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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

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 ''Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001'', or the ''MD–CARE Act''.

SEC. 2. FINDINGS.

Congress makes the following findings:

(1) Of the childhood muscular dystrophies, Duchenne Muscular Dystrophy (DMD) is the world's most common and catastrophic form of genetic childhood disease, 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) Duchenne is the most common lethal genetic disorder of childhood worldwide, affecting approximately 1 in every 3,500 boys worldwide.

(5) Children with 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.

(6) Other forms of muscular dystrophy affecting children and adults include Becker, limb girdle,

congenital, facioscapulohumeral, myotonic, oculopharyngeal, distal, and Emery-Dreifuss muscular dystrophies.

(7) Myotonic muscular dystrophy (also known as Steinert's disease and dystrophia myotonica) is the second most prominent form of muscular dystrophy and the type most commonly found in adults. Unlike any of the other muscular dystrophies, the muscle weakness is accompanied by myotonia (delayed relaxation of muscles after contraction) and by a variety of abnormalities in addition to those of muscle.

(8) Facioscapulohumeral muscular dystrophy (referred to in this section as ''FSHD'') is a neuromuscular disorder that is inherited genetically and has an estimated frequency of 1 in 20,000. FSHD, affecting between 15,000 to 40,000 persons, causes a progressive and sever loss of skeletal muscle gradually bringing weakness and reduced mobility. Many persons with FSHD become severely physically disabled and spend many decades in a wheelchair.

(9) FSHD is regarded as a novel genetic phenomenon resulting from a crossover of subtelomeric

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

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

(11) Muscular dystrophies have 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.

(12) Development of therapies for these disorders, while realistic with recent advances in research, is likely to require costly investments and infrastructure to support gene and other therapies.

(13) There is a shortage of qualified researchers in the field of neuromuscular research.

(14) 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.

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

(16) 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 MUSCULAR DYSTROPHY.

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

"SEC. 404E. MUSCULAR DYSTROPHY; INITIATIVE THROUGH DIRECTOR OF NATIONAL INSTITUTES OF HEALTH.

"(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, the National Institute of Arthritis and Muscoskeletal

and Skin Diseases, the National Institute of Child Health and Human Development, and the other national research institutes as appropriate, shall expand and intensify programs of such Institutes with respect to research and related activities concerning various forms of muscular dystrophy, including Duchenne, myotonic, facioscapulohumeral muscular dystrophy (referred to in this section as 'FSHD') and other forms of muscular dystrophy.

"(2) COORDINATION.—The Directors referred to in paragraph (1) shall jointly coordinate the programs referred to in such paragraph and consult with the Muscular Dystrophy Interagency Coordinating Committee established under section 6 of the MD–CARE Act.

"(3) ALLOCATIONS BY DIRECTOR OF NIH.—The Director of NIH shall allocate the amounts appropriated to carry out this section for each fiscal year among the national research institutes referred to in paragraph (1).

"(b) CENTERS OF EXCELLENCE.—

"(1) IN GENERAL.—The Director of NIH shall award grants and contracts under subsection (a)(1) 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 various forms of muscular dystrophy.

"(2) RESEARCH.—Each center under paragraph (1) shall supplement but not replace the establishment of a comprehensive research portfolio in all the muscular dystrophies. As a whole, the centers shall conduct basic and clinical research in all forms of muscular dystrophy including early detection, diagnosis, prevention, and treatment, including the fields of muscle biology, genetics, noninvasive imaging, genetics, pharmacological and other therapies.

"(3) COORDINATION OF CENTERS; REPORTS.—The Director of NIH—

"(A) shall, as appropriate, provide for the coordination of information among centers under paragraph (1) and ensure regular communication between such centers; and

"(B) shall 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 of NIH.

"(5) DURATION OF SUPPORT.—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 1 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 of NIH and if such group has recommended to the Director that such period should be extended.

"(c) FACILITATION OF RESEARCH.—The Director of NIH shall provide for a program under subsection (a)(1) under which samples of tissues and genetic materials that are of use in research on 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 the various forms of muscular dystrophy.

