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HOUSE OF REPRESENTATIVES
REPORT
113–556

PAUL D. WELLSTONE MUSCULAR DYSTROPHY COMMUNITY ASSISTANCE, RESEARCH AND EDUCATION AMENDMENTS OF 2014

JULY 24, 2014.—Committed to the Committee of the Whole House on the State of the Union and ordered to be printed

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

R E P O R T

[To accompany H.R. 594]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (H.R. 594) to reauthorize and extend the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2008, having considered the same, report favorably thereon with an amendment and recommend that the bill as amended do pass.

CONTENTS

The amendment is as follows:

Strike all after the enacting clause and insert the following:

SECTION 1. SHORT TITLE.

This Act may be cited as the "Paul D. Wellstone Muscular Dystrophy Community Assistance, Research and Education Amendments of 2014".

SEC. 2. INITIATIVE THROUGH THE DIRECTOR OF THE NATIONAL INSTITUTES OF HEALTH.

Section 404E of the Public Health Service Act (42 U.S.C. 283g) is amended—

(1) in subsection (a)(1)—

(A) by striking "Muscoskeletal" and inserting "Musculoskeletal"; and

(B) by inserting "Becker, congenital muscular dystrophy, limb-girdle muscular dystrophy," after "Duchenne,";

(2) in subsection (b)—

(A) in paragraph (2)—

(i) by striking "genetics," at the second place it appears; and

(ii) by inserting "cardiac and pulmonary function, and" after "imaging,"; and

(B) in paragraph (3), by inserting "and sharing of data" after "regular communication";

(3) in subsection (d)—

(A) in paragraph (2)—

(i) in the matter preceding subparagraph (A), by striking "15" and inserting "18"; and

(ii) in subparagraph (A)—

(I) by striking "and the Food and Drug Administration" and inserting ", the Food and Drug Administration, and the Administration for Community Living";

(II) by inserting "and adults" after "children"; and

(III) by striking "such as the Department of Education" and inserting "including the Department of Education and the Social Security Administration"; and

(B) in paragraph (4)(B), by inserting ", but shall meet no fewer than two times per calendar year" before the period; and

(4) in subsection (e)—

(A) in paragraph (1)—

(i) in the matter preceding subparagraph (A), by striking "through the national research institutes" and inserting "through the agencies represented on the Coordinating Committee pursuant to subsection (d)(2)(A)"; and

(ii) in subparagraph (A)—

(I) by inserting "public services," before "and rehabilitative issues"; and

(II) by inserting ", studies to demonstrate the cost-effectiveness of providing independent living resources and support to patients with various forms of muscular dystrophy, and studies to determine optimal clinical care interventions for adults with various forms of muscular dystrophy" after "including studies of the impact of such diseases in rural and underserved communities"; and

(B) in paragraph (2)(D), by inserting after "including new biological agents" the following: "and new clinical interventions to improve the health of those with muscular dystrophy".

SEC. 3. SURVEILLANCE AND RESEARCH REGARDING MUSCULAR DYSTROPHY.

The second sentence of section 317Q(b) of the Public Health Service Act (42 U.S.C. 247b–18(b)) is amended by inserting before the period the following: "and, to the extent possible, ensure that data be representative of all affected populations and shared in a timely manner".

SEC. 4. INFORMATION AND EDUCATION.

Section 5(c) of the Muscular Dystrophy Community Assistance, Research and Education Amendments of 2001 (42 U.S.C. 247b–19(c)) is amended—

(1) in paragraph (2)—

(A) by inserting "for pediatric and adult patients, including acute care considerations," after "issuance of care considerations";

(B) by inserting "various" before "other forms of muscular dystrophy"; and

(C) by striking "and" at the end;

(2) by redesignating paragraph (3) as paragraph (4);

(3) by inserting after paragraph (2) the following:

"(3) in developing and updating care considerations under paragraph (2), incorporate strategies specifically responding to the findings of the national transitions survey of minority, young adult, and adult communities of muscular dystrophy patients; and"; and

(4) in paragraph (4), as redesignated, by inserting "various" before "other forms of muscular dystrophy".

