

Calendar No. 208

107TH CONGRESS
1ST SESSION

H. R. 717

IN THE SENATE OF THE UNITED STATES

SEPTEMBER 25, 2001

Received; read twice and referred to the Committee on Health, Education,
Labor, and Pensions

OCTOBER 30, 2001

Reported by Mr. KENNEDY, with an amendment

[Insert the part printed in *italic*]

AN ACT

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,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “Muscular Dystrophy
5 Community Assistance, Research and Education Amend-
6 ments of 2001”, or the “MD–CARE Act”.

1 **SEC. 2. FINDINGS.**

2 Congress makes the following findings:

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

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

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

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

20 (5) Children with muscular dystrophy exhibit
21 extreme symptoms of weakness, delay in walking,
22 waddling gait, difficulty in climbing stairs, and pro-
23 gressive mobility problems often in combination with
24 muscle hypertrophy.

25 (6) Other forms of muscular dystrophy affect-
26 ing children and adults include Becker, limb girdle,

1 congenital, facioscapulohumeral, myotonic,
2 oculopharyngeal, distal, and Emery-Dreifuss mus-
3 cular dystrophies.

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

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

23 (9) FSHD is regarded as a novel genetic phe-
24 nomenon resulting from a crossover of subtelomeric

1 DNA and may be the only human disease caused by
2 a deletion-mutation.

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

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

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

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

20 (14) Many family physicians and health care
21 professionals lack the knowledge and resources to
22 detect and properly diagnose the disease as early as
23 possible, thus exacerbating the progressiveness of
24 symptoms in cases that go undetected or
25 misdiagnosed.

1 (15) There is a need for efficient mechanisms
2 to translate clinically relevant findings in muscular
3 dystrophy research from basic science to applied
4 work.

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

10 **SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINA-**
11 **TION OF ACTIVITIES OF NATIONAL INSTI-**
12 **TUTES OF HEALTH WITH RESPECT TO RE-**
13 **SEARCH ON MUSCULAR DYSTROPHY.**

14 Part A of title IV of the Public Health Service Act
15 (42 U.S.C. 281 et seq.) is amended by adding at the end
16 the following:

17 **“SEC. 404E. MUSCULAR DYSTROPHY; INITIATIVE THROUGH**
18 **DIRECTOR OF NATIONAL INSTITUTES OF**
19 **HEALTH.**

20 “(a) EXPANSION, INTENSIFICATION, AND COORDINA-
21 TION OF ACTIVITIES.—

22 “(1) IN GENERAL.—The Director of NIH, in
23 coordination with the Directors of the National In-
24 stitute of Neurological Disorders and Stroke, the
25 National Institute of Arthritis and Musculoskeletal

1 and Skin Diseases, the National Institute of Child
2 Health and Human Development, and the other na-
3 tional research institutes as appropriate, shall ex-
4 pand and intensify programs of such Institutes with
5 respect to research and related activities concerning
6 various forms of muscular dystrophy, including
7 Duchenne, myotonic, facioscapulohumeral muscular
8 dystrophy (referred to in this section as ‘FSHD’)
9 and other forms of muscular dystrophy.

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

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

21 “(b) CENTERS OF EXCELLENCE.—

22 “(1) IN GENERAL.—The Director of NIH shall
23 award grants and contracts under subsection (a)(1)
24 to public or nonprofit private entities to pay all or
25 part of the cost of planning, establishing, improving,

1 and providing basic operating support for centers of
2 excellence regarding research on various forms of
3 muscular dystrophy.

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

13 “(3) COORDINATION OF CENTERS; REPORTS.—
14 The Director of NIH—

15 “(A) shall, as appropriate, provide for the
16 coordination of information among centers
17 under paragraph (1) and ensure regular com-
18 munication between such centers; and

19 “(B) shall require the periodic preparation
20 of reports on the activities of the centers and
21 the submission of the reports to the Director.

