

MUSCULAR DYSTROPHY COMMUNITY ASSISTANCE,
RESEARCH AND EDUCATION AMENDMENTS OF 2001

SEPTEMBER 5, 2001.—Committed to the Committee of the Whole House on the State
of the Union and ordered to be printed

Mr. TAUZIN, from the Committee on Energy and Commerce,
submitted the following

R E P O R T

[To accompany H.R. 717]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 717) to amend the Public Health Service Act to provide for research and services with respect to Duchenne muscular dystrophy, having considered the same, report favorably thereon with amendments and recommend that the bill as amended do pass.

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AMENDMENT

The amendments are as follows:

Strike all after the enacting clause and insert the following:

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 severe 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 Musculoskeletal 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, facioscapulothoracic 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) $\frac{2}{3}$ 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 rep-

representatives of other governmental agencies that serve children with muscular dystrophy, such as the Department of Education; and

“(B) $\frac{1}{3}$ of such members shall be public members, including a broad cross section of persons affected with muscular dystrophies including 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 Director 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 “Secretary”) 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 provides 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.

Amend the title so as to read:

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.

PURPOSE AND SUMMARY

H.R. 717, the Muscular Dystrophy Childhood Assistance, Research and Education Amendments of 2001, allows the Director of the National Institutes of Health (NIH), in coordination with the Directors of the National Institute of Neurological Disorders and

Stroke, the National Institute of Arthritis, and the National Institute of Child Health and Human Development to expand programs with respect to activities concerning Duchenne. The legislation also creates Centers of Excellence for Duchenne, which shall conduct basic and clinical research into Duchenne and various other muscular dystrophies.

BACKGROUND AND NEED FOR LEGISLATION

Duchenne Muscular Dystrophy (DMD) is the most lethal genetic disorder of childhood worldwide, affecting approximately one in every 3,500 boys worldwide. The 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 care givers. Symptoms of DMD include loss of muscle tissue, inability to walk, decreased lung capacity, and inability to move the major joints of the body.

The course of DMD is fairly predictable. Children with the disorder are often late in learning to walk. A preschooler with DMD may seem clumsy and fall often. Soon, he has trouble climbing stairs, getting up from the floor, or running. By school age, the child may walk on his toes or the balls of his feet, with a slightly rolling gait. He has a waddling and unsteady gait, and can easily fall over. Nearly all children with DMD lose the ability to walk sometime between ages 7 and 12. In the teen years, activities involving the arms, legs, or trunk require assistance or mechanical support.

Thanks to advances in many areas of medicine, there are very good therapies available to assist children with all the effects of DMD and other muscular dystrophies. By using all available therapies, patients can prolong their comfort, function, and life expectancy. Despite these advances, however, current treatment options for Duchenne are minimal in efficacy and palliative, aimed at simply managing the symptoms in an effort to optimize the quality of life.

HEARINGS

The Subcommittee on Health held a hearing on June 27, 2001 on H.R. 717 entitled, "Advancing the Health of the American People: Addressing Various Public Health Needs." The witnesses testifying on the bill were Mr. Ed McMahon, National Vice President, Muscular Dystrophy Association and Ms. Pat Furlong, President, Parent Project Muscular Dystrophy.

COMMITTEE CONSIDERATION

On Wednesday, July 11, 2001, the Subcommittee on Health met in an open markup session and approved H.R. 717 for Full Committee consideration, as amended, by voice vote, a quorum being present. On Wednesday, July 18, 2001, the Full Committee met in open markup session and ordered H.R. 717 favorably reported to the House, as amended, by unanimous consent, a quorum being present.

COMMITTEE VOTES

Clause 3(b) of rule XIII of the Rules of the House of Representatives requires the Committee to list the record votes on the motion to report legislation and amendments thereto. There were no record votes taken in connection with ordering H.R. 717 reported. A motion by Mr. Tauzin to order H.R. 717 reported to the House, as amended, was agreed to by unanimous consent.

COMMITTEE OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII of the Rules of the House of Representatives, the Committee held a legislative hearing and made findings that are reflected in this report.

STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

The goal of H.R. 717 is to allow the Director of the National Institutes of Health to expand programs with respect to activities concerning Duchenne, and create Centers of Excellence for Duchenne to conduct basic and clinical research into Duchenne and other muscular dystrophies.

