112TH CONGRESS
1ST SESSION

S. 1167

To amend the Public Health Service Act to improve the diagnosis and treatment of hereditary hemorrhagic telangiectasia, and for other purposes.

IN THE SENATE OF THE UNITED STATES

JUNE 9, 2011

Mr. JOHNSON of South Dakota (for himself and Mr. BINGAMAN) 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 improve the diagnosis and treatment of hereditary hemorrhagic telangiectasia, 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 ''Hereditary Hemorrhagic Telangiectasia Diagnosis and Treatment Act of 2011''.

SEC. 2. FINDINGS.

The Congress finds as follows:

(1) Hereditary hemorrhagic telangiectasia (''HHT'') is a largely undiagnosed or misdiagnosed vascular genetic bleeding disorder that causes abnormalities of the blood vessels. A person with HHT has the tendency to form blood vessels that lack the capillaries between an artery and vein. HHT can cause spontaneous hemorrhage or stroke when brain or lung arteriovenous malformations, which are tangled blood vessels, rupture unexpectedly in all age groups. In addition to hemorrhagic stroke, embolic stroke, and brain abscess occur in approximately 30 percent of individuals with HHT caused by artery-vein malformations in the lung (due to lack of capillaries between the arterial and venous systems which prevent or normally filter out clots and bacteria), causing disability and sudden premature death.

(2) One in 5,000 American children and adults suffer from HHT.

(3) Studies have found an increase in morbidity and mortality rate for individuals who suffer from HHT.

(4) Due to the widespread lack of knowledge, accurate diagnosis, and appropriate intervention, 90 percent of HHT-affected families are at risk for pre-

ventable life-threatening and disabling medical incidents such as stroke.

(5) Early detection, screening, and treatment can prevent premature deaths, spontaneous hemorrhage, hemorrhagic stroke, embolic stroke, brain abscess, and other long-term health care complications resulting from HHT.

(6) HHT is an important health condition with serious health consequences which are amenable to early identification and diagnosis with suitable tests, and acceptable and available treatments in established treatment centers.

(7) Timely identification and management of HHT cases is an important public health objective because it will save lives, prevent disability, and reduce direct and indirect health care costs expenditures.

(8) Without a new program for early detection, screening, and treatment, 14,000 children and adults who suffer from HHT in the population today will suffer premature death and disability.

SEC. 3. PURPOSE.

The purpose of this Act is to create a federally led and financed initiative for early diagnosis and appropriate treatment of hereditary hemorrhagic telangiectasia that

will result in the reduction of the suffering of families, prevent premature death and disability, and lower health care costs through proven treatment interventions.

SEC. 4. NATIONAL INSTITUTES OF HEALTH.

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. HEREDITARY HEMORRHAGIC TELANGIECTASIA.

''(a) HHT INITIATIVE.—

''(1) ESTABLISHMENT.—The Secretary shall establish and implement an HHT initiative to assist in coordinating activities to improve early detection, screening, and treatment of people who suffer from HHT. Such initiative shall focus on—

''(A) advancing research on the causes, diagnosis, and treatment of HHT, including through the conduct or support of such research; and

''(B) increasing physician and public awareness of HHT.

''(2) CONSULTATION.—In carrying out this subsection, the Secretary shall consult with the Director of the National Institutes of Health and the Director of the Centers for Disease Control and Prevention.

''(b) HHT COORDINATING COMMITTEE.—

''(1) ESTABLISHMENT.—Not later than 60 days after the date of the enactment of this section, the Secretary, in consultation with the Director of the National Institutes of Health, shall establish a committee to be known as the HHT Coordinating Committee.

''(2) MEMBERSHIP.—

''(A) IN GENERAL.—The members of the Committee shall be appointed by the Secretary, in consultation with the Director of the National Institutes of Health, and shall consist of 12 individuals who are experts in HHT or arteriovenous malformation (AVM) as follows:

''(i) Four representatives of HHT Treatment Centers of Excellence designated under section 317U(c)(1).

''(ii) Four experts in vascular, molecular, or basic science.

''(iii) Four representatives of the National Institutes of Health.

''(B) CHAIR.—The Secretary shall designate the Chair of the Committee from among its members.

