

113TH CONGRESS
1ST SESSION

S. 908

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

MAY 8, 2013

Mr. JOHNSON of South Dakota 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 improve the diagnosis and treatment of hereditary hemorrhagic telangiectasia, 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 “Hereditary Hemor-
5 rhagic Telangiectasia Diagnosis and Treatment Act of
6 2013”.

7 **SEC. 2. FINDINGS.**

8 The Congress finds as follows:

1 (1) Hereditary hemorrhagic telangiectasia (re-
2 ferred to in this section as “HHT”) is a largely
3 undiagnosed or misdiagnosed vascular genetic bleed-
4 ing disorder resulting in artery-vein malformations
5 (referred to in this section as “AVMs”), which lead
6 to preventable, catastrophic and disabling con-
7 sequences. HHT can cause sudden death at any age,
8 unless detected and treated. Early detection, screen-
9 ing, and use of readily available treatment can pre-
10 vent premature deaths and long-term health com-
11 plications resulting from HHT. A person with HHT
12 has the tendency to form blood vessels that lack the
13 capillaries between an artery and vein. HHT often
14 results in spontaneous hemorrhage or stroke from
15 brain or lung AVMs. In addition to hemorrhagic
16 stroke, embolic stroke and brain abscess occur in ap-
17 proximately 30 percent of individuals with HHT
18 AVMs in the lung (due to lack of capillaries between
19 the arterial and venous systems that normally filter
20 out clots and bacteria).

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

23 (3) Studies have found an increase in morbidity
24 and mortality rates for individuals who suffer from
25 HHT.

1 (4) Due to the widespread lack of knowledge,
2 accurate diagnosis, and appropriate intervention, 90
3 percent of HHT-affected families are at risk for pre-
4 ventable life-threatening and disabling medical inci-
5 dents such as stroke.

6 (5) Early detection, screening, and treatment
7 can prevent premature deaths, spontaneous hemor-
8 rhage, hemorrhagic stroke, embolic stroke, brain ab-
9 scess, and other long-term health care complications
10 resulting from HHT.

11 (6) HHT is an important health condition with
12 serious health consequences which are amenable to
13 early identification and diagnosis with suitable tests,
14 and acceptable and available treatments in estab-
15 lished treatment centers.

16 (7) Timely identification and management of
17 HHT cases is an important public health objective
18 because it will save lives, prevent disability, and re-
19 duce direct and indirect health care costs expendi-
20 tures. A recent study has found use of a genetic
21 testing model for HHT diagnosis saves \$9,900,000
22 if screening is limited to individuals within the fam-
23 ily groups that have the gene defect, leading to early
24 intervention in individuals found to have treatable
25 AVMs.

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

5 **SEC. 3. PURPOSE.**

6 The purpose of this Act is to create a federally led
7 and financed initiative for early diagnosis and appropriate
8 treatment of hereditary hemorrhagic telangiectasia that
9 will result in the reduction of the suffering of families,
10 prevent premature death and disability, and lower health
11 care costs through proven treatment interventions.

12 **SEC. 4. NATIONAL INSTITUTES OF HEALTH.**

13 Part B of title IV of the Public Health Service Act
14 (42 U.S.C. 284 et seq.) is amended by adding at the end
15 the following:

16 **“SEC. 409K. HEREDITARY HEMORRHAGIC TELANGIECTASIA.**

17 “(a) HHT INITIATIVE.—

18 “(1) ESTABLISHMENT.—The Secretary shall es-
19 tablish and implement an HHT initiative to assist in
20 coordinating activities to improve early detection,
21 screening, and treatment of people who suffer from
22 HHT. Such initiative shall focus on—

23 “(A) advancing research on the causes, di-
24 agnosis, and treatment of HHT, including

1 through the conduct or support of such re-
2 search; and

3 “(B) increasing physician and public
4 awareness of HHT.

5 “(2) CONSULTATION.—In carrying out this sub-
6 section, the Secretary shall consult with the Director
7 of the National Institutes of Health and the Director
8 of the Centers for Disease Control and Prevention.

9 “(b) HHT COORDINATING COMMITTEE.—

10 “(1) ESTABLISHMENT.—Not later than 60 days
11 after the date of the enactment of this section, the
12 Secretary, in consultation with the Director of the
13 National Institutes of Health, shall establish a com-
14 mittee to be known as the HHT Coordinating Com-
15 mittee.

16 “(2) MEMBERSHIP.—

17 “(A) IN GENERAL.—The members of the
18 Committee shall be appointed by the Secretary,
19 in consultation with the Director of the Na-
20 tional Institutes of Health, and shall consist of
21 12 individuals who are experts in HHT or
22 arteriovenous malformation (AVM) as follows:

23 “(i) Four representatives of HHT
24 Treatment Centers of Excellence des-
25 ignated under section 317U(c)(1).

1 “(ii) Four experts in vascular, molec-
2 ular, or basic science.

