

§ 283g. 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 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, 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.¹

(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) of this section 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. Such centers of excellence shall be known as the "Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers".

(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

The Director of NIH shall, as appropriate, provide for the coordination of information among centers under paragraph (1) and ensure regular communication between such centers.

(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) of this section 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

¹ See References in Text note below.

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.²

(e) Plan for HHS activities

(1) In general

Not later than 1 year after December 18, 2001, 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) Public input

The Secretary shall, under subsection (a)(1) of this section, provide for a means through which the public can obtain information on the exist-

ing 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.

(g) Clinical research

The Coordinating Committee may evaluate the potential need to enhance the clinical research infrastructure required to test emerging therapies for the various forms of muscular dystrophy by prioritizing the achievement of the goals related to this topic in the plan under subsection (e)(1).

(July 1, 1944, ch. 373, title IV, §404E, as added Pub. L. 107-84, §3, Dec. 18, 2001, 115 Stat. 824; amended Pub. L. 109-482, title I, §§103(b)(4), 104(b)(1)(A), Jan. 15, 2007, 120 Stat. 3687, 3692; Pub. L. 110-154, §1(b)(3), Dec. 21, 2007, 121 Stat. 1827; Pub. L. 110-361, §2, Oct. 8, 2008, 122 Stat. 4010.)

REFERENCES IN TEXT

Section 6 of the MD-CARE Act, referred to in subsec. (a)(2), is section 6 of Pub. L. 107-84, which was formerly set out as a note under section 247b-18 of this title and does not relate to establishment of a coordinating committee. However, subsec. (d) of this section contains provisions relating to the establishment of the Muscular Dystrophy Coordinating Committee.

PRIOR PROVISIONS

A prior section 283g, act July 1, 1944, ch. 373, title IV, §404E, as added Pub. L. 103-43, title II, §209, June 10, 1993, 107 Stat. 149, related to Office of Alternative Medicine, prior to repeal by Pub. L. 105-277, div. A, §101(f) [title VI, §601(1)], Oct. 21, 1998, 112 Stat. 2681-337, 2681-387.

AMENDMENTS

2008—Subsec. (a)(1). Pub. L. 110-361, §2(b)(1), inserted “the National Heart, Lung, and Blood Institute,” after “the Eunice Kennedy Shriver National Institute of Child Health and Human Development.”

Subsec. (b)(1). Pub. L. 110-361, §2(b)(2), inserted at end “Such centers of excellence shall be known as the ‘Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers.’”

Subsec. (f). Pub. L. 110-361, §2(a), struck out subsec. (f) which related to reports.

Subsec. (g). Pub. L. 110-361, §2(a), (b)(3), added subsec. (g) and redesignated former subsec. (g) as (f).

2007—Pub. L. 109-482, §104(b)(1)(A)(ii), which directed amendment of subsec. (b) by striking subsec. (f) and redesignating subsec. (g) as (f), could not literally be executed and was not executed in view of amendments by Pub. L. 110-361. See 2008 Amendment notes above.

Subsec. (a)(1). Pub. L. 110-154 substituted “Eunice Kennedy Shriver National Institute of Child Health and Human Development” for “National Institute of Child Health and Human Development.”

Subsec. (b)(3). Pub. L. 109-482, §104(b)(1)(A)(i), amended heading and text of par. (3) generally. Text read as follows: “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.”

Subsec. (h). Pub. L. 109-482, §103(b)(4), struck out heading and text of subsec. (h). Text read as follows: “For the purpose of carrying out this section, there are authorized to be appropriated such sums as may be nec-

²So in original. Probably should be capitalized.

essary 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.”

EFFECTIVE DATE OF 2007 AMENDMENT

Amendment by Pub. L. 109-482 applicable only with respect to amounts appropriated for fiscal year 2007 or subsequent fiscal years, see section 109 of Pub. L. 109-482, set out as a note under section 281 of this title.

§ 283h. Office of Rare Diseases

(a) Establishment

There is established within the Office of the Director of NIH an office to be known as the Office of Rare Diseases (in this section referred to as the “Office”), which shall be headed by a Director (in this section referred to as the “Director”), appointed by the Director of NIH.

(b) Duties

(1) In general

The Director of the Office shall carry out the following:

(A) The Director shall recommend an agenda for conducting and supporting research on rare diseases through the national research institutes and centers. The agenda shall provide for a broad range of research and education activities, including scientific workshops and symposia to identify research opportunities for rare diseases.

