

111TH CONGRESS  
1ST SESSION

# H. R. 2149

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 HOUSE OF REPRESENTATIVES

APRIL 28, 2009

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

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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.

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 of 2009”.

1 **SEC. 2. CLINICAL TRIALS NETWORK FOR SPINAL MUS-**  
2 **CULAR ATROPHY.**

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

5 **“SEC. 409J. CLINICAL TRIALS NETWORK FOR SPINAL MUS-**  
6 **CULAR ATROPHY.**

7 “(a) CLINICAL TRIALS NETWORK.—The Director of  
8 NIH, in coordination with the Directors of the National  
9 Institute of Neurological Disorders and Stroke, the Na-  
10 tional Institute of Child Health and Human Development,  
11 and such other Institutes and Centers as specified by the  
12 Director shall provide for the upgrading and unification  
13 of spinal muscular atrophy clinical trial sites and the re-  
14 cruitment of new investigators and sites to establish a na-  
15 tional clinical trials network for spinal muscular atrophy.

16 The Director of NIH shall ensure that such network—

17 “(1) conducts coordinated, multi-site, clinical  
18 trials of therapies and clinical approaches to the  
19 treatment of spinal muscular atrophy; and

20 “(2) rapidly and efficiently disseminates sci-  
21 entific findings to the field.

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

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

4           “(1) provide expert assistance in the design,  
5           conduct, data analysis, data management, and data  
6           warehousing of collaborative clinical and descriptive  
7           research projects;

8           “(2) organize and conduct multi-site monitoring  
9           activities;

10           “(3) provide regular reports to the National In-  
11           stitute of Neurological Disorders and Stroke, the  
12           National Institute of Child Health and Human De-  
13           velopment, such other Institutes and Centers as  
14           specified by the Director, and the Food and Drug  
15           Administration on enrollment and the allocation of  
16           resources; and

17           “(4) conduct such other activities as are  
18           deemed necessary by the Secretary.

19           “(c) PRE-CLINICAL ACTIVITIES.—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 expand and intensify programs of such Insti-  
24 tutes with respect to pre-clinical translation research re-  
25 lated to spinal muscular atrophy.”.

1 **SEC. 3. NATIONAL PATIENT REGISTRY.**

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

4 **“SEC. 399S. NATIONAL SPINAL MUSCULAR ATROPHY PA-**  
5 **TIENT REGISTRY.**

6 “(a) IN GENERAL.—The Secretary, acting through  
7 the Director of the Centers for Disease Control and Pre-  
8 vention and in coordination with the Director of the NIH,  
9 shall enhance and provide ongoing support to an existing  
10 or new spinal muscular atrophy patient registry to provide  
11 for expanded epidemiological research towards improving  
12 awareness, management, treatment, and prevention of spi-  
13 nal muscular atrophy.

14 “(b) LONGITUDINAL DATA.—In carrying out sub-  
15 section (a), the Secretary shall ensure the collection and  
16 analysis of longitudinal data related to individuals of all  
17 ages with spinal muscular atrophy, including infants,  
18 young children, adolescents, and adults of all ages.”.

19 **SEC. 4. INTERAGENCY SPINAL MUSCULAR ATROPHY RE-**  
20 **SEARCH COORDINATING COMMITTEE.**

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

1 **“SEC. 409K. INTERAGENCY SPINAL MUSCULAR ATROPHY**  
2 **RESEARCH COORDINATING COMMITTEE.**

3 “(a) ESTABLISHMENT.—Not later than 6 months  
4 after the date of the enactment of this section, the Sec-  
5 retary shall establish a committee, to be known as the  
6 Interagency Spinal Muscular Atrophy Research Coordi-  
7 nating Committee (in this section referred to as the ‘Com-  
8 mittee’).

9 “(b) DUTIES.—The Committee shall—

10 “(1) share and coordinate information on exist-  
11 ing research activities, and make recommendations  
12 to the National Institutes of Health and other Fed-  
13 eral agencies regarding how to improve existing re-  
14 search programs, that are related to spinal muscular  
15 atrophy research and other related neurological dis-  
16 eases and disorders;

17 “(2) develop a comprehensive strategy related  
18 to spinal muscular atrophy research and other re-  
19 lated neurological diseases and disorders and advise  
20 the National Institutes of Health and other Federal  
21 agencies, expanding proposals for collaborative, mul-  
22 tidisciplinary research, including proposals for Com-  
23 mon Fund research described in section 402(b)(7)  
24 and other proposals that involve collaboration be-  
25 tween 2 or more national research institutes or na-  
26 tional centers;

1           “(3) provide annual reports to the Secretary re-  
2           garding the National Institutes of Health and other  
3           Federal agencies’ collaborative multidisciplinary re-  
4           search efforts to support spinal muscular atrophy,  
5           including the Spinal Muscular Atrophy Project at  
6           the National Institute of Neurological Disorders and  
7           Stroke, the ongoing and future research needs to ad-  
8           vance therapies for spinal muscular atrophy, and  
9           recommendations on how to strengthen the collabo-  
10          ration of research activities by the institutes and  
11          agencies to improve the results;

