

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.

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

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.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 This Act may be cited as the “SMA Treatment Accel-
5 eration Act”.

6 **SEC. 2. FINDINGS.**

7 The Congress makes the following findings:

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

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

7 (3) It is estimated that SMA occurs in nearly
8 1 of every 6,000 births and is therefore similar in
9 incidence and severity to other well-known genetic
10 diseases such as cystic fibrosis and Duchenne mus-
11 cular dystrophy, both of which may also benefit from
12 additional focus and progress on SMA.

13 (4) SMA is caused by the mutation of a single
14 gene. This is extremely advantageous for genetic
15 screening and therapeutic development. The gene
16 mutation that causes SMA is carried by one in every
17 40 people, or approximately 7,500,000 Americans.
18 Each child of 2 carriers of the mutant gene has a
19 1 in 4 chance of developing SMA.

20 (5) The discovery of the gene responsible for
21 the disease, SMN1, as well as a disease modifying
22 “back-up” SMN2 gene has opened the door to new
23 SMA treatments. Modulating genes exist not only
24 for SMA but also for other genetic disorders, includ-
25 ing Duchenne Muscular Dystrophy, Parkinson’s, and

1 Alzheimer's disease. The modulation of these genes
2 might be expected to impact these disorders. Success
3 with SMN2 induction for SMA will serve as an im-
4 portant proof of principle and impetus for ongoing
5 research in these other conditions.

6 (6) Based on the advanced genetic under-
7 standing of SMA, the disease was selected by the
8 National Institutes of Health (NIH) and the Na-
9 tional Institute of Neurological Disorders and Stroke
10 (NINDS) as the prototype for the National Insti-
11 tutes of Health's accelerated drug discovery effort.

12 (7) In 2003, the National Institute of Neuro-
13 logical Disorders and Stroke (NINDS) established
14 the Spinal Muscular Atrophy Project: A Collabo-
15 rative Program to Accelerate Therapeutics Develop-
16 ment for SMA. The SMA Project's unique collabo-
17 rative process between private, public, and non-profit
18 partners provides a model translational research pro-
19 gram that can be replicated to accelerate the devel-
20 opment of safe and effective treatments for a wide
21 variety of disorders.

22 (8) National non-profit organizations dedicated
23 to finding a treatment and cure for SMA continue
24 to provide substantial private funding and are col-
25 laborating with private biotechnology companies,

1 large pharmaceutical companies, and clinical inves-
2 tigators to identify new drug compounds and facili-
3 tate the rapid translation of promising new therapies
4 to individuals with SMA. The aforementioned invest-
5 ment by national non-profit organizations towards
6 finding a treatment and cure for SMA is approxi-
7 mately equal, on an annual basis, to the resources
8 committed by the Federal Government.

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

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

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

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

1 involvement by additional government partners to sup-
2 port the ongoing work of NINDS on the SMA
3 Project as well the work of private SMA voluntary
4 organizations, including most notably the need for
5 active engagement by the National Institute of Child
6 Health and Human Development (NICHD), along
7 with support from the National Center for Research
8 Resources, the Centers for Disease Control and Pre-
9 vention, the Food and Drug Administration, and the
10 Health Resources and Services Administration

11 (13) Educating the public and health care com-
12 munity throughout the country about this dev-
13 astating disease is of paramount importance and is
14 in every respect in the public interest and to the
15 benefit of all communities. Furthermore, greater
16 awareness of SMA may lead to the identification of
17 pre-symptomatic SMA-afflicted children, which has
18 significant benefits relative to clinical trials and the
19 search for a treatment and cure.

20 **SEC. 3. CLINICAL TRIALS NETWORK FOR SPINAL MUS-**
21 **CULAR ATROPHY.**

22 (a) CLINICAL TRIALS NETWORK.—The Director of
23 NIH, in coordination with the Directors of the National
24 Institute of Neurological Disorders and Stroke and the
25 National Institute of Child Health and Human Develop-

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

5 (1) conducts coordinated, multisite, clinical
6 trials of pharmacological approaches to the treat-
7 ment of SMA; and

8 (2) rapidly and efficiently disseminates sci-
9 entific findings to the field.

10 (b) DATA COORDINATING CENTER.—The Director of
11 NIH, in coordination with the Directors of the National
12 Institute of Neurological Disorders and Stroke and the
13 National Institute of Child Health and Human Develop-
14 ment, shall establish a data coordinating center with re-
15 spect to SMA to—

16 (1) provide expert assistance in the design, con-
17 duct, data analysis, and data management of col-
18 laborative clinical and descriptive research projects;

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

24 (3) provide research support activities in de-
25 signing data collection modules, operational and pro-

1 cedure manuals, quality control systems, and a com-
2 munications system for clinical site principal inves-
3 tigators, research coordinators, and other network
4 staff;

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

7 (5) provide regular reports to the National In-
8 stitute of Neurological Disorders and Stroke and the
9 National Institute of Child Health and Human De-
10 velopment on enrollment and the allocation of re-
11 sources.

12 (c) **PRE-CLINICAL ACTIVITIES.**—The Director of
13 NIH, in coordination with the Directors of the National
14 Institute of Neurological Disorders and Stroke and the
15 National Institute of Child Health and Human Develop-
16 ment, shall expand and intensify programs of such Insti-
17 tutes with respect to pre-clinical translation research and
18 medicinal chemistry related to SMA.

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

20 (a) **IN GENERAL.**—The Secretary of Health and
21 Human Services, acting through the Director of the Cen-
22 ters for Disease Control and Prevention, shall enhance
23 and provide ongoing support to the existing SMA patient
24 registry to provide for expanded research on the epidemi-
25 ology of SMA.

