

113TH CONGRESS
1ST SESSION

S. 1223

To amend the Public Health Service Act to expand and intensify programs of the National Institutes of Health and the Centers for Disease Control and Prevention with respect to translational research and related activities concerning cavernous angioma, and for other purposes.

IN THE SENATE OF THE UNITED STATES

JUNE 26, 2013

Mr. UDALL of New Mexico (for himself and Mr. HEINRICH) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To amend the Public Health Service Act to expand and intensify programs of the National Institutes of Health and the Centers for Disease Control and Prevention with respect to translational research and related activities concerning cavernous angioma, 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 “Cavernous Angioma
5 Research Resource Act of 2013”.

6 **SEC. 2. FINDINGS.**

7 Congress makes the following findings:

1 (1) Cavernous angioma, also termed “cerebral
2 cavernous malformations” or “CCM”, affects an es-
3 timated 1,500,000 people in the United States.

4 (2) Cavernous angioma is a devastating blood
5 vessel disease that is characterized by the presence
6 of vascular lesions that develop and grow within the
7 brain and spinal cord.

8 (3) Detection of cavernous angioma lesions is
9 achieved through costly and specialized medical im-
10 aging techniques. These techniques are often not
11 readily available where patients live, and require se-
12 dation for children and disabled adults.

13 (4) Cavernous angioma is a common type of
14 vascular anomaly, but individuals may not be aware
15 that they have the disease until the onset of serious
16 clinical symptoms. In the genetic forms, they may
17 not be aware that it may be passed on to their chil-
18 dren.

19 (5) Individuals diagnosed with cavernous
20 angioma may experience neurological deficits, sei-
21 zure, stroke, or sudden death.

22 (6) Due to limited research with respect to cav-
23 ernous angioma, there is no treatment regimen for
24 the disease other than brain and spinal surgery.

1 (7) Some individuals with cavernous angioma
2 are not candidates for brain surgery. No alternative
3 treatment option is available for such individuals.

4 (8) There is a shortage of physicians who are
5 familiar with cavernous angioma and affected indi-
6 viduals may find it difficult to receive timely diag-
7 nosis and appropriate care.

8 (9) Due to the presence of a specific disease-
9 causing mutation, termed the “common Hispanic
10 mutation” that has passed through as many as 17
11 generations of Americans descended from the origi-
12 nal Spanish settlers of the Southwest in the 1590s,
13 New Mexico has the highest population density of
14 cavernous angioma in the world. Cavernous angioma
15 affects thousands of individuals in New Mexico and
16 with ancestry in New Mexico.

17 (10) Other States with high rates of cavernous
18 angioma due to the common Hispanic Mutation in-
19 clude Texas, Arizona, and Colorado.

20 (11) To address the public health threat posed
21 by cavernous angioma in New Mexico and through-
22 out the United States, there is a need to identify in-
23 stitutions capable of running clinical trial for this
24 debilitating brain disorder.

1 **SEC. 3. CAVERNOUS ANGIOMA RESEARCH ACTIVITIES.**

2 Part B of title IV of the Public Health Service Act
3 (42 U.S.C. 284 et seq.) is amended by adding at the end
4 the following:

5 **“SEC. 409K. CAVERNOUS ANGIOMA RESEARCH ACTIVITIES.**

6 “(a) EXPANSION, INTENSIFICATION, AND COORDINA-
7 TION OF ACTIVITIES.—The Director of NIH, acting
8 through the director of the National Institute of Neuro-
9 logical Disorders and Stroke, shall expand and intensify
10 programs of the National Institutes of Health or may
11 award grants and cooperative agreements to public or non-
12 profit private entities (including State health departments,
13 political subdivisions of States, universities, and other edu-
14 cational entities) for research and related activities con-
15 cerning cavernous angioma.

16 “(b) ACTIVITIES.—In expanding and intensifying
17 programs under subsection (a), the Director of NIH may
18 carry out the following:

19 “(1) BASIC, TRANSLATIONAL, AND CLINICAL
20 RESEARCH.—Conduct or financially support basic,
21 clinical, and translational research on cavernous
22 angioma, including research on the following:

23 “(A) Proteomic, pharmacological, and cell
24 biological analysis of the cerebral cavernous
25 malformations (referred to in this section as the
26 ‘CCM’) molecules.