''(C) INTERIM MEMBERS.—In place of the 4 members otherwise required to be appointed

under paragraph (2)(A)(i), the Secretary may appoint 4 experts in vascular, molecular, or basic science to serve as members of the Committee during the period preceding designation and establishment of HHT Treatment Centers of Excellence under section 317U.

''(D) PUBLICATION OF NAMES.—Not later than 30 days after the establishment of the Committee, the Secretary shall publish the names of the Chair and members of the Committee on the Website of the Department of Health and Human Services.

''(E) TERMS.—The members of the Committee shall each be appointed for a 3-year term and, at the end of each such term, may be reappointed.

''(F) VACANCIES.—A vacancy on the Committee shall be filled by the Secretary in the same manner in which the original appointment was made.

''(3) RESPONSIBILITIES.—The Committee shall develop and coordinate implementation of a plan to advance research and understanding of HHT by—

''(A) conducting or supporting basic, translational, and clinical research on HHT

across the relevant national research institutes, national centers, and offices of the National Institutes of Health, including the National Heart, Lung, and Blood Institute; the National Institute of Neurological Disorders and Stroke; the National Institutes of Diabetes and Digestive and Kidney Diseases; the Eunice Kennedy Shriver National Institute of Child Health and Human Development; the National Cancer Institute; and the Office of Rare Diseases; and

''(B) conducting evaluations and making recommendations to the Secretary, the Director of the National Institutes of Health, and the Director of the National Cancer Institute regarding the prioritization and award of National Institutes of Health research grants relating to HHT, including with respect to grants for—

''(i) expand understanding of HHT through basic, translational, and clinical research on the cause, diagnosis, prevention, control, and treatment of HHT;

''(ii) training programs on HHT for scientists and health professionals; and

''(iii) HHT genetic testing research to improve the accuracy of genetic testing.

''(c) DEFINITIONS.—In this section:

''(1) The term 'Committee' means the HHT Coordinating Committee established under subsection (b).

''(2) The term 'HHT' means hereditary hemorrhagic telangiectasia.''.

SEC. 5. CENTERS FOR DISEASE CONTROL AND PREVENTION.

Part B of title III of the Public Health Service Act is amended by inserting after section 317T (42 U.S.C. 247b–22) the following:

''SEC. 317U. HEREDITARY HEMORRHAGIC TELANGIECTASIA.

''(a) IN GENERAL.—With respect to hereditary hemorrhagic telangiectasia (in this section referred to as 'HHT'), the Director of the Centers for Disease Control and Prevention (in this section referred to as the 'Director') shall carry out the following activities:

''(1) The conduct of population screening described in subsection (c).

''(2) The identification and conduct of investigations to further develop and support guidelines

for diagnosis of, and intervention for, HHT, including cost-benefit studies.

''(3) The development of a standardized survey and screening tool on family history.

''(4) The establishment, in collaboration with a voluntary health organization representing HHT families, of an HHT resource center within the Centers for Disease Control and Prevention to provide comprehensive education on, and disseminate information about, HHT to health professionals, patients, industry, and the public.

''(5) The conduct or support of public awareness programs in collaboration with medical, genetic, and professional organizations to improve the education of health professionals about HHT.

''(b) COLLABORATIVE APPROACHES.—The Director shall carry out this section through collaborative approaches within the National Center on Birth Defects and Developmental Disabilities and the Division for Heart Disease and Stroke Prevention of the Centers for Disease Control and Prevention.

''(c) POPULATION SCREENING.—In carrying out population screening under subsection (a)(1), the Director shall—

''(1) designate and provide funding for a sufficient number of HHT Treatment Centers of Excellence to improve patient access to information, treatment, and care by HHT experts;

''(2) conduct surveillance through a regional population study, supplemented by sentinel health care provider or center surveillance, and administrative database analyses as useful to accurately identify—

''(A) the prevalence of HHT; and

''(B) the prevalence of hemorrhagic and embolic stroke and brain abscess, resulting from HHT;

''(3) include HHT screening questions in the Behavioral Risk Factor Surveillance System survey conducted by the Centers for Disease Control and Prevention in order to screen a broader population and more accurately determine the prevalence of HHT;

''(4) disseminate data collected under paragraph (2)(B) to the Paul Coverdell National Acute Stroke Registry, to be utilized for analyses of natural history of hemorrhagic and embolic stroke in HHT, and to develop screening and artery-vein mal-

formation treatment guidelines specific to prevention of complications from HHT;

