110TH CONGRESS
1ST SESSION

# S. 2042

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.

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IN THE SENATE OF THE UNITED STATES

SEPTEMBER 12, 2007

Ms. STABENOW (for herself, Mr. ISAKSON, Mr. WARNER, 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''.

**SEC. 2. FINDINGS.**

The Congress makes the following findings:

(1) Spinal muscular atrophy (SMA) is the number one genetic killer of children under the age of 2.

(2) SMA is an inherited and often fatal disease that destroys the nerves controlling voluntary muscle movement, which affects crawling, walking, head and neck control, and even swallowing.

(3) It is estimated that SMA occurs in nearly 1 of every 6,000 births and is therefore similar in incidence and severity to other well-known genetic diseases such as cystic fibrosis and Duchenne muscular dystrophy, both of which may also benefit from additional focus and progress on SMA.

(4) SMA is caused by the mutation of a single gene. This is extremely advantageous for genetic screening and therapeutic development. The gene mutation that causes SMA is carried by one in every 40 people, or approximately 7,500,000 Americans. Each child of 2 carriers of the mutant gene has a 1 in 4 chance of developing SMA.

(5) The discovery of the gene responsible for the disease, SMN1, as well as a disease modifying ''back-up'' SMN2 gene has opened the door to new SMA treatments. Modulating genes exist not only for SMA but also for other genetic disorders, including Duchenne Muscular Dystrophy, Parkinson's, and

Alzheimer's disease. The modulation of these genes might be expected to impact these disorders. Success with SMN2 induction for SMA will serve as an important proof of principle and impetus for ongoing research in these other conditions.

(6) Based on the advanced genetic understanding of SMA, the disease was selected by the National Institutes of Health (NIH) and the National Institute of Neurological Disorders and Stroke (NINDS) as the prototype for the National Institutes of Health's accelerated drug discovery effort.

(7) In 2003, the National Institute of Neurological Disorders and Stroke (NINDS) established the Spinal Muscular Atrophy Project: A Collaborative Program to Accelerate Therapeutics Development for SMA. The SMA Project's unique collaborative process between private, public, and non-profit partners provides a model translational research program that can be replicated to accelerate the development of safe and effective treatments for a wide variety of disorders.

(8) National non-profit organizations dedicated to finding a treatment and cure for SMA continue to provide substantial private funding and are collaborating with private biotechnology companies,

large pharmaceutical companies, and clinical investigators to identify new drug compounds and facilitate the rapid translation of promising new therapies to individuals with SMA. The aforementioned investment by national non-profit organizations towards finding a treatment and cure for SMA is approximately equal, on an annual basis, to the resources committed by the Federal Government.

(9) A Food and Drug Administration-approved SMA animal model exists that closely mimics the human disease. A number of therapeutics have been identified which are effective in animal models of spinal muscular atrophy.

(10) There is an urgent need to provide Federal support enabling investigators to mount national clinical trials to demonstrate that these treatments are safe and effective for SMA patients.

(11) The establishment and support of a national clinical trials network and a data coordinating center will promote rigorous patient evaluation using common protocols and allow investigators to study large numbers of patients to provide answers more rapidly than individual sites acting alone.

(12) There is a demonstrated need for greater interagency coordination on SMA research and in-

volvement by additional government partners to support the ongoing work of NINDS on the SMA Project as well the work of private SMA voluntary organizations, including most notably the need for active engagement by the National Institute of Child Health and Human Development (NICHD), along with support from the National Center for Research Resources, the Centers for Disease Control and Prevention, the Food and Drug Administration, and the Health Resources and Services Administration

(13) 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. Furthermore, greater awareness of SMA may lead to the identification of pre-symptomatic SMA-afflicted children, which has significant benefits relative to clinical trials and the search for a treatment and cure.

**SEC. 3. 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 and the National Institute of Child Health and Human Develop-

ment, shall provide for the upgrading and unification of existing SMA clinical trial sites to establish a national clinical trials network for SMA. The Director of NIH shall ensure that such network—

(1) conducts coordinated, multisite, clinical trials of pharmacological approaches to the treatment of SMA; and

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

(b) DATA COORDINATING CENTER.—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 establish a data coordinating center with respect to SMA to—

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

(2) provide appropriate and capable leadership and expertise in biostatistics, developmental study design, data management, data analysis, and project management, including staff and site training and quality assurance procedures;

(3) provide research support activities in designing data collection modules, operational and pro-

cedure manuals, quality control systems, and a communications system for clinical site principal investigators, research coordinators, and other network staff;

(4) organize and conduct multi-site monitoring activities; and

(5) provide regular reports to the National Institute of Neurological Disorders and Stroke and the National Institute of Child Health and Human Development on enrollment and the allocation of resources.

(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 and medicinal chemistry related to SMA.

**SEC. 4. NATIONAL PATIENT REGISTRY.**

(a) IN GENERAL.—The Secretary of Health and Human Services, acting through the Director of the Centers for Disease Control and Prevention, shall enhance and provide ongoing support to the existing SMA patient registry to provide for expanded research on the epidemiology of SMA.

(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 SMA, including infants, young children, adolescents, and adults of all ages.