PURPOSE AND SUMMARY

H.R. 594, the "Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2014" (MD CARE Amendments) was introduced on February 8, 2013, by Rep. Burgess (R–TX) and Rep. Engel (D–NY) to update and improve the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2008.

BACKGROUND AND NEED FOR LEGISLATION

Muscular dystrophy (MD) is a group of more than 30 genetic diseases characterized by progressive weakness and loss of voluntary muscles that control movement. The muscular dystrophies are a category of multi-systemic disorders that range in age of onset, progression, and severity. Muscular dystrophy affects hundreds of thousands of children and adults throughout the U.S. and around the world. Some forms of muscular dystrophy are seen in infancy or childhood, while others may not appear until adulthood. Many of the muscular dystrophies are characterized by cardiovascular, pulmonary, and digestive issues. Of the nine types of muscular dystrophy, Duchenne muscular dystrophy is the most common. There is no cure for muscular dystrophy. The goal of treatment is a reduction in symptoms and a delay in progress.[1]

The MD CARE Act was signed into law in December 2001, reauthorized in October 2008, and has yielded great advances in understanding the specific causes of the various forms of the muscular dystrophies, the mechanisms of these diseases, identification of therapeutic targets, and clinical trial development. It supports programs that have benefited the lives of muscular dystrophy patients and their families through Federal biomedical research at National Institutes of Health (NIH). It also supports surveillance, research, and outreach activities related to muscular dystrophy at the Centers for Disease Control and Prevention (CDC) that would improve diagnosis, data collection, and care. As a result, there is a growing population of men and women with pediatric-onset forms of Muscular dystrophy now living into adulthood.

H.R. 594 improves upon current programs and updates them to reflect the scientific developments in the field of muscular dystrophy. It would revise the composition of the Muscular Dystrophy Coordinating Committee (MDCC) to include the Social Security Administration (SSA) and the Administration for Community Living (ACL), and ensure that data collected by the CDC captures data related to all affected populations.

HEARINGS

The Committee on Energy and Commerce has not held hearings on the legislation.

[1] *http://www.ninds.nih.gov/disorders/md/md.htm*

COMMITTEE CONSIDERATION

On June 19, 2014, the Subcommittee on Health met in open markup session and forwarded H.R. 594 to the full Committee, as amended, by a voice vote.

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. 594 reported. A motion by Mr. Upton to order H.R. 594 reported to the House, as amended, was agreed to by a voice vote.

COMMITTEE OVERSIGHT FINDINGS

Pursuant to clause 3(c)(1) of rule XIII of the Rules of the House of Representatives, the Committee has not held hearings on this legislation.

STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

Pursuant to clause 3(c)(1) of rule XIII of the House of Representatives, the goal of the legislation is to reauthorize Federal programs related to muscular dystrophy.

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. 594 would result in no new or increased budget authority, entitlement authority, or tax expenditures or revenues.

EARMARK, LIMITED TAX BENEFITS, AND LIMITED TARIFF BENEFITS

In compliance with clause 9(e), 9(f), and 9(g) of rule XXI of the Rules of the House of Representatives, the Committee finds that H.R. 594 contains no earmarks, limited tax benefits, or limited tariff benefits.

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, July 23, 2014.

Hon. FRED UPTON,
Chairman, Committee on Energy and Commerce,
House of Representatives, Washington, DC.

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed cost estimate for H.R. 594, the Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2014.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contacts are Jamease Miles and Santiago Vallinas.

Sincerely,

DOUGLAS W. ELMENDORF.

Enclosure.

H.R. 594—Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2014

Summary: H.R. 594 would amend the Public Health Service Act to reauthorize surveillance, research, and education activities relating to muscular dystrophy. The bill would expand the portfolios of the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC) to include additional forms of muscular dystrophy. It also would direct CDC to capture more representative data regarding muscular dystrophy across populations.

CBO estimates that implementing H.R. 594 would cost $323 million over the 2015–2019 period, assuming appropriation of the necessary amounts. Pay-as-you-go procedures do not apply to this legislation because it would not affect direct spending or revenues.

