111TH CONGRESS
1ST SESSION

S. 1158

To authorize the Secretary of Health and Human Services to conduct activities to rapidly advance treatments for spinal muscular atrophy, neuromuscular disease, and other pediatric diseases, and for other purposes.

IN THE SENATE OF THE UNITED STATES

MAY 21, 2009

Ms. STABENOW (for herself, Mr. ISAKSON, and Mr. WHITEHOUSE) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

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A BILL

To authorize the Secretary of Health and Human Services to conduct activities to rapidly advance treatments for spinal muscular atrophy, neuromuscular disease, and other pediatric diseases, and for other purposes.

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 ''SMA Treatment Acceleration Act of 2009''.

SEC. 2. CLINICAL TRIALS NETWORK FOR SPINAL MUSCULAR ATROPHY.

Part B of title IV of the Public Health Service Act is amended by adding at the end the following new section:

"SEC. 409J. CLINICAL TRIALS NETWORK FOR SPINAL MUSCULAR ATROPHY.

''(a) CLINICAL TRIALS NETWORK.—The Director of NIH, in coordination with the Directors of the National Institute of Neurological Disorders and Stroke, the National Institute of Child Health and Human Development, and such other Institutes and Centers as specified by the Director shall provide for the upgrading and unification of spinal muscular atrophy clinical trial sites and the recruitment of new investigators and sites to establish a national clinical trials network for spinal muscular atrophy. The Director of NIH shall ensure that such network—

''(1) conducts coordinated, multisite, clinical trials of therapies and clinical approaches to the treatment of spinal muscular atrophy; and

''(2) rapidly and efficiently disseminates scientific findings to the field.

''(b) DATA COORDINATING CENTER.—The Director of NIH, in coordination with the Commissioner of Food and Drugs and the Directors of the National Institute of Neurological Disorders and Stroke, the National Institute of Child Health and Human Development, and such other

Institutes and Centers as specified by the Director, shall establish a data coordinating center with respect to spinal muscular atrophy to—

''(1) provide expert assistance in the design, conduct, data analysis, data management, and data warehousing of collaborative clinical and descriptive research projects;

''(2) organize and conduct multi-site monitoring activities;

''(3) provide regular reports to the National Institute of Neurological Disorders and Stroke, the National Institute of Child Health and Human Development, such other Institutes and Centers as specified by the Director, and the Food and Drug Administration on enrollment and the allocation of resources; and

''(4) conduct such other activities as are deemed necessary by the Secretary.

''(c) PRE-CLINICAL ACTIVITIES.—The Director of NIH, in coordination with the Directors of the National Institute of Neurological Disorders and Stroke and the National Institute of Child Health and Human Development, shall expand and intensify programs of such Institutes with respect to pre-clinical translation research related to spinal muscular atrophy.''.

SEC. 3. NATIONAL PATIENT REGISTRY.

Part P of title III of the Public Health Service Act is amended by adding at the end the following new section:

"SEC. 399S. NATIONAL SPINAL MUSCULAR ATROPHY PATIENT REGISTRY.

''(a) IN GENERAL.—The Secretary, acting through the Director of the Centers for Disease Control and Prevention and in coordination with the Director of the NIH, shall enhance and provide ongoing support to a spinal muscular atrophy patient registry to provide for expanded epidemiological research towards improving awareness, management, treatment, and prevention of spinal muscular atrophy.

''(b) LONGITUDINAL DATA.—In carrying out subsection (a), the Secretary shall ensure the collection and analysis of longitudinal data related to individuals of all ages with spinal muscular atrophy, including infants, young children, adolescents, and adults of all ages.''.

SEC. 4. INTERAGENCY SPINAL MUSCULAR ATROPHY RESEARCH COORDINATING COMMITTEE.

Part B of title IV of the Public Health Service Act, as amended by section 2, is further amended by adding at the end the following new section:

"SEC. 409K. INTERAGENCY SPINAL MUSCULAR ATROPHY RESEARCH COORDINATING COMMITTEE.

''(a) ESTABLISHMENT.—Not later than 6 months after the date of the enactment of this section, the Secretary shall establish a committee, to be known as the Interagency Spinal Muscular Atrophy Research Coordinating Committee (in this section referred to as the 'Committee').

