

110TH CONGRESS
1ST SESSION

H. R. 3334

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 HOUSE OF REPRESENTATIVES

AUGUST 2, 2007

Mr. KENNEDY (for himself and Mr. CANTOR) introduced the following bill;
which was referred to the Committee on Energy and Commerce

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 singling out SMA as the disease closest to treatment
13 of more than 600 neurological disorders.

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

24 (8) National non-profit organizations dedicated
25 to finding a treatment and cure for SMA continue

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

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

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

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

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

13 (13) Despite such landmark legislation as the
14 Orphan Drug Act and the Best Pharmaceuticals for
15 Children Act, additional incentives for industry to
16 engage early in the drug development process and
17 through to drug approval are warranted for diseases
18 as severe and devastating in infant and children
19 populations as SMA.

20 (14) Educating the public and health care com-
21 munity throughout the country about this dev-
22 astating disease is of paramount importance and is
23 in every respect in the public interest and to the
24 benefit of all communities. Furthermore, greater
25 awareness of SMA may lead to the identification of

1 pre-symptomatic SMA-afflicted children, which has
2 significant benefits relative to clinical trials and the
3 search for a treatment and cure.

4 **SEC. 3. CLINICAL TRIALS NETWORK FOR SPINAL MUS-**
5 **CULAR ATROPHY.**

6 (a) **CLINICAL TRIALS NETWORK.**—The Director of
7 NIH, in coordination with the Directors of the National
8 Institute of Neurological Disorders and Stroke and the
9 National Institute of Child Health and Human Develop-
10 ment, shall provide for the upgrading and unification of
11 existing SMA clinical trial sites to establish a national
12 clinical trials network for SMA. The Director of NIH shall
13 ensure that such network—

14 (1) conducts coordinated, multisite, clinical
15 trials of pharmacological approaches to the treat-
16 ment of SMA; and

17 (2) rapidly and efficiently disseminates sci-
18 entific findings to the field.

19 (b) **DATA COORDINATING CENTER.**—The Director of
20 NIH, in coordination with the Directors of the National
21 Institute of Neurological Disorders and Stroke and the
22 National Institute of Child Health and Human Develop-
23 ment, shall establish a data coordinating center with re-
24 spect to SMA to—

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

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

9 (3) provide research support activities in de-
10 signing data collection modules, operational and pro-
11 cedure manuals, quality control systems, and a com-
12 munications system for clinical site principal inves-
13 tigators, research coordinators, and other network
14 staff;

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

17 (5) provide regular reports to the National In-
18 stitute of Neurological Disorders and Stroke and the
19 National Institute of Child Health and Human De-
20 velopment on enrollment and the allocation of re-
21 sources.

22 (c) PRE-CLINICAL ACTIVITIES.—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 expand and intensify programs of such Insti-
2 tutes with respect to pre-clinical translation research and
3 medicinal chemistry related to SMA.

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

5 (a) IN GENERAL.—The Secretary of Health and
6 Human Services, acting through the Director of the Cen-
7 ters for Disease Control and Prevention, shall enhance
8 and provide ongoing support to the existing SMA patient
9 registry to provide for expanded research on the epidemi-
10 ology of SMA.

11 (b) LONGITUDINAL DATA.—In carrying out sub-
12 section (a), the Secretary shall ensure the collection and
13 analysis of longitudinal data related to individuals of all
14 ages with SMA, including infants, young children, adoles-
15 cents, and adults of all ages.

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

17 (a) COORDINATING COMMITTEE.—

18 (1) IN GENERAL.—The Secretary shall establish
19 the Spinal Muscular Atrophy Coordinating Com-
20 mittee to coordinate activities across the National
21 Institutes of Health and with other Federal health
22 programs and activities relating to SMA.