**SEC. 5. NIH COORDINATING COMMITTEE ON SMA.**

(a) COORDINATING COMMITTEE.—

(1) IN GENERAL.—The Secretary shall establish the Spinal Muscular Atrophy Coordinating Committee to coordinate activities across the National Institutes of Health and with other Federal health programs and activities relating to SMA.

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

(A) 2/3 of such members shall represent governmental agencies, including—

(i) the Directors (or their designees) of the National Institute of Neurological Disorders and Stroke, the National Institute of Child Health and Human Development, other national research institutes involved in research with respect to SMA, and the National Center for Research Resources;

(ii) representatives of all other Federal departments, agencies, and advisory committees whose programs involve health functions or responsibilities relevant to SMA, including the Centers for Disease Control and Prevention, the Health Resources and Services Administration, the Food and Drug Administration, and the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children; and

(iii) representatives of other governmental agencies that serve children with SMA, such as the Department of Education; and

(B) 1/3 of such members shall be public members, including a broad cross section of persons affected with SMA, including parents or legal guardians, affected individuals, researchers, and clinicians.

(3) TERM.—Members of the Coordinating Committee appointed under paragraph (2)(B) shall be appointed for a term of 3 years, and may serve for an unlimited number of terms if reappointed.

(4) CHAIR.—

(A) IN GENERAL.—With respect to SMA, 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.

(B) APPOINTMENT.—The Secretary shall appoint the Chair of the Coordinating Committee from among individuals nominated by the Coordinating Committee. The Chair shall be appointed for a term not to exceed 2 years and may be reappointed for not more than 1 additional term.

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

(A) The Secretary shall provide the Coordinating Committee with necessary and appropriate administrative support.

(B) The Coordinating Committee shall meet as determined appropriate by the Secretary, in consultation with the Chair of the Co-

ordinating Committee, but not less than twice each year.

(b) STUDY ON BARRIERS TO DRUG DEVELOPMENT.—

(1) STUDY.—The Coordinating Committee shall conduct a study to identify current and potential future barriers to the development of drugs for treating SMA and other similar genetic disorders. Such study shall—

(A) identify barriers related to the activities of government, industry, and academic medicine;

(B) include substantial input from scientists, patient advocacy groups, and other organizations with direct involvement in SMA research and drug development; and

(C) consider obstacles to drug development at all points along the research continuum from preclinical research to new drug approval.

(2) REPORT TO CONGRESS.—Not later than 1 year after the date of the enactment of this Act, the Coordinating Committee shall submit to the Congress a report on the results of the study described in paragraph (1) together with such recommenda-

tions for legislation or administrative action as the Coordinating Committee determines appropriate.

**SEC. 6. NIH TRANS-INSTITUTE COLLABORATION ON SMA RESEARCH.**

(a) IN GENERAL.—To ensure the success of the SMA Project that was initiated and has been led by National Institute of Neurological Disorders and Stroke, the Director of NIH shall establish a trans-National Institutes of Health cooperative research initiative on SMA.

(b) DUTIES.—The cooperative research initiative established under subsection (a) shall consist of the following activities:

(1) The Director of the National Institute of Neurological Disorders and Stroke shall report to the Director of NIH on the ongoing needs of the SMA Project and required next steps to ensure the continued success of the Project.

(2) Based on the needs of the SMA Project identified in the report required by paragraph (1), the Director of the National Institute of Child Health and Human Development shall provide direct and ongoing support of SMA research and drug development.

(3) The Director of NIH shall identify and promote opportunities for greater collaboration and in-

volvement in SMA research and drug development by other national research institutes.

**SEC. 7. DRUG DEVELOPMENT PROMOTION.**

Not later than 6 months after the date of the enactment of this Act, the Secretary, in direct consultation with the Commissioner of Food and Drugs and the Coordinating Committee, shall submit specific recommendations to Congress to improve and expand on the incentives provided pursuant to the Orphan Drug Act (Public Law 97–414) and related statutes to directly and in-directly promote SMA drug development, such as through the creation of unique incentives for treatments of rare pediatric diseases.

**SEC. 8. EDUCATION AND AWARENESS ON SMA FOR HEALTH CARE PROFESSIONALS.**

(a) IN GENERAL.—The Secretary shall establish and implement a program to provide information and education on SMA to health professionals and the general public, including information and education on advances in the diagnosis and treatment of SMA and training and continuing education through programs for scientists, physicians, medical students, and other health professionals who provide care for patients with SMA.

(b) STIPENDS.—The Secretary may award stipends to health professionals who are enrolled in training programs under this section.

**SEC. 9. DEFINITIONS.**

In this Act:

(1) The term ''Director of NIH'' means the Director of the National Institutes of Health.

(2) The term ''Coordinating Committee'' means the Spinal Muscular Atrophy Coordinating Committee.

(3) The term ''Secretary'' means the Secretary of Health and Human Services.

(4) The term ''SMA'' means spinal muscular atrophy.

**SEC. 10. AUTHORIZATION OF APPROPRIATIONS.**

There is authorized to be appropriated such sums as may be necessary in each fiscal year to carry out this Act.

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