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

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A BILL

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

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 ''Duchenne Muscular Dystrophy Childhood Assistance, Research and Education Amendments of 2001'', or the ''DMD CARE Act''.

SEC. 2. FINDINGS.

The Congress finds as follows:

(1) Duchenne is the most common form of muscular dystrophy, and is characterized by a rapidly progressive muscle weakness that almost always results in death, usually by 20 years of age.

(2) Duchenne muscular dystrophy is genetically inherited, and mothers are the carriers in approximately 70 percent of all cases.

(3) If a female is a carrier of the dystrophin gene, there is a 50 percent chance per birth that her male offspring will have Duchenne muscular dystrophy, and a 50 percent chance per birth that her female offspring will be carriers.

(4) Children with Duchenne muscular dystrophy exhibit extreme symptoms of weakness, delay in walking, waddling gait, difficulty in climbing

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

(5) Duchenne is the most common lethal genetic disorder of childhood worldwide, affecting approximately one in every 3,500 boys worldwide.

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

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

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

(9) This disease has 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.

(10) Currently there exists only a small quantity of public information about Duchenne muscular dystrophy, and what little information does exist remains inadequately disseminated and insufficient in addressing the needs of specific diverse populations and other underserved groups.

(11) Educating the public and health care community throughout the country about this devastating disease is of paramount importance and is in every respect in the public interest and to the benefit of all communities.

SEC. 3. EXPANSION, INTENSIFICATION, AND COORDINATION OF ACTIVITIES OF NATIONAL INSTITUTES OF HEALTH WITH RESPECT TO RESEARCH ON DUCHENNE MUSCULAR DYSTROPHY.

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

‘‘DUCHENNE MUSCULAR DYSTROPHY; INITIATIVE THROUGH DIRECTOR OF NATIONAL INSTITUTES OF HEALTH

‘‘SEC. 441B. (a) EXPANSION, INTENSIFICATION, AND COORDINATION OF ACTIVITIES.—

‘‘(1) IN GENERAL.—The Director of NIH, in coordination with the Directors of the National Institute of Neurological Disorders and Stroke, National Institute of Arthritis and Muscoskeletal and Skin Diseases and the National Institute of Child Health and Human Development, shall expand and intensify programs of such Institutes with respect to research and related activities concerning Duchenne muscular dystrophy.

"(2) COORDINATION.—The Directors referred to in subsection (a) shall jointly coordinate the programs referred to in such subsection and consult with the Muscular Dystrophy Interagency Coordinating Committee.

"(3) ALLOCATIONS BY DIRECTOR OF NIH.—With respect to amounts appropriated to carry out this section for a fiscal year, the Director of NIH shall allocate the amounts among the national research institutes that are referred to in subsection (a).

"(b) CENTERS OF EXCELLENCE.—

"(1) IN GENERAL.—The Director shall under subsection (a)(1) make awards of grants and contracts to public or nonprofit private entities to pay all or part of the cost of planning, establishing, improving, and providing basic operating support for centers of excellence regarding research on Duchenne muscular dystrophy.

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

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

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

“(5) NUMBER OF CENTERS; DURATION OF SUPPORT.—

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

"(B) DURATION.—Support for a center established under paragraph (1) may be provided under this section for a period of not to exceed 5 years. Such period may be extended for one or more additional periods not exceeding 5 years if the operations of such center have been reviewed by an appropriate technical and scientific peer review group established by the Director and if such group has recommended to the Director that such period should be extended.

"(c) FACILITATION OF RESEARCH.—The Director shall under subsection (a)(1) provide for a program under which samples of tissues and genetic materials that are of use in research on Duchenne muscular dystrophy are donated, collected, preserved, and made available for such research. The program shall be carried out in accordance with accepted scientific and medical standards for the donation, collection, and preservation of such samples.

"(d) COORDINATING COMMITTEE.—

"(1) IN GENERAL.—The Secretary shall establish the Muscular Dystrophy Coordinating Committee (referred to in this section as the 'Coordinating Committee') to coordinate activities across the National Institutes and with other Federal health programs and activities relating to such diseases.