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

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

3 (i) the Directors (or their designees)
4 of the National Institute of Neurological
5 Disorders and Stroke, the National Insti-
6 tute of Child Health and Human Develop-
7 ment, other national research institutes in-
8 volved in research with respect to SMA,
9 and the National Center for Research Re-
10 sources;

11 (ii) representatives of all other Fed-
12 eral departments, agencies, and advisory
13 committees whose programs involve health
14 functions or responsibilities relevant to
15 SMA, including the Centers for Disease
16 Control and Prevention, the Health Re-
17 sources and Services Administration, the
18 Food and Drug Administration, and the
19 Advisory Committee on Heritable Dis-
20 orders and Genetic Diseases in Newborns
21 and Children; and

22 (iii) representatives of other govern-
23 mental agencies that serve children with
24 SMA, such as the Department of Edu-
25 cation; and

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

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

10 (4) CHAIR.—

11 (A) IN GENERAL.—With respect to SMA,
12 the Chair of the Coordinating Committee shall
13 serve as the principal advisor to the Secretary,
14 the Assistant Secretary for Health, and the Di-
15 rector of NIH, and shall provide advice to the
16 Director of the Centers for Disease Control and
17 Prevention, the Commissioner of Food and
18 Drugs, and to the heads of other relevant agen-
19 cies.

20 (B) APPOINTMENT.—The Secretary shall
21 appoint the Chair of the Coordinating Com-
22 mittee from among individuals nominated by
23 the Coordinating Committee. The Chair shall be
24 appointed for a term not to exceed 2 years and

1 may be reappointed for not more than 1 addi-
2 tional term.

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

6 (A) The Secretary shall provide the Co-
7 ordinating Committee with necessary and ap-
8 propriate administrative support.

9 (B) The Coordinating Committee shall
10 meet as determined appropriate by the Sec-
11 retary, in consultation with the Chair of the Co-
12 ordinating Committee, but no less than twice
13 each year.

14 (b) STUDY ON BARRIERS TO DRUG DEVELOP-
15 MENT.—

16 (1) STUDY.—The Coordinating Committee shall
17 conduct a study to identify current and potential fu-
18 ture barriers to the development of drugs for treat-
19 ing SMA and other similar genetic disorders. Such
20 study shall—

21 (A) identify barriers related to the activi-
22 ties of government, industry, and academic
23 medicine;

24 (B) include substantial input from sci-
25 entists and organizations with direct involve-

1 SMA Project and required next steps to ensure the
2 continued success of the Project.

3 (2) Based on the needs of the SMA Project
4 identified in the report required by paragraph (1),
5 the Director of the National Institute of Child
6 Health and Human Development shall provide direct
7 and ongoing support of SMA research and drug de-
8 velopment.

9 (3) The Director of NIH shall identify and pro-
10 mote opportunities for greater collaboration and in-
11 volvement in SMA research and drug development
12 by other national research institutes.

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

14 Not later than 6 months after the date of the enact-
15 ment of this Act, the Secretary, in direct consultation with
16 the Commissioner of Food and Drugs and the Coordi-
17 nating Committee, shall submit specific recommendations
18 to the Congress to improve and expand on the incentives
19 provided pursuant to the Orphan Drug Act (Public Law
20 97–414) and related statutes to directly and indirectly
21 promote SMA drug development, such as through the cre-
22 ation of unique incentives for rare pediatric treatments.

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

3 (a) IN GENERAL.—The Secretary shall establish and
4 implement a program to provide information and edu-
5 cation on SMA to health professionals and the general
6 public, including information and education on advances
7 in the diagnosis and treatment of SMA and training and
8 continuing education through programs for scientists, phy-
9 sicians, medical students, and other health professionals
10 who provide care for patients with SMA.

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

14 **SEC. 9. DEFINITIONS.**

15 In this Act:

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

18 (2) The term “Coordinating Committee” means
19 the Spinal Muscular Atrophy Coordinating Com-
20 mittee.

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

23 (4) The term “SMA” means spinal muscular
24 atrophy.

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