"(2) COMPOSITION.—The Coordinating Committee shall consist of not more than 15 members to be appointed by the Secretary, of which—

"(A) ⅔ of such members shall represent governmental agencies, including the directors or their designees of each of the national research institutes involved in research with respect to 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 Resources and Services Administration and the Food and Drug Administration and representatives of other governmental agencies that serve children with muscular dystrophy, such as the Department of Education; and

"(B) ⅓ of such members shall be public members, including a broad cross section of persons affected with muscular dystrophies in-

cluding parents or legal guardians, affected individuals, researchers, and clinicians.

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

"(3) CHAIR.—

"(A) IN GENERAL.—With respect to muscular dystrophy, the Chair of the Coordinating 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 to the heads of other relevant agencies. The Coordinating Committee shall select the Chair for a term not to exceed 2 years.

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

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

"(A) The Coordinating Committee shall receive necessary and appropriate administrative

support from the Department of Health and Human Services.

"(B) The Coordinating Committee shall meet as appropriate as determined by the Secretary, in consultation with the chair.

"(e) PLAN FOR HHS ACTIVITIES.—

"(1) IN GENERAL.—Not later than 1 year after the date of enactment of this section, the Coordinating Committee shall develop a plan for conducting and supporting research and education on 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, epidemiological, psychosocial, and rehabilitative issues, including studies of the impact of such diseases 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 each form

of muscular dystrophy, provide for the following as appropriate:

"(A) Research to determine the reasons underlying the incidence and prevalence of various forms of 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.

"(f) REPORTS TO CONGRESS.—The Coordinating Committee shall biennially submit to the Committee on Energy and 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 muscular dystrophy being conducted or supported through the Department of Health and Human Services. Each such report shall include the following:

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

"(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.

"(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.

"(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.

"(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

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 muscular dystrophy.''.

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

"SEC. 317Q. SURVEILLANCE AND RESEARCH REGARDING MUSCULAR DYSTROPHY.

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

''(b) NATIONAL MUSCULAR DYSTROPHY EPIDEMIOLOGY PROGRAM.—The Secretary, acting through the Di-

rector of the Centers for Disease Control and Prevention, may award grants to public or nonprofit private entities (including health departments of States and political subdivisions of States, and including universities and other educational entities) for the purpose of carrying out epidemiological activities regarding Duchenne and other forms of muscular dystrophies, including collecting and analyzing information on the number, incidence, correlates, and symptoms of cases. In carrying out the preceding sentence, the Secretary shall provide for a national surveillance program. In making awards under this subsection, the Secretary may provide direct technical assistance in lieu of cash.

''(c) COORDINATION WITH CENTERS OF EXCELLENCE.—The Secretary shall ensure that epidemiological information under subsections (a) and (b) is made available to centers of excellence supported under section 404E(b) by the Director of the National Institutes of Health.

''(d) 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 of Health and Human Services (referred to in this Act as the ''Sec-

retary'') shall establish and implement a program to provide information and education on muscular dystrophy to health professionals and the general public, including information and education on advances in the diagnosis and treatment of muscular dystrophy and training and continuing education through programs for scientists, physicians, medical students, and other health professionals who provide care for patients with muscular dystrophy.

(b) STIPENDS.—The Secretary may use amounts made available under this section providing 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. 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 Act and the amendments made by this Act.

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

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

Human Services shall enter into a contract with the Institute of Medicine for the purpose of conducting a study and making recommendations on the impact of, need for, and other issues associated with Centers of Excellence at the National Institutes of Health.

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

(1) The current areas of research incorporating Centers of Excellence (which shall include a description of such areas) and the relationship of this form of funding mechanism to other forms of funding for research grants, including investigator initiated research, contracts and other types of research support awards.

(2) The distinctive aspects of Centers of Excellence, including the additional knowledge that may be expected to be gained through Centers of Excellence as compared to other forms of grant or contract mechanisms.

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

should be assessed on their own merits and in comparison with other forms of research support.

(4) Specific areas of research in which Centers of Excellence may be useful, needed, or underused, as well as areas of research in which Centers of Excellence may not be helpful.

(5) Criteria that may be applied in determining when Centers of Excellence are an appropriate and cost-effective research investment and conditions that should be present in order to consider the establishment of Centers of Excellence.

(6) Alternative research models that may accomplish results similar to or greater than Centers of Excellence.

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

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