
14 educational entities) for the conduct of a National Mus-
15 cular Dystrophy Surveillance Program. In making such
16 awards, the Secretary may provide direct technical assist-
17 ance in lieu of cash.

18 “(c) CENTERS OF EXCELLENCE IN MUSCULAR DYS-
19 TROPHY EPIDEMIOLOGY.—

20 “(1) IN GENERAL.—The Secretary, acting
21 through the Director of the Centers for Disease
22 Control and Prevention, shall establish not less than
23 3 regional centers of excellence in muscular dys-
24 trophy epidemiology for the purpose of collecting
25 and analyzing information on the number, incidence,

1 correlates, and symptoms of Duchenne and other
2 forms of muscular dystrophies.

3 “(2) RECIPIENTS OF AWARDS FOR ESTABLISH-
4 MENT OF CENTERS.—Centers under paragraph (1)
5 shall be established and operated through the award-
6 ing of grants or cooperative agreements to public or
7 nonprofit private entities (including health depart-
8 ments of States and political subdivisions of States,
9 and including universities and other educational en-
10 tities) that conduct research.

11 “(3) CERTAIN REQUIREMENTS.—An award for
12 a center under paragraph (1) may be made only if
13 the entity involved submits to the Secretary an ap-
14 plication containing such agreements and informa-
15 tion as the Secretary may require, including an
16 agreement that the center involved will operate in
17 accordance with the following:

18 “(A) The center will collect, analyze, and
19 report muscular dystrophy data according to
20 guidelines prescribed by the Director, after con-
21 sultation with relevant State and local public
22 health officials, private sector researchers, and
23 advocates for those with muscular dystrophy.

24 “(B) The center will assist with the devel-
25 opment and coordination of State and related

1 muscular dystrophy surveillance efforts within a
2 region.

3 “(C) The center will identify eligible cases
4 and controls through its surveillance systems
5 and conduct research into factors which may
6 cause muscular dystrophy.

7 “(D) The center will develop or extend an
8 area of special research expertise (including ge-
9 netics, immunology, and other relevant research
10 specialty areas).

11 “(d) DEFINITION.—In this title, the term ‘State’
12 means each of the several States, the District of Columbia,
13 the Commonwealth of Puerto Rico, American Samoa,
14 Guam, the Commonwealth of the Northern Mariana Is-
15 lands, the United States Virgin Islands, and the Trust
16 Territory of the Pacific Islands.

17 “(e) AUTHORIZATION OF APPROPRIATIONS.—There
18 are authorized to be appropriated such sums as may be
19 necessary to carry out this section.”.

20 **SEC. 5. INFORMATION AND EDUCATION.**

21 (a) IN GENERAL.—The Secretary of Health and
22 Human Services (referred to in this Act as the “Sec-
23 retary”) shall establish and implement a program to pro-
24 vide information and education on muscular dystrophy to
25 health professionals and the general public, including in-

1 formation and education on advances in the diagnosis and
2 treatment of muscular dystrophy and training and con-
3 tinuing education through programs for scientists, physi-
4 cians, medical students, and other health professionals
5 who provide care for patients with muscular dystrophy.

6 (b) STIPENDS.—The Secretary may use amounts
7 made available under this section provides stipends for
8 health professionals who are enrolled in training programs
9 under this section.

10 (c) AUTHORIZATION OF APPROPRIATIONS.—There
11 are authorized to be appropriated such sums as may be
12 necessary to carry out this section.

13 **SEC. 6. REPORT TO CONGRESS.**

14 Not later than January 1, 2003, and each January
15 1 thereafter, the Secretary shall prepare and submit to
16 the appropriate committees of Congress, a report con-
17 cerning the implementation of this title and the amend-
18 ments made by this Act.

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