

107TH CONGRESS
1ST SESSION

H. R. 717

To amend the Public Health Service Act to provide for research and services with respect to Duchenne muscular dystrophy.

IN THE HOUSE OF REPRESENTATIVES

FEBRUARY 14, 2001

Mr. WICKER (for himself, Mr. PETERSON of Minnesota, Mr. GREENWOOD, Mr. TANNER, Mr. EHRLICH, Mr. CRAMER, Mr. GORDON, Mrs. EMERSON, Mr. RILEY, Mr. BRYANT, Mr. FORD, Mr. FOLEY, Ms. HOOLEY of Oregon, Mr. KING, Mr. HOBSON, Mr. PICKERING, Mr. CHAMBLISS, Mr. EHLERS, Mr. TOWNS, Mr. MCGOVERN, Mr. LATOURETTE, Mr. DOOLITTLE, Mr. WATTS of Oklahoma, Ms. GRANGER, Mr. BLUMENAUER, Mr. MURTHA, Mr. OLVER, Mr. BOEHLERT, Mr. GOODLATTE, Mr. HOLDEN, Mr. WATKINS, Mr. COBLE, Mr. ISAKSON, Mr. LOBIONDO, Mr. MCCRERY, Mr. KERNS, Mr. GILMAN, Mr. ROHRABACHER, Mr. ISSA, Mr. CALVERT, Mr. LANGEVIN, Mrs. MEEK of Florida, Mr. HASTINGS of Florida, Ms. BROWN of Florida, Mr. MILLER of Florida, Mr. OTTER, Mr. WALDEN of Oregon, Mrs. MYRICK, Mr. LAHOOD, Mr. LIPINSKI, Mr. LEWIS of Kentucky, Mr. WOLF, Mr. HOSTETTLER, Mr. KINGSTON, Mr. SCARBOROUGH, Mr. UPTON, Mr. LEACH, Mr. GILLMOR, Mr. WALSH, Mr. QUINN, Mr. GANSKE, Mr. JONES of North Carolina, Mr. BACHUS, Mr. OXLEY, Mr. TIAHRT, Mr. WELLER, Mr. MATSUI, Mr. WELDON of Florida, Mr. REYNOLDS, Mr. GUTKNECHT, Mr. CHABOT, Mr. HUNTER, Mr. GOODE, Mr. FLETCHER, Mr. SKELTON, Mr. MORAN of Virginia, Mr. RODRIGUEZ, Mr. TURNER, Mr. BENTSEN, Mr. ABERCROMBIE, Mr. GONZALEZ, Mr. BILIRAKIS, Mr. ARMEY, Mr. MCHUGH, Mr. JENKINS, Mr. BOYD, Mr. PUTNAM, Mr. ROGERS of Michigan, Mr. KELLER, Mrs. KELLY, and Mr. MANZULLO) introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To amend the Public Health Service Act to provide for

research and services with respect to Duchenne muscular dystrophy.

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 “Duchenne Muscular
5 Dystrophy Childhood Assistance, Research and Education
6 Amendments of 2001”, or the “DMD CARE Act”.

7 **SEC. 2. FINDINGS.**

8 The Congress finds as follows:

9 (1) Duchenne is the most common form of mus-
10 cular dystrophy, and is characterized by a rapidly
11 progressive muscle weakness that almost always re-
12 sults 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) Children with Duchenne muscular dys-
22 trophy exhibit extreme symptoms of weakness, delay
23 in walking, waddling gait, difficulty in climbing

1 stairs, and progressive mobility problems often in
2 combination with muscle hypertrophy.

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

6 (6) Although the dystrophin gene that causes
7 DMD was successfully identified and isolated by
8 medical researchers in 1987, Federal research de-
9 voted to potential treatment options or a cure since
10 this initial discovery has been minimal.

11 (7) Because of the limited Federal support for
12 medical research specific to this form of muscular
13 dystrophy, current treatment options are minimal in
14 efficacy and palliative, aimed at simply managing
15 the symptoms in an effort to optimize the quality of
16 life.

17 (8) Many family physicians and health care pro-
18 fessionals lack the knowledge and resources to detect
19 and properly diagnose the disease as early as pos-
20 sible, thus exacerbating the progressiveness of symp-
21 toms in cases that go undetected or misdiagnosed.

22 (9) This disease has a significant impact on
23 quality of life—not only for the individual who expe-
24 riences its painful symptoms and resulting disability,
25 but also for family members and caregivers.