H.R. 594 contains no intergovernmental and private-sector mandates as defined in the Unfunded Mandates Reform Act (UMRA).

Estimated cost to the Federal Government: The estimated budgetary effect of H.R. 594 is shown in the following table. The costs of this legislation fall within budget function 550 (health).

	By fiscal year, in millions of dollars—					
	2015	2016	2017	2018	2019	2015–2019
CHANGES IN SPENDING SUBJECT TO APPROPRIATION						
Estimated Authorization Level	78	80	81	83	85	407
Estimated Outlays	20	64	77	80	83	323

Note: Numbers may not add to totals because of rounding.

Basis of estimate: For this estimate, CBO assumes that H.R. 594 will be enacted near the end of fiscal year 2014, that the necessary amounts will be appropriated each year, and that outlays will follow historical spending patterns for the affected programs.

The CDC and NIH administer activities that support surveillance, research, and education activities for muscular dystrophy of various forms. Authority to operate NIH-funded programs expired at the end of fiscal year 2009. However, since 2009 the Congress has appropriated funds each year for NIH to continue operating its research programs. The Congress appropriated about $30 billion to

the NIH for fiscal year 2014. Of that total, NIH allocated about $78 million for activities related to muscular dystrophy.

H.R. 594 would reauthorize NIH-funded initiatives for the advancement of muscular dystrophy education, research, and treatment. CBO estimates that implementing H.R. 594 would cost $323 million for NIH activities over the 2015–2019 period, assuming the availability of appropriated funds. The bill would not increase CDC's current surveillance and research activities regarding muscular dystrophy; thus, CBO expects that the legislation would not affect spending by CDC to administer those programs.

Pay-As-You-Go considerations: None.

Intergovernmental and private-sector impact: H.R. 594 contains no intergovernmental or private-sector mandates as defined in UMRA and would impose no costs on state, local, or tribal governments.

Estimate prepared by: Federal costs: Jamease Miles and Santiago Vallinas; Impact on state, local, and tribal governments: J'nell L. Blanco; Impact on the private sector: Alexia Diorio.

Estimate approved by: Holly Harvey, Deputy 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.

DUPLICATION OF FEDERAL PROGRAMS

No provision of H.R. 594 establishes or reauthorizes a program of the Federal Government known to be duplicative of another Federal program, a program that was included in any report from the Government Accountability Office to Congress pursuant to section 21 of Public Law 111–139, or a program related to a program identified in the most recent Catalog of Federal Domestic Assistance.

DISCLOSURE OF DIRECTED RULE MAKINGS

The Committee estimates that enacting H.R. 594 would not specifically direct a rulemaking within the meaning of 5 U.S.C. 551.

ADVISORY COMMITTEE STATEMENT

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

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

Section 1 states the legislation may be cited as the "Paul D. Wellstone Muscular Dystrophy Community Assistance, Research, and Education Amendments of 2014".

Section 2. Initiative through the Director of the National Institutes of Health

Section 2 would increase the membership MDCC) from 15 to 18 members to accommodate the inclusion of the other Federal agencies, such as the SSA and the ACL. The plan developed by the MDCC would include public services, various studies, and the development of clinical interventions to improve the health of those with muscular dystrophy.

The Committee encourages the MDCC to ensure that the needs of a new population of adults living with muscular dystrophy are addressed within the Action Plan and other initiatives. These individuals have been well served by the Individuals with Disabilities Education Act and the Americans with Disability Act, but barriers exist for young people transitioning to adulthood to live independently and maintain employment. The Committee encourages the MDCC to evaluate the cost-effectiveness of providing independent living resources and promoting employment of this population, including the impact of doing so on overall Federal spending.

Section 3. Surveillance and research regarding muscular dystrophy

Section 3 would ensure that the existing program captures data from across the muscular dystrophies and that the data would be shared in a timely manner.

The incidence and prevalence of the muscular dystrophies is the same across all ethnic and racial groups. The Committee is aware that there are gaps in those who are represented within multi-disciplinary clinical care centers and clinical trials. Therefore, the Committee encourages the CDC to continue carrying out its national surveillance program and work to capture and share, in a timely manner, data across all affected populations.