3 “(iii) Four representatives of the Na-
4 tional Institutes of Health.

5 “(B) CHAIR.—The Secretary shall des-
6 ignate the Chair of the Committee from among
7 its members.

8 “(C) INTERIM MEMBERS.—In place of the
9 4 members otherwise required to be appointed
10 under subparagraph (A)(i), the Secretary may
11 appoint 4 experts in vascular, molecular, or
12 basic science to serve as members of the Com-
13 mittee during the period preceding designation
14 and establishment of HHT Treatment Centers
15 of Excellence under section 317U.

16 “(D) PUBLICATION OF NAMES.—Not later
17 than 30 days after the establishment of the
18 Committee, the Secretary shall publish the
19 names of the Chair and members of the Com-
20 mittee on the Website of the Department of
21 Health and Human Services.

22 “(E) TERMS.—The members of the Com-
23 mittee shall each be appointed for a 3-year term
24 and, at the end of each such term, may be re-
25 appointed.

1 “(F) VACANCIES.—A vacancy on the Com-
2 mittee shall be filled by the Secretary in the
3 same manner in which the original appointment
4 was made.

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

8 “(A) conducting or supporting basic,
9 translational, and clinical research on HHT
10 across the relevant national research institutes,
11 national centers, and offices of the National In-
12 stitutes of Health, including the National
13 Heart, Lung, and Blood Institute; the National
14 Institute of Neurological Disorders and Stroke;
15 the National Institutes of Diabetes and Diges-
16 tive and Kidney Diseases; the Eunice Kennedy
17 Shriver National Institute of Child Health and
18 Human Development; the National Cancer In-
19 stitute; and the Office of Rare Diseases; and

20 “(B) conducting evaluations and making
21 recommendations to the Secretary, the Director
22 of the National Institutes of Health, and the
23 Director of the National Cancer Institute re-
24 garding the prioritization and award of Na-
25 tional Institutes of Health research grants re-

lating to HHT, including with respect to grants
for—

“(i) expand understanding of HHT
through basic, translational, and clinical
research on the cause, diagnosis, preven-
tion, 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 sub-
section (b).

“(2) The term ‘HHT’ means hereditary hemor-
rhagic telangiectasia.”.

**SEC. 5. CENTERS FOR DISEASE CONTROL AND PREVEN-
TION.**

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 hem-
orrhagic telangiectasia (in this section referred to as

1 ‘HHT’), the Director of the Centers for Disease Control
2 and Prevention (in this section referred to as the ‘Direc-
3 tor’) shall carry out the following activities:

4 “(1) The conduct of population screening de-
5 scribed in subsection (c).

6 “(2) The identification and conduct of inves-
7 tigations to further develop and support guidelines
8 for diagnosis of, and intervention for, HHT, includ-
9 ing cost-benefit studies.

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

12 “(4) The establishment, in collaboration with a
13 voluntary health organization representing HHT
14 families, of an HHT resource center within the Cen-
15 ters for Disease Control and Prevention to provide
16 comprehensive education on, and disseminate infor-
17 mation about, HHT to health professionals, pa-
18 tients, industry, and the public.

19 “(5) The conduct or support of public aware-
20 ness programs in collaboration with medical, genetic,
21 and professional organizations to improve the edu-
22 cation of health professionals about HHT.

23 “(b) COLLABORATIVE APPROACHES.—The Director
24 shall carry out this section through collaborative ap-
25 proaches within the National Center on Birth Defects and

1 Developmental Disabilities and the Division for Heart Dis-
 2 ease and Stroke Prevention of the Centers for Disease
 3 Control and Prevention with respect to clotting and bleed-
 4 ing disorders.

5 “(c) POPULATION SCREENING.—In carrying out pop-
 6 ulation screening under subsection (a)(1), the Director
 7 shall—

8 “(1) designate and provide funding for a suffi-
 9 cient number of HHT Treatment Centers of Excel-
 10 lence to improve patient access to information, treat-
 11 ment, and care by HHT experts;

12 “(2) conduct surveillance through a population
 13 study, supplemented by sentinel health care provider
 14 or center surveillance, and by administrative data-
 15 base analyses, as useful, to accurately identify—

16 “(A) the prevalence of HHT; and

17 “(B) the prevalence of hemorrhagic and
 18 embolic stroke and brain abscess, resulting
 19 from HHT;

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

1 “(4) provide data collected under paragraph
2 (2)(B) to the Paul Coverdell National Acute Stroke
3 Registry, to facilitate—

4 “(A) analyses of the natural history of
5 hemorrhagic and embolic stroke in HHT; and

6 “(B) the development of screening and ar-
7 tery-vein malformation treatment guidelines
8 specific to prevention of complications from
9 HHT;

10 “(5) develop and implement programs, targeted
11 for physicians and health care professional groups
12 likely to be accessed by families with HHT, to in-
13 crease HHT diagnosis and treatment rates through
14 the—