"(2) COMPOSITION.—The Coordinating Committee shall be composed of the directors or their designees of each of the national research institutes involved in research with respect to Duchenne muscular dystrophy and representatives of all other Federal departments and agencies whose programs involve health functions or responsibilities relevant to such diseases, including the Centers for Disease Control and Prevention, the Health Services and Resources Administration and the Food and Drug Administration.

"(3) CHAIR.—

"(A) IN GENERAL.—With respect to Duchenne muscular dystrophy, the Chair of the Committee shall serve as the principal advisor to the Secretary, the Assistant Secretary for Health, and the Director of NIH, and shall provide advice to the Director of the Centers for Disease Control and Prevention, the Commissioner of Food and Drugs, and other relevant agencies.

"(B) DIRECTOR OF NIH.—The Chair of the Committee shall be directly responsible to the Director of NIH.

"(e) PLAN FOR NIH ACTIVITIES.—

"(1) IN GENERAL.—Not later than 1 year after the date of the enactment of this section, the Coordinating Committee shall develop a plan for conducting and supporting research and education on Duchenne muscular dystrophy through the national research institutes and shall periodically review and revise the plan. The plan shall—

"(A) provide for a broad range of research and education activities relating to biomedical, psychosocial, and rehabilitative issues, including studies of the impact of such diseases on boys in rural and underserved communities;

"(B) identify priorities among the programs and activities of the National Institutes of Health regarding such diseases; and

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

"(2) CERTAIN ELEMENTS OF PLAN.—The plan under paragraph (1) shall, with respect to Duchenne muscular dystrophy, provide for the following as appropriate:

“(A) Research to determine the reasons underlying the incidence and prevalence of Duchenne muscular dystrophy.

“(B) Basic research concerning the etiology and genetic links of the disease and potential causes of mutations.

“(C) The development of improved screening techniques.

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

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

“(3) IMPLEMENTATION OF PLAN.—The Director of NIH shall ensure that programs and activities of the National Institutes of Health regarding Duchenne muscular dystrophy are implemented in accordance with the plan under paragraph (1).

“(f) REPORTS TO CONGRESS.—The Coordinating Committee under subsection (b)(1) shall biennially submit to the Committee on Commerce of the House of Representatives, and the Committee on Health, Education, Labor and Pensions of the Senate, a report that describes the research, education, and other activities on Duchenne muscular dystrophy being conducted or supported through the national research institutes, and that in addition includes the following:

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

"(2) Provisions specifying the amounts expended by the National Institutes of Health with respect to Duchenne muscular dystrophy.

"(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 Duchenne muscular dystrophy.

"(g) PUBLIC INPUT.—The Director shall under subsection (a)(1) provide for means through which the public can obtain information on the existing and planned programs and activities of the National Institutes of Health with respect to Duchenne muscular dystrophy and through which the Director can receive comments from the public regarding such programs and activities.

"(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 the fiscal years 2001 through 2005. The authorization of appropriations established in the preceding sentence is in addition to any other authorization of appropriations that is available for conducting or supporting through the National Institutes of Health research and other activities with respect to Duchenne muscular dystrophy.''.

SEC. 4. DEVELOPMENT AND EXPANSION OF ACTIVITIES OF CENTERS FOR DISEASE CONTROL AND PREVENTION WITH RESPECT TO EPIDEMIOLOGICAL RESEARCH ON DUCHENNE MUSCULAR DYSTROPHY.

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

''SURVEILLANCE AND RESEARCH REGARDING DUCHENNE MUSCULAR DYSTROPHY

''SEC. 317Q. (a) IN GENERAL.—The Secretary, acting through the Director of the Centers for Disease Control and Prevention, may make awards of grants and cooperative agreements for the collection, analysis, and reporting of data on Duchenne muscular dystrophy. An entity may receive such an award only if the entity is a public or nonprofit private entity (including health departments of States and subdivisions of States, and including universities and other educational entities). In making such awards, the Secretary may provide direct technical assistance in lieu of cash.