22 “(4) ORGANIZATION OF CENTERS.—Each cen-
23 ter under paragraph (1) shall use the facilities of a
24 single institution, or be formed from a consortium of

1 cooperating institutions, meeting such requirements
2 as may be prescribed by the Director of NIH.

3 “(5) DURATION OF SUPPORT.—Support for a
4 center established under paragraph (1) may be pro-
5 vided under this section for a period of not to exceed
6 5 years. Such period may be extended for 1 or more
7 additional periods not exceeding 5 years if the oper-
8 ations of such center have been reviewed by an ap-
9 propriate technical and scientific peer review group
10 established by the Director of NIH and if such
11 group has recommended to the Director that such
12 period should be extended.

13 “(c) FACILITATION OF RESEARCH.—The Director of
14 NIH shall provide for a program under subsection (a)(1)
15 under which samples of tissues and genetic materials that
16 are of use in research on muscular dystrophy are donated,
17 collected, preserved, and made available for such research.
18 The program shall be carried out in accordance with ac-
19 cepted scientific and medical standards for the donation,
20 collection, and preservation of such samples.

21 “(d) COORDINATING COMMITTEE.—

22 “(1) IN GENERAL.—The Secretary shall estab-
23 lish the Muscular Dystrophy Coordinating Com-
24 mittee (referred to in this section as the ‘Coordi-
25 nating Committee’) to coordinate activities across

1 the National Institutes and with other Federal
2 health programs and activities relating to the var-
3 ious forms of muscular dystrophy.

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

7 “(A) $\frac{2}{3}$ of such members shall represent
8 governmental agencies, including the directors
9 or their designees of each of the national re-
10 search institutes involved in research with re-
11 spect to muscular dystrophy and representatives
12 of all other Federal departments and agencies
13 whose programs involve health functions or re-
14 sponsibilities relevant to such diseases, includ-
15 ing the Centers for Disease Control and Pre-
16 vention, the Health Resources and Services Ad-
17 ministration and the Food and Drug Adminis-
18 tration and representatives of other govern-
19 mental agencies that serve children with mus-
20 cular dystrophy, such as the Department of
21 Education; and

22 “(B) $\frac{1}{3}$ of such members shall be public
23 members, including a broad cross section of
24 persons affected with muscular dystrophies in-

1 including parents or legal guardians, affected in-
2 dividuals, researchers, and clinicians.

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

6 “(3) CHAIR.—

7 “(A) IN GENERAL.—With respect to mus-
8 cular dystrophy, the Chair of the Coordinating
9 Committee shall serve as the principal advisor
10 to the Secretary, the Assistant Secretary for
11 Health, and the Director of NIH, and shall pro-
12 vide advice to the Director of the Centers for
13 Disease Control and Prevention, the Commis-
14 sioner of Food and Drugs, and to the heads of
15 other relevant agencies. The Coordinating Com-
16 mittee shall select the Chair for a term not to
17 exceed 2 years.

18 “(B) APPOINTMENT.—The Chair of the
19 Committee shall be appointed by and be directly
20 responsible to the Secretary.

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

24 “(A) The Coordinating Committee shall re-
25 ceive necessary and appropriate administrative

1 support from the Department of Health and
2 Human Services.

3 “(B) The Coordinating Committee shall
4 meet as appropriate as determined by the Sec-
5 retary, in consultation with the chair.

6 “(e) PLAN FOR HHS ACTIVITIES.—

7 “(1) IN GENERAL.—Not later than 1 year after
8 the date of enactment of this section, the Coordi-
9 nating Committee shall develop a plan for con-
10 ducting and supporting research and education on
11 muscular dystrophy through the national research
12 institutes and shall periodically review and revise the
13 plan. The plan shall—

14 “(A) provide for a broad range of research
15 and education activities relating to biomedical,
16 epidemiological, psychosocial, and rehabilitative
17 issues, including studies of the impact of such
18 diseases in rural and underserved communities;

19 “(B) identify priorities among the pro-
20 grams and activities of the National Institutes
21 of Health regarding such diseases; and

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

24 “(2) CERTAIN ELEMENTS OF PLAN.—The plan
25 under paragraph (1) shall, with respect to each form

1 of muscular dystrophy, provide for the following as
2 appropriate:

3 “(A) Research to determine the reasons
4 underlying the incidence and prevalence of var-
5 ious forms of muscular dystrophy.

