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

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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.

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 ''Cavernous Angioma Research Resource Act of 2013''.

SEC. 2. FINDINGS.

Congress makes the following findings:

(1) Cavernous angioma, also termed ''cerebral cavernous malformations'' or ''CCM'', affects an estimated 1,500,000 people in the United States.

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

(3) Detection of cavernous angioma lesions is achieved through costly and specialized medical imaging techniques. These techniques are often not readily available where patients live, and require sedation for children and disabled adults.

(4) Cavernous angioma is a common type of vascular anomaly, but individuals may not be aware that they have the disease until the onset of serious clinical symptoms. In the genetic forms, they may not be aware that it may be passed on to their children.

(5) Individuals diagnosed with cavernous angioma may experience neurological deficits, seizure, stroke, or sudden death.

(6) Due to limited research with respect to cavernous angioma, there is no treatment regimen for the disease other than brain and spinal surgery.

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

(8) There is a shortage of physicians who are familiar with cavernous angioma and affected individuals may find it difficult to receive timely diagnosis and appropriate care.

(9) Due to the presence of a specific disease-causing mutation, termed the ''common Hispanic mutation'' that has passed through as many as 17 generations of Americans descended from the original Spanish settlers of the Southwest in the 1590s, New Mexico has the highest population density of cavernous angioma in the world. Cavernous angioma affects thousands of individuals in New Mexico and with ancestry in New Mexico.

(10) Other States with high rates of cavernous angioma due to the common Hispanic Mutation include Texas, Arizona, and Colorado.

(11) To address the public health threat posed by cavernous angioma in New Mexico and throughout the United States, there is a need to identify institutions capable of running clinical trial for this debilitating brain disorder.

SEC. 3. CAVERNOUS ANGIOMA RESEARCH ACTIVITIES.

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

"SEC. 409K. CAVERNOUS ANGIOMA RESEARCH ACTIVITIES.

''(a) EXPANSION, INTENSIFICATION, AND COORDINATION OF ACTIVITIES.—The Director of NIH, acting through the director of the National Institute of Neurological Disorders and Stroke, shall expand and intensify programs of the National Institutes of Health or may award grants and cooperative agreements to public or nonprofit private entities (including State health departments, political subdivisions of States, universities, and other educational entities) for research and related activities concerning cavernous angioma.

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

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

''(A) Proteomic, pharmacological, and cell biological analysis of the cerebral cavernous malformations (referred to in this section as the 'CCM') molecules.

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

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

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

"(E) Biological mechanisms for lesion bleeding and symptomology.

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

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

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

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

"(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.

"(2) FACILITATION OF RESEARCH RESOURCES; 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;

''(III) establishing an education program for elementary and secondary school nurses to facilitate early detection and diagnosis of cavernous angioma in areas of high cavernous angioma population density;

''(IV) coordinating regular patient and family-oriented educational conferences; and

''(V) developing nationally relevant electronic health teaching and communication tools and a network of professional capacity and patient and family support;

''(iii) have the capacity to establish and maintain communication with other major cavernous angioma research and care institutions internationally for information sharing and coordination of research activities;

''(iv) have demonstrated clinical expertise in cavernous angioma management;

''(v) have a sufficient number of eligible patients for participation with particular 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 consultation for remote and underserved communities.

"(B) PARTICIPATION.—Identify and support the development of clinical and research participation centers with the potential to participate 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 national and international centers. Such participation centers shall—

"(i) have an institutional infrastructure 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 expertise in cavernous angioma management; and

''(iv) have a sufficient numbers of eligible patients for participation with particular focus on unique subpopulations including 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 SCIENTISTS.—

''(1) IN GENERAL.—Eligible coordinating and participation centers under this section shall establish or expand training programs for medical and allied health clinicians and scientists in clinical practice 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 National Institutes of Health pursuant to other

provisions of law or prior to the date of the enactment of this section;

"(B) take into consideration the availability of other research resources;

"(C) encourage the use of research resources for research on, and development of, therapies and treatments for individuals with cavernous angioma; and

"(D) encourage the inclusion of individuals with cavernous angioma in clinical trials conducted or supported by the National Institutes of Health.

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

SEC. 4. CENTERS FOR DISEASE CONTROL AND PREVENTION CAVERNOUS ANGIOMA SURVEILLANCE AND RESEARCH PROGRAMS.

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

"SEC. 317U. CAVERNOUS ANGIOMA SURVEILLANCE AND RESEARCH PROGRAMS.

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

''(b) NATIONAL CAVERNOUS ANGIOMA EPIDEMIOLOGY PROGRAM.—

''(1) GRANTS.—The Secretary, acting through the Director of the Centers for Disease Control and Prevention, may award grants to public or nonprofit private entities (including State health departments, political subdivisions of States, universities, and other educational entities) for the purpose of carrying 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.

(b) ORPHAN PRODUCT DEVELOPMENT.—Where applicable in rare subpopulations of cavernous angioma requiring unique pharmacological intervention, including those with the Common Hispanic Mutation or CCM3 gene mutations, the Commissioner of Food and Drugs shall support appropriate requests for designations of drugs as orphan drugs under section 526 of the Federal Food, Drug, and Cosmetic Act.

SEC. 6. REPORT TO CONGRESS.

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

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