12           “(4) develop a summary of advances in research  
13          related to spinal muscular atrophy research and  
14          other related neurological diseases and disorders re-  
15          search supported or conducted by Federal agencies;  
16          and

17           “(5) not later than 1 year after the date of the  
18          establishment of the Committee, make recommenda-  
19          tions to the Secretary—

20           “(A) regarding any appropriate changes to  
21          research activities, including recommendations  
22          to improve the research portfolio of the Na-  
23          tional Institutes of Health to ensure that sci-  
24          entifically based strategic planning is imple-  
25          mented in support of research priorities that

1 impact research activities related to spinal mus-  
2 cular atrophy and other related neurological  
3 diseases and disorders;

4 “(B) identifying barriers to the develop-  
5 ment of new treatments and cures for spinal  
6 muscular atrophy and other related neurological  
7 diseases and disorders;

8 “(C) regarding public participation in deci-  
9 sions relating to spinal muscular atrophy re-  
10 search and other related neurological diseases  
11 and disorders to increase the involvement of pa-  
12 tient advocacy and community organizations  
13 representing a broad geographical area;

14 “(D) on how best to disseminate informa-  
15 tion on spinal muscular atrophy progress; and

16 “(E) on how to expand partnerships be-  
17 tween public entities, including Federal agen-  
18 cies, and private entities to expand collabo-  
19 rative, cross-cutting research.

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

1 cular atrophy or other related neurological diseases and  
2 disorders.

3 “(d) MEMBERSHIP.—

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

6 “(A) Not more than 11 voting Federal rep-  
7 resentatives as follows:

8 “(i) The Director of the Centers for  
9 Disease Control and Prevention.

10 “(ii) The Director of the National In-  
11 stitutes of Health and the directors of such  
12 national research institutes and national  
13 centers (which shall include the National  
14 Institute of Neurological Disorders and  
15 Stroke, the National Institute of Child  
16 Health and Human Development, and the  
17 National Center for Research Resources)  
18 as the Secretary determines appropriate.

19 “(iii) The Commissioner of Food and  
20 Drugs.

21 “(iv) The heads of such other agencies  
22 and advisory committees as the Secretary  
23 determines appropriate, including the  
24 Health Resources and Services Administra-  
25 tion, the Agency for Healthcare Research



1 and Quality, and the Advisory Committee  
2 on Heritable Disorders and Genetic Dis-  
3 eases in Newborns and Children.

4 “(v) Representatives of other Federal  
5 agencies that conduct or support neuro-  
6 logical research, or provide support serv-  
7 ices and resources for individuals with Spi-  
8 nal Muscular Atrophy, such as the Depart-  
9 ment of Education and the Social Security  
10 Administration.

11 “(B) 9 additional voting members ap-  
12 pointed under paragraph (2).

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

16 “(A) 6 members shall be appointed from  
17 among scientists, physicians, and other health  
18 professionals, who—

19 “(i) are not officers or employees of  
20 the United States;

21 “(ii) represent multiple disciplines, in-  
22 cluding clinical, basic, and public health  
23 sciences;

24 “(iii) represent different geographical  
25 regions of the United States;

1                   “(iv) are from practice settings, aca-  
2                   demia, or other research settings; and

3                   “(v) are experienced in scientific peer  
4                   review process.

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

8                   “(3) NONVOTING MEMBERS.—The Committee  
9                   shall include such nonvoting members as the Sec-  
10                  retary determines to be appropriate.

11                  “(e) CHAIRPERSON.—The voting members of the  
12                  Committee shall select a chairperson from among the Fed-  
13                  eral members of the Committee described in subsection  
14                  (d)(1)(A). The selection of a chairperson may be subject  
15                  to the approval of the Secretary.

16                  “(f) MEETINGS.—The Committee shall meet at the  
17                  call of the chairperson of the Committee or upon the re-  
18                  quest of the Secretary, but in no case less often than once  
19                  each year.

20                  “(g) REVIEW.—In 2012, and biennially thereafter,  
21                  the Secretary shall review the necessity of the Com-  
22                  mittee.”.

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

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

6 **“SEC. 399T. INFORMATION AND EDUCATION ON SMA.**

7 “The Secretary shall establish and implement a pro-  
8 gram to provide information and education on spinal mus-  
9 cular atrophy to health professionals and the general pub-  
10 lic, including information and education on advances in  
11 the screening, diagnosis, and treatment of spinal muscular  
12 atrophy and training and continuing education through  
13 programs for scientists, physicians, medical students, and  
14 other health professionals who provide care for patients  
15 with spinal muscular atrophy.”.

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