''(b) DUTIES.—The Committee shall—

''(1) share and coordinate information on existing research activities, and make recommendations to the National Institutes of Health and other Federal agencies regarding how to improve existing research programs, that are related to spinal muscular atrophy research and other related neurological diseases and disorders;

''(2) develop a comprehensive strategy related to spinal muscular atrophy research and other related neurological diseases and disorders and advise the National Institutes of Health and other Federal agencies, expanding proposals for collaborative, multidisciplinary research, including proposals for Common Fund research described in section 402(b)(7) and other proposals that involve collaboration between 2 or more national research institutes or national centers;

''(3) provide annual reports to the Secretary regarding the National Institutes of Health and other Federal agencies' collaborative multidisciplinary research efforts to support spinal muscular atrophy, including the Spinal Muscular Atrophy Project at the National Institute of Neurological Disorders and Stroke, the ongoing and future research needs to advance therapies for spinal muscular atrophy, and recommendations on how to strengthen the collaboration of research activities by the institutes and agencies to improve the results;

''(4) develop a summary of advances in research related to spinal muscular atrophy research and other related neurological diseases and disorders research supported or conducted by Federal agencies; and

''(5) not later than 1 year after the date of the establishment of the Committee, make recommendations to the Secretary—

''(A) regarding any appropriate changes to research activities, including recommendations to improve the research portfolio of the National Institutes of Health to ensure that scientifically-based strategic planning is implemented in support of research priorities that

impact research activities related to spinal muscular atrophy and other related neurological diseases and disorders;

"(B) identifying barriers to the development of new treatments and cures for spinal muscular atrophy and other related neurological diseases and disorders;

"(C) regarding public participation in decisions relating to spinal muscular atrophy research and other related neurological diseases and disorders to increase the involvement of patient advocacy and community organizations representing a broad geographical area;

"(D) on how best to disseminate information on spinal muscular atrophy progress; and

"(E) on how to expand partnerships between public entities, including Federal agencies, and private entities to expand collaborative, cross-cutting research.

"(c) RULE OF CONSTRUCTION.—In carrying out the duties described in subsection (b) with respect to research on spinal muscular atrophy, nothing in this section shall be construed to restrict the Secretary from including other neurological or genetic disorders, as appropriate, in such research if doing so may advance research in spinal mus-

cular atrophy or other related neurological diseases and disorders.

''(d) MEMBERSHIP.—

''(1) IN GENERAL.—The Committee shall be composed of the following voting members:

''(A) Not more than 11 voting Federal representatives as follows:

''(i) The Director of the Centers for Disease Control and Prevention.

''(ii) The Director of the National Institutes of Health and the directors of such national research institutes and national centers (which shall include the National Institute of Neurological Disorders and Stroke, the National Institute of Child Health and Human Development, and the National Center for Research Resources) as the Secretary determines appropriate.

''(iii) The Commissioner of Food and Drugs.

''(iv) The heads of such other agencies and advisory committees as the Secretary determines appropriate, including the Health Resources and Services Administration, the Agency for Healthcare Research

and Quality, and the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children.

''(v) Representatives of other Federal agencies that conduct or support neurological research, or provide support services and resources for individuals with spinal muscular atrophy, such as the Department of Education and the Social Security Administration.

''(B) 9 additional voting members appointed under paragraph (2).

''(2) ADDITIONAL MEMBERS.—The Committee shall include additional voting members appointed by the Secretary as follows:

''(A) 6 members shall be appointed from among scientists, physicians, and other health professionals, who—

''(i) are not officers or employees of the United States;

''(ii) represent multiple disciplines, including clinical, basic, and public health sciences;

''(iii) represent different geographical regions of the United States;

''(iv) are from practice settings, academia, or other research settings; and

''(v) are experienced in scientific peer review process.

''(B) 3 members shall be appointed from members of the general public, who represent individuals with spinal muscular atrophy.

''(3) NONVOTING MEMBERS.—The Committee shall include such nonvoting members as the Secretary determines to be appropriate.

''(e) CHAIRPERSON.—The voting members of the Committee shall select a chairperson from among the Federal members of the Committee described in subsection (d)(1)(A). The selection of a chairperson may be subject to the approval of the Secretary. The chairperson shall serve for a term of not to exceed 2 years, but may be re-elected as provided for in the first sentence.

''(f) MEETINGS.—The Committee shall meet at the call of the chairperson of the Committee or upon the request of the Secretary, but in no case less often than once each year.

''(g) REVIEW.—In 2012, and biennially thereafter, the Secretary shall review the necessity of the Committee.''.

SEC. 5. EDUCATION AND AWARENESS ON SMA FOR HEALTH CARE PROFESSIONALS.

Part P of title III of the Public Health Service Act, as amended by section 3, is further amended by adding at the end the following new section:

''SEC. 399T. INFORMATION AND EDUCATION ON SMA.

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

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