1 “(B) Continued development and expansion of novel animal models for cavernous angioma preclinical research.

4 “(C) Early detection, diagnosis, and treatment of cavernous angioma.

6 “(D) Biological mechanisms for lesion genesis, development, and maturation.

8 “(E) Biological mechanisms for lesion bleeding and symptomology.

10 “(F) Novel biomedical and pharmacological interventions designed to prohibit new lesion development, lesion growth, and lesion bleeding.

13 “(G) Contributions of genetic variation to clinical presentation as targets for therapy.

15 “(H) Identification and development of biomarkers to measure phenotypic variation.

17 “(I) Research related to improving the quality of life for individuals with cavernous angioma and their families.

20 “(J) Clinical training programs aimed at increasing the number of scientists and clinicians who are trained to treat patients and carry out these research directions.

24 “(2) FACILITATION OF RESEARCH RESOURCES;
25 CLINICAL TRIAL PREPAREDNESS.—

“(A) COORDINATION.—Identify and support the development of a clinical and research coordinating center with the potential of coordinating a multi-site clinical drug trial for cavernous angioma. Such coordinating center shall provide a model for additional trial sites, facilitate medical research to develop a cure for cavernous angioma, and enhance the medical care of individuals with cavernous angioma nationwide. Such coordinating center shall—

“(i) have an institutional infrastructure that is capable of hosting a clinical trial site and facilitating translational projects and collaborations for clinical trials;

“(ii) have the capacity to maintain programs dedicated to patient education, patient outreach, and awareness, including—

“(I) launching a national multimedia public awareness campaign;

“(II) creating and distributing patient education materials for distribution by national physician and surgeon offices;

1 “(III) establishing an education
 2 program for elementary and sec-
 3 ondary school nurses to facilitate early
 4 detection and diagnosis of cavernous
 5 angioma in areas of high cavernous
 6 angioma population density;

7 “(IV) coordinating regular pa-
 8 tient and family-oriented educational
 9 conferences; and

10 “(V) developing nationally rel-
 11 evant electronic health teaching and
 12 communication tools and a network of
 13 professional capacity and patient and
 14 family support;

15 “(iii) have the capacity to establish
 16 and maintain communication with other
 17 major cavernous angioma research and
 18 care institutions internationally for infor-
 19 mation sharing and coordination of re-
 20 search activities;

21 “(iv) have demonstrated clinical ex-
 22 pertise in cavernous angioma management;

23 “(v) have a sufficient number of eligi-
 24 ble patients for participation with par-
 25 ticular focus on unique subpopulations in-

cluding Common Hispanic Mutation and
CCM3 gene mutation carriers; and

“(vi) have a telehealth infrastructure
to support and to provide clinical consulta-
tion for remote and underserved commu-
nities.

“(B) PARTICIPATION.—Identify and sup-
port the development of clinical and research
participation centers with the potential to par-
ticipate in a multi-site clinical drug trial for
cavernous angioma. Such participation centers
may facilitate medical research to develop a
cure for cavernous angioma and enhance the
medical care of individuals with cavernous
angioma in partnership with the coordinating
center under subparagraph (A) and other na-
tional and international centers. Such participa-
tion centers shall—

“(i) have an institutional infrastruc-
ture capable of hosting a clinical trial site
and facilitating translational projects and
collaborations for clinical trials;

“(ii) have the capacity to maintain
communication with other major cavernous
angioma research and care institutions

internationally for information sharing and
coordination of research activities;

“(iii) have demonstrated clinical ex-
pertise in cavernous angioma management;
and

“(iv) have a sufficient numbers of eli-
gible patients for participation with par-
ticular focus on unique subpopulations in-
cluding Common Hispanic Mutation and
CCM3 gene mutation carriers as these
unique populations may provide insight to
other genetic and non-genetic forms of the
illness.

“(c) TRAINING PROGRAM FOR CLINICIANS AND SCI-
ENTISTS.—

“(1) IN GENERAL.—Eligible coordinating and
participation centers under this section shall estab-
lish or expand training programs for medical and al-
lied health clinicians and scientists in clinical prac-
tice and research relevant to cavernous angioma.