1 (10) Currently there exists only a small quan-
2 tity of public information about Duchenne muscular
3 dystrophy, and what little information does exist re-
4 mains inadequately disseminated and insufficient in
5 addressing the needs of specific diverse populations
6 and other underserved groups.

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

12 **SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINA-**
13 **TION OF ACTIVITIES OF NATIONAL INSTI-**
14 **TUTES OF HEALTH WITH RESPECT TO RE-**
15 **SEARCH ON DUCHENNE MUSCULAR DYS-**
16 **TROPHY.**

17 Subpart 4 of part C of title IV of the Public Health
18 Service Act (42 U.S.C. 285d et seq.) is amended by insert-
19 ing after section 441A the following section:

20 “DUCHENNE MUSCULAR DYSTROPHY; INITIATIVE
21 THROUGH DIRECTOR OF NATIONAL INSTITUTES OF
22 HEALTH

23 “SEC. 441B. (a) EXPANSION, INTENSIFICATION, AND
24 COORDINATION OF ACTIVITIES.—

25 “(1) IN GENERAL.—The Director of NIH, in
26 coordination with the Directors of the National In-

1 stitute of Neurological Disorders and Stroke, Na-
2 tional Institute of Arthritis and Musculoskeletal and
3 Skin Diseases and the National Institute of Child
4 Health and Human Development, shall expand and
5 intensify programs of such Institutes with respect to
6 research and related activities concerning Duchenne
7 muscular dystrophy.

8 “(2) COORDINATION.—The Directors referred
9 to in subsection (a) shall jointly coordinate the pro-
10 grams referred to in such subsection and consult
11 with the Muscular Dystrophy Interagency Coordi-
12 nating Committee.

13 “(3) ALLOCATIONS BY DIRECTOR OF NIH.—
14 With respect to amounts appropriated to carry out
15 this section for a fiscal year, the Director of NIH
16 shall allocate the amounts among the national re-
17 search institutes that are referred to in subsection
18 (a).

19 “(b) CENTERS OF EXCELLENCE.—

20 “(1) IN GENERAL.—The Director shall under
21 subsection (a)(1) make awards of grants and con-
22 tracts to public or nonprofit private entities to pay
23 all or part of the cost of planning, establishing, im-
24 proving, and providing basic operating support for

1 centers of excellence regarding research on
2 Duchenne muscular dystrophy.

3 “(2) RESEARCH.—Each center under para-
4 graph (1) shall conduct basic and clinical research
5 into Duchenne muscular dystrophy. Such research
6 should include investigations into the diagnosis,
7 early detection, prevention, control, and adequate
8 treatment of Duchenne muscular dystrophy. The
9 centers, as a group, shall conduct research including
10 the fields of muscle biology, genetics, non-invasive
11 imaging, gene and pharmaceutical therapies.

12 “(3) COORDINATION OF CENTERS; REPORTS.—
13 The Director shall, as appropriate, provide for the
14 coordination of information among centers under
15 paragraph (1) and ensure regular communication
16 between such centers, and may require the periodic
17 preparation of reports on the activities of the centers
18 and the submission of the reports to the Director.

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

24 “(5) NUMBER OF CENTERS; DURATION OF SUP-
25 PORT.—

1 “(A) IN GENERAL.—The Director shall
2 provide for the establishment of not less than
3 three centers under paragraph (1).

4 “(B) DURATION.—Support for a center es-
5 tablished under paragraph (1) may be provided
6 under this section for a period of not to exceed
7 5 years. Such period may be extended for one
8 or more additional periods not exceeding 5
9 years if the operations of such center have been
10 reviewed by an appropriate technical and sci-
11 entific peer review group established by the Di-
12 rector and if such group has recommended to
13 the Director that such period should be ex-
14 tended.

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

23 “(d) COORDINATING COMMITTEE.—

24 “(1) IN GENERAL.—The Secretary shall estab-
25 lish the Muscular Dystrophy Coordinating Com-

1 mittee (referred to in this section as the ‘Coordi-
2 nating Committee’) to coordinate activities across
3 the National Institutes and with other Federal
4 health programs and activities relating to such dis-
5 eases.

6 “(2) COMPOSITION.—The Coordinating Com-
7 mittee shall be composed of the directors or their
8 designees of each of the national research institutes
9 involved in research with respect to Duchenne mus-
10 cular dystrophy and representatives of all other Fed-
11 eral departments and agencies whose programs in-
12 volve health functions or responsibilities relevant to
13 such diseases, including the Centers for Disease
14 Control and Prevention, the Health Services and Re-
15 sources Administration and the Food and Drug Ad-
16 ministration.