NEW BUDGET AUTHORITY, ENTITLEMENT AUTHORITY, AND TAX EXPENDITURES

In compliance with clause 3(c)(2) of rule XIII of the Rules of the House of Representatives, the Committee finds that H.R. 717, the Muscular Dystrophy Childhood Assistance, Research and Education Amendments of 2001, would result in no new or increased budget authority, entitlement authority, or tax expenditures or revenues.

COMMITTEE COST ESTIMATE

The Committee adopts as its own the cost estimate prepared by the Director of the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974.

CONGRESSIONAL BUDGET OFFICE ESTIMATE

Pursuant to clause 3(c)(3) of rule XIII of the Rules of the House of Representatives, the following is the cost estimate provided by the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974:

U.S. CONGRESS,
CONGRESSIONAL BUDGET OFFICE,
Washington, DC, August 10, 2001.

Hon. W.J. "BILLY" TAUZIN,
*Chairman, Committee on Energy and Commerce,
House of Representatives, Washington, DC.*

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed estimate of H.R. 717, the MD-CARE Act, as ordered reported by the Committee on Energy and Commerce on July 18, 2001.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contact is Christopher J. Topoleski.
Sincerely,

ROBERT A. SUNSHINE
(For Dan L. Crippen, Director).

Enclosure.

H.R. 717—Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001 (MD-CARE Act)

Summary: H.R. 717 would require the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC) to provide grants and expand research on the health needs of individuals with muscular dystrophy. Assuming the appropriation of the necessary amounts, CBO estimates that implementing H.R. 717 would cost \$4 million in 2002 and \$56 million over the 2002–2006 period. The legislation would not affect direct spending or receipts; therefore, pay-as-you-go procedures would not apply.

H.R. 717 contains no intergovernmental or private-sector mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local, and tribal governments would be eligible for grants authorized by the bill for research activities associated with muscular dystrophy.

Estimated Cost to the Federal Government: The estimated budgetary impact of H.R. 717 is shown in the following table. The costs of this legislation fall within budget function 550 (health).

	By fiscal year, in millions of dollars—					
	2001	2002	2003	2004	2005	2006
SPENDING SUBJECT TO APPROPRIATION						
Spending Under Current Law:						
Estimated Authorization Level ¹	6504	6664	6809	6959	7107	7265
Estimated Outlays	5326	6063	6474	6773	6992	7100
Proposed Changes:						
Estimated Authorization Level	0	11	14	15	15	15
Estimated Outlays	0	4	10	13	14	15
Spending Under H.R. 717:						
Estimated Authorization Level	6504	6675	6823	6974	7122	7280
Estimated Outlays	5326	6067	6484	6786	7006	7115

¹The 2001 level is the amount appropriated for that year for the agencies that would be affected by H.R. 717. The 2002–2006 levels are CBO baseline projections, including adjustments for anticipated inflation.

Basis of estimate: The bill would authorize the NIH to award grants to and contract with public and nonprofit private entities known as “Centers of Excellence” to provide basic and clinical research on muscular dystrophy, including diagnosis, early detection, prevention, and treatment. The centers would be awarded renewable contracts for up to five years for each contract period. CBO assumes that NIH would designate one center of excellence in 2002 and two additional centers in 2003.

The NIH would also be required to establish a program under which samples of tissue and other genetic materials used in muscular dystrophy research would be collected, stored, and made available for study.

The Secretary of Health and Human Services (HHS) would be required to establish a coordinating committee to organize muscular dystrophy research activities across the NIH and other federal health programs. The committee would consist of 15 members ap-

pointed from both government agencies and from members of the public affected by muscular dystrophy. The committee would be required to produce a plan that identifies opportunities for research and education on muscular dystrophy. In addition, the committee would be required to submit a biennial report to the Congress summarizing expenditures by HHS and the current and future research agenda.

Based on amounts spent in the past for similar activities, CBO estimates that the activities of the NIH and the coordinating committee would cost \$2 million in 2002 and \$28 million over the 2002–2006 period if the necessary amounts are appropriated.

The bill would authorize appropriations of such sums as necessary for the Centers for Disease Control and Prevention to award grants to public or nonprofit private entities to conduct research, carry out epidemiological activities, and establish a national muscular dystrophy surveillance program. Based on Information provided by the CDC about amounts spent in the past for similar activities and on the agency’s historical spending patterns, CBO estimates the agency would spend \$2 million in 2002 and \$28 million over the 2002–2006 period for those purposes if the necessary amounts are appropriated.

This estimate assumes that the bill would be enacted and initial appropriations provided by October 1, 2001.

Pay as you-go considerations: None.