Section 4. Information and education

Section 4 would include pediatric and adult patients and acute care considerations in the development and issuance of care considerations for muscular dystrophy. In addition, care considerations should incorporate strategies from the findings of the national transition survey of minority, young adult, and adult communities of muscular dystrophy patients.

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 (existing law proposed to be omitted is enclosed in black brackets, new matter is printed in italic, existing law in which no change is proposed is shown in roman):

PUBLIC HEALTH SERVICE ACT

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TITLE III—GENERAL POWERS AND DUTIES OF PUBLIC HEALTH SERVICE

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PART B—FEDERAL-STATE COOPERATION

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

(a) * * *

(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 *and, to the extent possible, ensure that data be representative of all affected populations and shared in a timely manner*. In making awards under this subsection, the Secretary may provide direct technical assistance in lieu of cash.

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TITLE IV—NATIONAL RESEARCH INSTITUTES

PART A—NATIONAL INSTITUTES OF HEALTH

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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] *Musculoskeletal* and Skin Diseases, the Eunice Kennedy Shriver National Institute of Child Health and Human Development, the National Heart, Lung, and Blood Institute, 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, *Becker, congenital muscular dystrophy, limb-girdle muscular dystrophy,* myotonic, facioscapulohumeral muscular dystrophy (referred to in this section as "FSHD") and other forms of muscular dystrophy.

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(b) CENTERS OF EXCELLENCE.—

(1) * * *

(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, *cardiac and pulmonary function, and* [genetics,] pharmacological and other therapies.

(3) COORDINATION OF CENTERS.—The Director of NIH shall, as appropriate, provide for the coordination of information among centers under paragraph (1) and ensure regular communication *and sharing of data* between such centers.

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(d) COORDINATING COMMITTEE.—

(1) * * *

(2) COMPOSITION.—The Coordinating Committee shall consist of not more than [15] *18* 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]*, the Food and Drug Administration, and the Administration for Community Living* and representatives of other governmental agencies that serve children *and adults* with muscular dystrophy, [such as the Department of Education] *including the Department of Education and the Social Security Administration*; and

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

(A) * * *

(B) The Coordinating Committee shall meet as appropriate as determined by the Secretary, in consultation with the chair*, but shall meet no fewer than two times per calendar year*.

(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] *through the agencies represented on the Coordinating Committee pursuant to subsection (d)(2)(A)* 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, *public services,* and rehabilitative issues, including

studies of the impact of such diseases in rural and underserved communities*, studies to demonstrate the cost-effectiveness of providing independent living resources and support to patients with various forms of muscular dystrophy, and studies to determine optimal clinical care interventions for adults with various forms of muscular dystrophy*;

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(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) * * *

* * * * * * *

(D) Basic and clinical research for the development and evaluation of new treatments, including new biological agents *and new clinical interventions to improve the health of those with muscular dystrophy*.

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MUSCULAR DYSTROPHY COMMUNITY ASSISTANCE, RESEARCH AND EDUCATION AMENDMENTS OF 2001

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SEC. 5. INFORMATION AND EDUCATION.

(a) * * *

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(c) REQUIREMENTS.—In carrying out this section, the Secretary may—

(1) partner with leaders in the muscular dystrophy patient community;

(2) cooperate with professional organizations and the patient community in the development and issuance of care considerations *for pediatric and adult patients, including acute care considerations,* for Duchenne-Becker muscular dystrophy, and *various* other forms of muscular dystrophy, and in periodic review and updates, as appropriate; **[**and**]**

(3) in developing and updating care considerations under paragraph (2), incorporate strategies specifically responding to the findings of the national transitions survey of minority, young adult, and adult communities of muscular dystrophy patients; and

[(3)**]** *(4)* widely disseminate the Duchenne-Becker muscular dystrophy and *various* other forms of muscular dystrophy care considerations as broadly as possible, including through partnership opportunities with the muscular dystrophy patient community.

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