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

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

“(2) ELIGIBILITY.—To be eligible to receive an award under paragraph (1) an entity shall be a public or nonprofit private entity (including health departments of States and political subdivisions of States, and including universities and other educational entities).

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

“(1) IN GENERAL.—The Secretary, acting through the Director of the Centers for Disease Control and Prevention, shall establish not less than three regional centers of excellence in Duchenne muscular dystrophy epidemiology for the purpose of collecting and analyzing information on the number, incidence, correlates, and symptoms of Duchenne and related muscular dystrophies.

“(2) RECIPIENTS OF AWARDS FOR ESTABLISHMENT OF CENTERS.—Centers under paragraph (1) shall be established and operated through the awarding of grants or cooperative agreements to public or nonprofit private entities that conduct research, including health departments of States and political subdivisions of States, and including universities and other educational entities.

“(3) CERTAIN REQUIREMENTS.—An award for a center under paragraph (1) may be made only if the entity involved submits to the Secretary an application containing such agreements and information as the Secretary may require, including an agreement that the center involved will operate in accordance with the following:

“(A) The center will collect, analyze, and report Duchenne muscular dystrophy data according to guidelines prescribed by the Director, after consultation with relevant State and local public health officials, private sector researchers, and advocates for those with Duchenne muscular dystrophy.

"(B) The center will assist with the development and coordination of State Duchenne and related muscular dystrophy surveillance efforts within a region.

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

"(D) The center will develop or extend an area of special research expertise (including genetics, immunology, and other relevant research specialty areas).

"(d) DEFINITION.—In this title, the term 'State' means each of the several States, the District of Columbia, the Commonwealth of Puerto Rico, American Samoa, Guam, the Commonwealth of the Northern Mariana Islands, the Virgin Islands, and the Trust Territory of the Pacific Islands.

"(e) AUTHORIZATION OF APPROPRIATIONS.—There are authorized to be appropriated such sums as may be necessary to carry out this section.".

SEC. 5. INFORMATION AND EDUCATION.

(a) IN GENERAL.—The Secretary shall establish and implement a program to provide information and education on Duchenne muscular dystrophy to health professionals and the general public, including information and education on advances in the diagnosis and treatment of Duchenne muscular dystrophy and training and continuing education through programs for scientists, physicians, and other health professionals who provide care for patients with Duchenne muscular dystrophy.

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

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

SEC. 6. INTER-AGENCY MUSCULAR DYSTROPHY COORDINATING COMMITTEE.

(a) ESTABLISHMENT.—The Secretary shall establish a committee to be known as the ‘‘Muscular Dystrophy Coordinating Committee’’ (in this section referred to as the ‘‘Committee’’) to coordinate all efforts within the Department of Health and Human Services concerning Duchenne muscular dystrophy, including activities carried out through the National Institutes of Health and the Centers for Disease Control and Prevention under this Act (and the amendments made by this Act).

(b) MEMBERSHIP.—

(1) IN GENERAL.—The Committee shall be composed of the Directors of such national research institutes, of the Centers for Disease Control and Prevention, and of such other agencies and such other officials as the Secretary determines appropriate.

(2) ADDITIONAL MEMBERS.—If determined appropriate by the Secretary, the Secretary may appoint to the Committee—

(A) parents or legal guardians of individuals with Duchenne muscular dystrophy; and

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

(c) ADMINISTRATIVE SUPPORT; TERMS OF SERVICE; OTHER PROVISIONS.—The following shall apply with respect to the Committee:

(1) The Committee shall receive necessary and appropriate administrative support from the Department of Health and Human Services.

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

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

SEC. 7. REPORT TO CONGRESS.

Not later than January 1, 2003, and each January 1 thereafter, the Secretary shall prepare and submit to the appropriate committees of Congress, a report concerning the implementation of this title and the amendments made by this title.

SEC. 8. DEFINITION.

For purposes of this Act, the term ''Secretary'' means the Secretary of Health and Human Services.

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