Intergovernmental and private-sector impact: H.R. 717 contains no intergovernmental or private-sector mandates as defined in UMRA. State, local, and tribal governments would be eligible for grants authorized by the bill for research activities associated with muscular dystrophy.

Estimate prepared by: Federal Costs: Christopher J. Topoleski and Jeanne De Sa. Impact on State, Local, and Tribal Governments: Leo Lex. Impact on the Private Sector: Amy Fedigan.

Estimate approved by: Robert A. Sunshine, Assistant Director for Budget Analysis.

FEDERAL MANDATES STATEMENT

The Committee adopts as its own the estimate of Federal mandates prepared by the Director of the Congressional Budget Office pursuant to section 423 of the Unfunded Mandates Reform Act.

ADVISORY COMMITTEE STATEMENT

No advisory committees within the meaning of section 5(b) of the Federal Advisory Committee Act were created by this legislation.

CONSTITUTIONAL AUTHORITY STATEMENT

Pursuant to clause 3(d)(1) of rule XIII of the Rules of the House of Representatives, the Committee finds that the Constitutional authority for this legislation is provided in Article I, section 8, clause 3, which grants Congress the power to regulate commerce with foreign nations, among the several States, and with the Indian tribes.

APPLICABILITY TO LEGISLATIVE BRANCH

The Committee finds that the legislation does not relate to the terms and conditions of employment or access to public services or accommodations within the meaning of section 102(b)(3) of the Congressional Accountability Act.

SECTION-BY-SECTION ANALYSIS OF THE LEGISLATION

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.”

Section 2. Findings

Section 2 finds that: (1) Duchenne Muscular Dystrophy (DMD) is the world’s most common and catastrophic form of genetic childhood disease; (2) DMD is genetically inherited, and mothers are the carriers in approximately 70 percent of all cases; (3) DMD affects 1 in 3,500 boys worldwide; (4) there are other dystrophies which also have a devastating impact on the quality of life for patients, including Myotonic muscular dystrophy and Facioscapulohumeral muscular dystrophy; (5) there is a shortage of qualified researchers in the field of neuromuscular research; (6) development of therapies for these disorders is costly; and, (7) educating the public and health care community is in the public interest and to the benefit of all communities.

Section 3. Expansion, intensification, and coordination of activities of National Institutes of Health with respect to research on muscular dystrophy

Section 3 directs the Director of the NIH to expand and intensify activities with respect to various forms of muscular dystrophy, and to allocate the amounts appropriated to carry out this section.

In addition, this section directs the Director of NIH to award grants and contracts to public or nonprofit private entities to pay all, or part of, the costs of planning, establishing, improving, and providing support to centers of excellence regarding research on various forms of muscular dystrophy.

The Secretary of Health and Human Services is directed to establish the Muscular Dystrophy Coordinating Committee to coordinate activities across other Federal health programs relating to the various forms of muscular dystrophy.

Section 4. Development and expansion of activities of Centers for Disease Control and Prevention with respect to epidemiological research on muscular dystrophy

Section 4 allows the Secretary, acting through the Director of the Centers for Disease Control and Prevention, to award grants and cooperative agreements to public or nonprofit private entities for the collection, analysis, and reporting of data on Duchenne and other forms of muscular dystrophy. This section also allows the Secretary to award grants to public or nonprofit private entities for the purpose of carrying out epidemiological activities regarding muscular dystrophy.

Section 5. Information and education

Section 5 requires the Secretary of Health and Human Services to establish and implement a program to provide information and education on muscular dystrophy, including information and education on advances in the diagnosis and treatment of muscular dystrophy.

Section 6. Report to Congress

Section 6 requires that 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.

CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

In compliance with clause 3(e) of rule XIII of the Rules of the House of Representatives, changes in existing law made by the bill, as reported, are shown as follows (new matter is printed in italic and existing law in which no change is proposed is shown in roman):

PUBLIC HEALTH SERVICE ACT

* * * * *

TITLE III—GENERAL POWERS AND DUTIES OF PUBLIC HEALTH SERVICE

* * * * *

PART B—FEDERAL-STATE COOPERATION

* * * * *

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 Director 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 sub-

section, 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.

TITLE IV—NATIONAL RESEARCH INSTITUTES

PART A—NATIONAL INSTITUTES OF HEALTH

* * * * *

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 Musculoskeletal 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) *$\frac{2}{3}$ 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) *$\frac{1}{3}$ of such members shall be public members, including a broad cross section of persons affected with muscular dystrophies including 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.

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