17 “(3) CHAIR.—

18 “(A) IN GENERAL.—With respect to
19 Duchenne muscular dystrophy, the Chair of the
20 Committee shall serve as the principal advisor
21 to the Secretary, the Assistant Secretary for
22 Health, and the Director of NIH, and shall pro-
23 vide advice to the Director of the Centers for
24 Disease Control and Prevention, the Commis-

1 sioner of Food and Drugs, and other relevant
2 agencies.

3 “(B) DIRECTOR OF NIH.—The Chair of
4 the Committee shall be directly responsible to
5 the Director of NIH.

6 “(e) PLAN FOR NIH ACTIVITIES.—

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

14 “(A) provide for a broad range of research
15 and education activities relating to biomedical,
16 psychosocial, and rehabilitative issues, including
17 studies of the impact of such diseases on boys
18 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 Duchenne

1 muscular dystrophy, provide for the following as ap-
2 propriate:

3 “(A) Research to determine the reasons
4 underlying the incidence and prevalence of
5 Duchenne 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 “(3) IMPLEMENTATION OF PLAN.—The Direc-
17 tor of NIH shall ensure that programs and activities
18 of the National Institutes of Health regarding
19 Duchenne muscular dystrophy are implemented in
20 accordance with the plan under paragraph (1).

21 “(f) REPORTS TO CONGRESS.—The Coordinating
22 Committee under subsection (b)(1) shall biennially submit
23 to the Committee on Commerce of the House of Rep-
24 resentatives, and the Committee on Health, Education,
25 Labor and Pensions of the Senate, a report that describes

1 the research, education, and other activities on Duchenne
2 muscular dystrophy being conducted or supported through
3 the national research institutes, and that in addition in-
4 cludes the following:

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

7 “(2) Provisions specifying the amounts ex-
8 pended by the National Institutes of Health with re-
9 spect to Duchenne muscular dystrophy.

10 “(3) Provisions identifying particular projects
11 or types of projects that should in the future be con-
12 sidered by the national research institutes or other
13 entities in the field of research on Duchenne mus-
14 cular dystrophy.

15 “(g) PUBLIC INPUT.—The Director shall under sub-
16 section (a)(1) provide for means through which the public
17 can obtain information on the existing and planned pro-
18 grams and activities of the National Institutes of Health
19 with respect to Duchenne muscular dystrophy and through
20 which the Director can receive comments from the public
21 regarding such programs and activities.

22 “(h) AUTHORIZATION OF APPROPRIATIONS.—For the
23 purpose of carrying out this section, there are authorized
24 to be appropriated such sums as may be necessary for
25 each of the fiscal years 2001 through 2005. The author-

1 ization of appropriations established in the preceding sen-
 2 tence is in addition to any other authorization of appro-
 3 priations that is available for conducting or supporting
 4 through the National Institutes of Health research and
 5 other activities with respect to Duchenne muscular dys-
 6 trophy.”.

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

12 Part B of title III of the Public Health Service Act
 13 (42 U.S.C. 243 et seq.) is amended by inserting after sec-
 14 tion 317P the following section:

15 “SURVEILLANCE AND RESEARCH REGARDING DUCHENNE
 16 MUSCULAR DYSTROPHY

17 “SEC. 317Q. (a) IN GENERAL.—The Secretary, act-
 18 ing through the Director of the Centers for Disease Con-
 19 trol and Prevention, may make awards of grants and coop-
 20 erative agreements for the collection, analysis, and report-
 21 ing of data on Duchenne muscular dystrophy. An entity
 22 may receive such an award only if the entity is a public
 23 or nonprofit private entity (including health departments
 24 of States and subdivisions of States, and including univer-
 25 sities and other educational entities). In making such

1 awards, the Secretary may provide direct technical assist-
2 ance in lieu of cash.

3 “(b) NATIONAL DUCHENNE MUSCULAR DYSTROPHY
4 SURVEILLANCE PROGRAM.—

5 “(1) IN GENERAL.—The Secretary of Health
6 and Human Services (in this section referred to as
7 the ‘Secretary’), acting through the Director of the
8 Centers for Disease Control and Prevention, may
9 make awards of grants and cooperative agreements
10 for the collection, analysis, and reporting of data on
11 Duchenne muscular dystrophy. In making such
12 awards, the Secretary may provide direct technical
13 assistance in lieu of cash.