(B) The Director shall, with respect to rare diseases, promote coordination and cooperation among the national research institutes and centers and entities whose research is supported by such institutes.

(C) The Director, in collaboration with the directors of the other relevant institutes and centers of the National Institutes of Health, may enter into cooperative agreements with and make grants for regional centers of excellence on rare diseases in accordance with section 283i of this title.

(D) The Director shall promote the sufficient allocation of the resources of the National Institutes of Health for conducting and supporting research on rare diseases.

(E) The Director shall promote and encourage the establishment of a centralized clearinghouse for rare and genetic disease information that will provide understandable information about these diseases to the public, medical professionals, patients and families.

(2) Principal advisor regarding orphan diseases

With respect to rare diseases, the Director shall serve as the principal advisor to the Director of NIH and shall provide advice to other relevant agencies. The Director shall provide liaison with national and international patient, health and scientific organizations concerned with rare diseases.

(c) Definition

For purposes of this section, the term “rare disease” means any disease or condition that af-

fects less than 200,000 persons in the United States.

(July 1, 1944, ch. 373, title IV, §404F, as added Pub. L. 107-280, §3, Nov. 6, 2002, 116 Stat. 1989; amended Pub. L. 109-482, title I, §§103(b)(5), 104(b)(1)(B), Jan. 15, 2007, 120 Stat. 3687, 3693.)

AMENDMENTS

2007—Subsec. (b)(1)(F), (G). Pub. L. 109-482, §104(b)(1)(B), struck out subpars. (F) and (G) which read as follows:

“(F) The Director shall biennially prepare a report that describes the research and education activities on rare diseases being conducted or supported through the national research institutes and centers, and that identifies particular projects or types of projects that should in the future be conducted or supported by the national research institutes and centers or other entities in the field of research on rare diseases.

“(G) The Director shall prepare the NIH Director’s annual report to Congress on rare disease research conducted by or supported through the national research institutes and centers.”

Subsec. (d). Pub. L. 109-482, §103(b)(5), struck out heading and text of subsec. (d). Text read as follows: “For the purpose of carrying out this section, there are authorized to be appropriated such sums as already have been appropriated for fiscal year 2002, and \$4,000,000 for each of the fiscal years 2003 through 2006.”

EFFECTIVE DATE OF 2007 AMENDMENT

Amendment by Pub. L. 109-482 applicable only with respect to amounts appropriated for fiscal year 2007 or subsequent fiscal years, see section 109 of Pub. L. 109-482, set out as a note under section 281 of this title.

FINDINGS AND PURPOSES

Pub. L. 107-280, §2, Nov. 6, 2002, 116 Stat. 1988, provided that:

“(a) FINDINGS.—Congress makes the following findings:

“(1) Rare diseases and disorders are those which affect small patient populations, typically populations smaller than 200,000 individuals in the United States. Such diseases and conditions include Huntington’s disease, amyotrophic lateral sclerosis (Lou Gehrig’s disease), Tourette syndrome, Crohn’s disease, cystic fibrosis, cystinosis, and Duchenne muscular dystrophy.

“(2) For many years, the 25,000,000 Americans suffering from the over 6,000 rare diseases and disorders were denied access to effective medicines because prescription drug manufacturers could rarely make a profit from marketing drugs for such small groups of patients. The prescription drug industry did not adequately fund research into such treatments. Despite the urgent health need for these medicines, they came to be known as ‘orphan drugs’ because no companies would commercialize them.

“(3) During the 1970s, an organization called the National Organization for Rare Disorders (NORD) was founded to provide services and to lobby on behalf of patients with rare diseases and disorders. NORD was instrumental in pressing Congress for legislation to encourage the development of orphan drugs.

“(4) The Orphan Drug Act [Pub. L. 97-414, see Short Title of 1983 Amendments note set out under section 301 of Title 21, Food and Drugs] created financial incentives for the research and production of such orphan drugs. New Federal programs at the National Institutes of Health and the Food and Drug Administration encouraged clinical research and commercial product development for products that target rare diseases. An Orphan Products Board was established to promote the development of drugs and devices for rare diseases or disorders.

“(5) Before 1983, some 38 orphan drugs had been developed. Since the enactment of the Orphan Drug Act