“(2) RESEARCH RESOURCES.—In carrying out
this subsection, the Director of NIH may—

“(A) use information collected by the Na-
tional Institutes of Health pursuant to other

1 provisions of law or prior to the date of the en-
2 actment of this section;

3 “(B) take into consideration the avail-
4 ability of other research resources;

5 “(C) encourage the use of research re-
6 sources for research on, and development of,
7 therapies and treatments for individuals with
8 cavernous angioma; and

9 “(D) encourage the inclusion of individuals
10 with cavernous angioma in clinical trials con-
11 ducted or supported by the National Institutes
12 of Health.

13 “(3) CAVERNOUS ANGIOMA CONSORTIUM.—The
14 Director of NIH may provide for the participation of
15 agencies of the National Institutes of Health in a
16 consortium to facilitate the exchange of information
17 and to make the research effort on cavernous
18 angioma more efficient and effective by ensuring
19 consistent communication, minimizing duplication of
20 effort, and integrating the varied perspectives of
21 partner agencies, organizations, and individuals.
22 Such consortium shall include at least one national
23 cavernous angioma patient advocacy organization
24 and may be the same consortium receiving a grant
25 or contract under subsection (b)(2)(A).”.

1 **SEC. 4. CENTERS FOR DISEASE CONTROL AND PREVEN-**
2 **TION CAVERNOUS ANGIOMA SURVEILLANCE**
3 **AND RESEARCH PROGRAMS.**

4 Part B of title III of the Public Health Service Act
5 (42 U.S.C. 243 et seq.) is amended by inserting after sec-
6 tion 317T the following:

7 **“SEC. 317U. CAVERNOUS ANGIOMA SURVEILLANCE AND RE-**
8 **SEARCH PROGRAMS.**

9 “(a) IN GENERAL.—The Secretary, acting through
10 the Director of the Centers for Disease Control and Pre-
11 vention, may award grants and cooperative agreements to
12 public or nonprofit private entities (including State health
13 departments, political subdivisions of States, universities,
14 and other educational entities) for the collection, analysis,
15 and reporting of data on cavernous angioma. In making
16 such awards, the Secretary may provide direct technical
17 assistance, including personnel support, in lieu of cash.

18 “(b) NATIONAL CAVERNOUS ANGIOMA EPIDEMI-
19 OLOGY PROGRAM.—

20 “(1) GRANTS.—The Secretary, acting through
21 the Director of the Centers for Disease Control and
22 Prevention, may award grants to public or nonprofit
23 private entities (including State health departments,
24 political subdivisions of States, universities, and
25 other educational entities) for the purpose of car-
26 rying out epidemiological activities regarding cav-

ernous angioma, including collecting and analyzing information on the number, incidence, correlates, and symptoms of cases and the clinical utility (including costs and benefits) of specific practice patterns. In making such awards, the Secretary may provide direct technical assistance, including personnel support, in lieu of cash.

“(2) NATIONAL SURVEILLANCE PROGRAM.—In carrying out subsection (a), the Secretary shall—

“(A) provide for a national surveillance program; and

“(B) where possible, ensure that the surveillance program is coordinated with the data and sample collection activities of the National Institutes of Health under section 409K.”.

SEC. 5. FOOD AND DRUG ADMINISTRATION CAVERNOUS ANGIOMA CLINICAL TRIAL PREPAREDNESS AND SUPPORT PROGRAM.

(a) INVESTIGATIONAL NEW DRUG APPLICATION.—

The Commissioner of Food and Drugs shall work with clinical centers, investigators, and advocates to support appropriate investigational new drug application under section 505(i) of the Federal Food, Drug, and Cosmetic Act in an effort to hasten the pace of clinical trials for cavernous angioma.

1 (b) ORPHAN PRODUCT DEVELOPMENT.—Where ap-
2 plicable in rare subpopulations of cavernous angioma re-
3 quiring unique pharmacological intervention, including
4 those with the Common Hispanic Mutation or CCM3 gene
5 mutations, the Commissioner of Food and Drugs shall
6 support appropriate requests for designations of drugs as
7 orphan drugs under section 526 of the Federal Food,
8 Drug, and Cosmetic Act.

9 **SEC. 6. REPORT TO CONGRESS.**

10 Not later than January 1, 2015, and each January
11 1 thereafter, the Secretary of Health and Human Services
12 shall prepare and submit to the appropriate committees
13 of the Congress a report concerning the implementation
14 of this Act and the amendments made by this Act.

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