6 “(B) Basic research concerning the eti-
7 ology and genetic links of the disease and po-
8 tential causes of mutations.

9 “(C) The development of improved screen-
10 ing techniques.

11 “(D) Basic and clinical research for the
12 development and evaluation of new treatments,
13 including new biological agents.

14 “(E) Information and education programs
15 for health care professionals and the public.

16 “(f) REPORTS TO CONGRESS.—The Coordinating
17 Committee shall biennially submit to the Committee on
18 Energy and Commerce of the House of Representatives,
19 and the Committee on Health, Education, Labor, and
20 Pensions of the Senate, a report that describes the re-
21 search, education, and other activities on muscular dys-
22 trophy being conducted or supported through the Depart-
23 ment of Health and Human Services. Each such report
24 shall include the following:

1 “(1) The plan under subsection (e)(1) (or revisions to the plan, as the case may be).

3 “(2) Provisions specifying the amounts expended by the Department of Health and Human Services with respect to various forms of muscular dystrophy, including Duchenne, myotonic, FSHD and other forms of muscular dystrophy.

8 “(3) Provisions identifying particular projects or types of projects that should in the future be considered by the national research institutes or other entities in the field of research on all muscular dystrophies.

13 “(g) PUBLIC INPUT.—The Secretary shall, under subsection (a)(1), provide for a means through which the public can obtain information on the existing and planned programs and activities of the Department of Health and Human Services with respect to various forms of muscular dystrophy and through which the Secretary can receive comments from the public regarding such programs and activities.

21 “(h) AUTHORIZATION OF APPROPRIATIONS.—For the purpose of carrying out this section, there are authorized to be appropriated such sums as may be necessary for each of fiscal years 2002 through 2006. The authorization of appropriations established in the preceding sentence is

1 in addition to any other authorization of appropriations
 2 that is available for conducting or supporting through the
 3 National Institutes of Health research and other activities
 4 with respect to muscular dystrophy.”.

5 **SEC. 4. DEVELOPMENT AND EXPANSION OF ACTIVITIES OF**
 6 **CENTERS FOR DISEASE CONTROL AND PRE-**
 7 **VENTION WITH RESPECT TO EPIDEMIOLOG-**
 8 **ICAL RESEARCH ON MUSCULAR DYSTROPHY.**

9 Part B of title III of the Public Health Service Act
 10 (42 U.S.C. 243 et seq.) is amended by inserting after sec-
 11 tion 317P the following:

12 **“SEC. 317Q. SURVEILLANCE AND RESEARCH REGARDING**
 13 **MUSCULAR DYSTROPHY.**

14 “(a) IN GENERAL.—The Secretary, acting through
 15 the Director of the Centers for Disease Control and Pre-
 16 vention, may award grants and cooperative agreements to
 17 public or nonprofit private entities (including health de-
 18 partments of States and political subdivisions of States,
 19 and including universities and other educational entities)
 20 for the collection, analysis, and reporting of data on
 21 Duchenne and other forms of muscular dystrophy. In
 22 making such awards, the Secretary may provide direct
 23 technical assistance in lieu of cash.

24 “(b) NATIONAL MUSCULAR DYSTROPHY EPIDEMI-
 25 OLOGY PROGRAM.—The Secretary, acting through the Di-

1 rector of the Centers for Disease Control and Prevention,
 2 may award grants to public or nonprofit private entities
 3 (including health departments of States and political sub-
 4 divisions of States, and including universities and other
 5 educational entities) for the purpose of carrying out epide-
 6 miological activities regarding Duchenne and other forms
 7 of muscular dystrophies, including collecting and ana-
 8 lyzing information on the number, incidence, correlates,
 9 and symptoms of cases. In carrying out the preceding sen-
 10 tence, the Secretary shall provide for a national surveil-
 11 lance program. In making awards under this subsection,
 12 the Secretary may provide direct technical assistance in
 13 lieu of cash.