1 (b) LONGITUDINAL DATA.—In carrying out sub-
2 section (a), the Secretary shall ensure the collection and
3 analysis of longitudinal data related to individuals of all
4 ages with SMA, including infants, young children, adoles-
5 cents, and adults of all ages.

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

7 (a) COORDINATING COMMITTEE.—

8 (1) IN GENERAL.—The Secretary shall establish
9 the Spinal Muscular Atrophy Coordinating Com-
10 mittee to coordinate activities across the National
11 Institutes of Health and with other Federal health
12 programs and activities relating to SMA.

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

16 (A) $\frac{2}{3}$ of such members shall represent
17 governmental agencies, including—

18 (i) the Directors (or their designees)
19 of the National Institute of Neurological
20 Disorders and Stroke, the National Insti-
21 tute of Child Health and Human Develop-
22 ment, other national research institutes in-
23 volved in research with respect to SMA,
24 and the National Center for Research Re-
25 sources;

1 (ii) representatives of all other Fed-
2 eral departments, agencies, and advisory
3 committees whose programs involve health
4 functions or responsibilities relevant to
5 SMA, including the Centers for Disease
6 Control and Prevention, the Health Re-
7 sources and Services Administration, the
8 Food and Drug Administration, and the
9 Advisory Committee on Heritable Dis-
10 orders and Genetic Diseases in Newborns
11 and Children; and

12 (iii) representatives of other govern-
13 mental agencies that serve children with
14 SMA, such as the Department of Edu-
15 cation; and

16 (B) 1/3 of such members shall be public
17 members, including a broad cross section of
18 persons affected with SMA, including parents
19 or legal guardians, affected individuals, re-
20 searchers, and clinicians.

21 (3) TERM.—Members of the Coordinating Com-
22 mittee appointed under paragraph (2)(B) shall be
23 appointed for a term of 3 years, and may serve for
24 an unlimited number of terms if reappointed.

25 (4) CHAIR.—

1 (A) IN GENERAL.—With respect to SMA,
2 the Chair of the Coordinating Committee shall
3 serve as the principal advisor to the Secretary,
4 the Assistant Secretary for Health, and the Di-
5 rector of NIH, and shall provide advice to the
6 Director of the Centers for Disease Control and
7 Prevention, the Commissioner of Food and
8 Drugs, and to the heads of other relevant agen-
9 cies.

10 (B) APPOINTMENT.—The Secretary shall
11 appoint the Chair of the Coordinating Com-
12 mittee from among individuals nominated by
13 the Coordinating Committee. The Chair shall be
14 appointed for a term not to exceed 2 years and
15 may be reappointed for not more than 1 addi-
16 tional term.

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

20 (A) The Secretary shall provide the Co-
21 ordinating Committee with necessary and ap-
22 propriate administrative support.

23 (B) The Coordinating Committee shall
24 meet as determined appropriate by the Sec-
25 retary, in consultation with the Chair of the Co-

1 ordinating Committee, but not less than twice
2 each year.

3 (b) STUDY ON BARRIERS TO DRUG DEVELOP-
4 MENT.—

5 (1) STUDY.—The Coordinating Committee shall
6 conduct a study to identify current and potential fu-
7 ture barriers to the development of drugs for treat-
8 ing SMA and other similar genetic disorders. Such
9 study shall—

10 (A) identify barriers related to the activi-
11 ties of government, industry, and academic
12 medicine;

13 (B) include substantial input from sci-
14 entists, patient advocacy groups, and other or-
15 ganizations with direct involvement in SMA re-
16 search and drug development; and

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

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

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

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

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

10 (b) DUTIES.—The cooperative research initiative es-
11 tablished under subsection (a) shall consist of the fol-
12 lowing activities:

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

18 (2) Based on the needs of the SMA Project
19 identified in the report required by paragraph (1),
20 the Director of the National Institute of Child
21 Health and Human Development shall provide direct
22 and ongoing support of SMA research and drug de-
23 velopment.

24 (3) The Director of NIH shall identify and pro-
25 mote opportunities for greater collaboration and in-

1 involvement in SMA research and drug development
2 by other national research institutes.

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

4 Not later than 6 months after the date of the enact-
5 ment of this Act, the Secretary, in direct consultation with
6 the Commissioner of Food and Drugs and the Coordi-
7 nating Committee, shall submit specific recommendations
8 to Congress to improve and expand on the incentives pro-
9 vided pursuant to the Orphan Drug Act (Public Law 97–
10 414) and related statutes to directly and in-directly pro-
11 mote SMA drug development, such as through the cre-
12 ation of unique incentives for treatments of rare pediatric
13 diseases.

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

16 (a) IN GENERAL.—The Secretary shall establish and
17 implement a program to provide information and edu-
18 cation on SMA to health professionals and the general
19 public, including information and education on advances
20 in the diagnosis and treatment of SMA and training and
21 continuing education through programs for scientists, phy-
22 sicians, medical students, and other health professionals
23 who provide care for patients with SMA.

1 (b) STIPENDS.—The Secretary may award stipends
2 to health professionals who are enrolled in training pro-
3 grams under this section.

4 **SEC. 9. DEFINITIONS.**

5 In this Act:

6 (1) The term “Director of NIH” means the Di-
7 rector of the National Institutes of Health.

8 (2) The term “Coordinating Committee” means
9 the Spinal Muscular Atrophy Coordinating Com-
10 mittee.

11 (3) The term “Secretary” means the Secretary
12 of Health and Human Services.

13 (4) The term “SMA” means spinal muscular
14 atrophy.

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

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

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