''(5) develop and implement programs, targeted for physicians and health care professional groups likely to be accessed by families with HHT, to increase HHT diagnosis and treatment rates through the—

''(A) establishment of a partnership with HHT Treatment Centers of Excellence designated under paragraph (1) through the creation of an international database of patients assessed at such HHT Treatment Centers of Excellence (including with respect to phenotype information, genotype information, transfusion dependence, and radiological findings);

''(B) integration of such database with the universal data collection system used by the Centers for monitoring hemophilia with the blood disorders and the Paul Coverdell National Acute Stroke Registry; and

''(C) inclusion of other medical providers who treat HHT patients; and

''(6) use existing administrative databases on non-HHT Treatment Center of Excellence patients to learn about the natural history of HHT, the effi-

cacy of various treatment modalities, and to better inform and develop screening and treatment guidelines associated with improvement in health care outcomes, and research priorities relevant to HHT.

''(d) ELIGIBILITY FOR DESIGNATION AS HHT TREATMENT CENTER OF EXCELLENCE.—In carrying out subsection (c)(1), the Director may designate as an HHT Treatment Center of Excellence only academic health centers demonstrating each of the following:

''(1) The academic health center possesses a team of medical experts capable of providing comprehensive evaluation, treatment, and education to individuals with known or suspected HHT and their health care providers.

''(2) The academic health center has sufficient personnel with knowledge about HHT, or formal collaboration with partnering organizations for personnel or resources, to be able to—

''(A) respond in a coordinated, multidisciplinary way to patient inquiries; and

''(B) coordinate evaluation, treatment, and education of patients and their families in a timely manner.

''(3) The academic health center has the following personnel, facilities, and patient volume:

''(A) A medical director with—

''(i) specialized knowledge of the main organ manifestations of HHT; and

''(ii) the ability to coordinate the multidisciplinary diagnosis and treatment of patients referred to the center.

''(B) Administrative staff with—

''(i) sufficient knowledge to respond to patient inquiries and coordinate patient care in a timely fashion; and

''(ii) adequate financial support to allow the staff to commit at least 25 to 50 percent of their time on the job to HHT.

''(C) An otolaryngologist with experience and expertise in the treatment of recurrent epistaxis in HHT patients.

''(D) An interventional radiologist with experience and expertise in the treatment of pulmonary arteriovenous malformations (AVM).

''(E) A genetic counselor or geneticist with the expertise to provide HHT-specific genetic counseling to patients and families.

''(F) On-site facilities to screen for all major organ manifestations of HHT.

''(G) A patient volume of at least 25 new HHT patients per year.

''(H) Established mechanisms to coordinate surveillance and outreach with HHT patient advocacy organizations.''.

SEC. 6. ADDITIONAL HEALTH AND HUMAN SERVICES ACTIVITIES.

With respect to hereditary hemorrhagic telangiectasia (in this sec referred to as ''HHT''), the Secretary of Health and Human Services, acting through the Administrator of the Centers for Medicare & Medicaid Services, shall award grants on a competitive basis—

(1) for an analysis by grantees of the Medicare Provider Analysis and Review (MEDPAR) file to develop preliminary estimates on the total costs to the Medicare program under title XVIII of the Social Security Act for items, services, and treatments for HHT furnished to individuals with HHT who are entitled to benefits under part A of title XVIII of the Social Security Act or enrolled under part B of such title; and

(2) to make recommendations regarding an enhanced data collection protocol to permit a more precise determination of the total costs described in paragraph (1).

SEC. 7. AUTHORIZATION OF APPROPRIATIONS.

(a) IN GENERAL.—To carry out section 409K of the Public Health Service Act as added by section 4 of this Act, section 317U of the Public Health Service Act as added by section 5 of this Act, and section 6 of this Act, there is authorized to be appropriated $5,000,000 for each of fiscal years 2012 through 2016.

(b) RESOURCE CENTER.—Of the amount authorized to be appropriated under subsection (a) for each of fiscal years 2012 through 2016, $1,000,000 shall be for carrying out section 317U(a)(4) of the Public Health Service Act, as added by section 5 of this Act.

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