14 “(c) COORDINATION WITH CENTERS OF EXCEL-
 15 LENCE.—The Secretary shall ensure that epidemiological
 16 information under subsections (a) and (b) is made avail-
 17 able to centers of excellence supported under section
 18 404E(b) by the Director of the National Institutes of
 19 Health.

20 “(d) AUTHORIZATION OF APPROPRIATIONS.—There
 21 are authorized to be appropriated such sums as may be
 22 necessary to carry out this section.”.

23 **SEC. 5. INFORMATION AND EDUCATION.**

24 (a) IN GENERAL.—The Secretary of Health and
 25 Human Services (referred to in this Act as the “Sec-

1 retary”) shall establish and implement a program to pro-
 2 vide information and education on muscular dystrophy to
 3 health professionals and the general public, including in-
 4 formation and education on advances in the diagnosis and
 5 treatment of muscular dystrophy and training and con-
 6 tinuing education through programs for scientists, physi-
 7 cians, medical students, and other health professionals
 8 who provide care for patients with muscular dystrophy.

9 (b) STIPENDS.—The Secretary may use amounts
 10 made available under this section providing stipends for
 11 health professionals who are enrolled in training programs
 12 under this section.

13 (c) AUTHORIZATION OF APPROPRIATIONS.—There
 14 are authorized to be appropriated such sums as may be
 15 necessary to carry out this section.

16 **SEC. 6. REPORT TO CONGRESS.**

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

22 **SEC. 7. STUDY ON THE USE OF CENTERS OF EXCELLENCE**
 23 **AT THE NATIONAL INSTITUTES OF HEALTH.**

24 (a) REVIEW.—*Not later than 60 days after the date*
 25 *of enactment of this Act, the Secretary of Health and*

1 *Human Services shall enter into a contract with the Insti-*
2 *tute of Medicine for the purpose of conducting a study and*
3 *making recommendations on the impact of, need for, and*
4 *other issues associated with Centers of Excellence at the Na-*
5 *tional Institutes of Health.*

6 (b) *AREAS OF REVIEW.—In conducting the study*
7 *under subsection (a), the Institute of Medicine shall at a*
8 *minimum consider the following:*

9 (1) *The current areas of research incorporating*
10 *Centers of Excellence (which shall include a descrip-*
11 *tion of such areas) and the relationship of this form*
12 *of funding mechanism to other forms of funding for*
13 *research grants, including investigator initiated re-*
14 *search, contracts and other types of research support*
15 *awards.*

16 (2) *The distinctive aspects of Centers of Excel-*
17 *lence, including the additional knowledge that may be*
18 *expected to be gained through Centers of Excellence as*
19 *compared to other forms of grant or contract mecha-*
20 *nisms.*

21 (3) *The costs associated with establishing and*
22 *maintaining Centers of Excellence, and the record of*
23 *scholarship and training resulting from such Centers.*
24 *The research and training contributions of Centers*

1 *should be assessed on their own merits and in com-*
2 *parison with other forms of research support.*

3 (4) *Specific areas of research in which Centers of*
4 *Excellence may be useful, needed, or underused, as*
5 *well as areas of research in which Centers of Excel-*
6 *lence may not be helpful.*

7 (5) *Criteria that may be applied in determining*
8 *when Centers of Excellence are an appropriate and*
9 *cost-effective research investment and conditions that*
10 *should be present in order to consider the establish-*
11 *ment of Centers of Excellence.*

12 (6) *Alternative research models that may accom-*
13 *plish results similar to or greater than Centers of Ex-*
14 *cellence.*

15 (c) *REPORT.—Not later than 1 year after the date on*
16 *which the contract is entered into under subsection (a), the*
17 *Institute of Medicine shall complete the study under such*
18 *subsection and submit a report to the Secretary of Health*
19 *and Human Services and the appropriate committees of*
20 *Congress that contains the results of such study.*

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