15 “(A) establishment of a partnership with
16 HHT Treatment Centers of Excellence des-
17 ignated under paragraph (1) through the cre-
18 ation of a database of patients assessed at such
19 HHT Treatment Centers of Excellence (includ-
20 ing with respect to phenotype information, gen-
21 otype information, transfusion dependence, and
22 radiological findings);

23 “(B) integration of such database with—

1 “(i) the universal data collection sys-
 2 tem used by the Centers for monitoring he-
 3 mophilia with the blood disorders; and

4 “(ii) the Paul Coverdell National
 5 Acute Stroke Registry; and

6 “(C) inclusion of other medical providers
 7 who treat HHT patients; and

8 “(6) use existing administrative databases on
 9 non-HHT Treatment Center of Excellence pa-
 10 tients—

11 “(A) to learn about the natural history of
 12 HHT and the efficacy of various treatment mo-
 13 dalities; and

14 “(B) to better inform and develop screen-
 15 ing and treatment guidelines associated with
 16 improvement in health care outcomes, and re-
 17 search priorities relevant to HHT.

18 “(d) ELIGIBILITY FOR DESIGNATION AS HHT
 19 TREATMENT CENTER OF EXCELLENCE.—In carrying out
 20 subsection (c)(1), the Director may designate as an HHT
 21 Treatment Center of Excellence an academic health center
 22 demonstrating each of the following:

23 “(1) The academic health center possesses a
 24 team of medical experts capable of providing com-
 25 prehensive evaluation, treatment, and education to

1 individuals with known or suspected HHT and their
2 health care providers.

3 “(2) The academic health center has sufficient
4 personnel with knowledge about HHT, or formal col-
5 laboration with one or more partnering organizations
6 for personnel or resources, to be able to—

7 “(A) respond in a coordinated, multidisci-
8 plinary way to patient inquiries; and

9 “(B) coordinate evaluation, treatment, and
10 education of patients and their families in a
11 timely manner.

12 “(3) The academic health center has the fol-
13 lowing personnel, facilities, and patient volume:

14 “(A) A medical director with—

15 “(i) specialized knowledge of the main
16 organ manifestations of HHT; and

17 “(ii) the ability to coordinate the mul-
18 tidisciplinary diagnosis and treatment of
19 patients referred to the center.

20 “(B) Administrative staff with—

21 “(i) sufficient knowledge to respond to
22 patient inquiries and coordinate patient
23 care in a timely fashion; and

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

4 “(C) An otolaryngologist with experience
5 and expertise in the treatment of recurrent epi-
6 staxis in HHT patients.

7 “(D) An interventional radiologist with ex-
8 perience and expertise in the treatment of pul-
9 monary arteriovenous malformations (AVM).

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

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

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

17 “(H) Established mechanisms to coordi-
18 nate surveillance and outreach with HHT pa-
19 tient advocacy organizations.”.

20 **SEC. 6. ADDITIONAL HEALTH AND HUMAN SERVICES AC-**
21 **TIVITIES.**

22 With respect to hereditary hemorrhagic telangiectasia
23 (referred to in this section as “HHT”), the Secretary of
24 Health and Human Services, acting through the Adminis-

1 trator of the Centers for Medicare & Medicaid Services,
 2 shall award grants on a competitive basis—

3 (1) for an analysis by grantees of the Medicare
 4 Provider Analysis and Review (MEDPAR) file to de-
 5 velop preliminary estimates from the Medicare pro-
 6 gram under title XVIII of the Social Security Act
 7 for—

8 (A) preventable costs of annual health care
 9 expenditures associated with untreated HHT
 10 furnished to individuals with HHT, including
 11 items, services, and treatments; and

12 (B) socioeconomic costs associated with
 13 preventable medical events among individuals
 14 with HHT who are entitled to benefits under
 15 part A of title XVIII of the Social Security Act
 16 or enrolled under part B of such title, such as
 17 disability expenditures; and

18 (2) to make recommendations regarding an en-
 19 hanced data collection protocol to permit a more
 20 precise determination of the total costs described in
 21 paragraph (1).

22 **SEC. 7. AUTHORIZATION OF APPROPRIATIONS.**

23 (a) IN GENERAL.—To carry out section 409K of the
 24 Public Health Service Act as added by section 4 of this
 25 Act, section 317U of the Public Health Service Act as

1 added by section 5 of this Act, and section 6 of this Act,
2 there is authorized to be appropriated \$5,000,000 for each
3 of fiscal years 2014 through 2018.

4 (b) RESOURCE CENTER.—Of the amount authorized
5 to be appropriated under subsection (a) for each of fiscal
6 years 2014 through 2018, \$1,000,000 shall be for car-
7 rying out section 317U(a)(4) of the Public Health Service
8 Act, as added by section 5 of this Act.

○