14 “(2) ELIGIBILITY.—To be eligible to receive an
15 award under paragraph (1) an entity shall be a pub-
16 lic or nonprofit private entity (including health de-
17 partments of States and political subdivisions of
18 States, and including universities and other edu-
19 cational entities).

20 “(c) CENTERS OF EXCELLENCE IN DUCHENNE AND
21 RELATED MUSCULAR DYSTROPHIES EPIDEMIOLOGY.—

22 “(1) IN GENERAL.—The Secretary, acting
23 through the Director of the Centers for Disease
24 Control and Prevention, shall establish not less than
25 three regional centers of excellence in Duchenne

1 muscular dystrophy epidemiology for the purpose of
2 collecting and analyzing information on the number,
3 incidence, correlates, and symptoms of Duchenne
4 and related muscular dystrophies.

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

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

20 “(A) The center will collect, analyze, and
21 report Duchenne muscular dystrophy data ac-
22 cording to guidelines prescribed by the Director,
23 after consultation with relevant State and local
24 public health officials, private sector research-

1 ers, and advocates for those with Duchenne
2 muscular dystrophy.

3 “(B) The center will assist with the devel-
4 opment and coordination of State Duchenne
5 and related muscular dystrophy surveillance ef-
6 forts within a region.

7 “(C) The center will identify eligible cases
8 and controls through its surveillance systems
9 and conduct research into factors which may
10 cause Duchenne muscular dystrophy.

11 “(D) The center will develop or extend an
12 area of special research expertise (including ge-
13 netics, immunology, and other relevant research
14 specialty areas).

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

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

1 **SEC. 5. INFORMATION AND EDUCATION.**

2 (a) IN GENERAL.—The Secretary shall establish and
3 implement a program to provide information and edu-
4 cation on Duchenne muscular dystrophy to health profes-
5 sionals and the general public, including information and
6 education on advances in the diagnosis and treatment of
7 Duchenne muscular dystrophy and training and con-
8 tinuing education through programs for scientists, physi-
9 cians, and other health professionals who provide care for
10 patients with Duchenne muscular dystrophy.

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

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

18 **SEC. 6. INTER-AGENCY MUSCULAR DYSTROPHY COORDI-**
19 **NATING COMMITTEE.**

20 (a) ESTABLISHMENT.—The Secretary shall establish
21 a committee to be known as the “Muscular Dystrophy Co-
22 ordinating Committee” (in this section referred to as the
23 “Committee”) to coordinate all efforts within the Depart-
24 ment of Health and Human Services concerning Duchenne
25 muscular dystrophy, including activities carried out
26 through the National Institutes of Health and the Centers

1 for Disease Control and Prevention under this Act (and
2 the amendments made by this Act).

3 (b) MEMBERSHIP.—

4 (1) IN GENERAL.—The Committee shall be
5 composed of the Directors of such national research
6 institutes, of the Centers for Disease Control and
7 Prevention, and of such other agencies and such
8 other officials as the Secretary determines appro-
9 priate.

10 (2) ADDITIONAL MEMBERS.—If determined ap-
11 propriate by the Secretary, the Secretary may ap-
12 point to the Committee—

13 (A) parents or legal guardians of individ-
14 uals with Duchenne muscular dystrophy; and

15 (B) representatives of other governmental
16 agencies that serve children with Duchenne
17 muscular dystrophy such as the Department of
18 Education.

19 (c) ADMINISTRATIVE SUPPORT; TERMS OF SERVICE;
20 OTHER PROVISIONS.—The following shall apply with re-
21 spect to the Committee:

22 (1) The Committee shall receive necessary and
23 appropriate administrative support from the Depart-
24 ment of Health and Human Services.

1 (2) Members of the Committee appointed under
2 subsection (b)(2)(A) shall serve for a term of 3
3 years, and may serve for an unlimited number of
4 terms if reappointed.

5 (3) The Committee shall meet not less than two
6 times each year.

7 **SEC. 7. REPORT TO CONGRESS.**

8 Not later than January 1, 2003, and each January
9 1 thereafter, the Secretary shall prepare and submit to
10 the appropriate committees of Congress, a report con-
11 cerning the implementation of this title and the amend-
12 ments made by this title.

13 **SEC. 8. DEFINITION.**

14 For purposes of this Act, the term “Secretary”
15 means the Secretary of Health and Human Services.

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