

113TH CONGRESS
2D SESSION

H. R. 4592

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

IN THE HOUSE OF REPRESENTATIVES

MAY 7, 2014

Mr. GERLACH (for himself and Mr. HIMES) introduced the following bill; which was referred to the Committee on Energy and Commerce, and in addition to the Committee on Ways and Means, for a period to be subsequently determined by the Speaker, in each case for consideration of such provisions as fall within the jurisdiction of the committee concerned

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 2014”.

7 **SEC. 2. FINDINGS.**

8 The Congress finds as follows:

1 (1) Hereditary hemorrhagic telangiectasia
2 (HHT) is a largely undiagnosed or misdiagnosed
3 vascular genetic bleeding disorder resulting in ar-
4 tery-vein malformations (AVMs) which lead to pre-
5 ventable catastrophic and disabling consequences.
6 HHT can cause sudden death at any age, unless de-
7 tected and treated. Early detection, screening, and
8 use of readily available treatment can prevent pre-
9 mature deaths and long-term health complications
10 resulting from HHT. A person with HHT has the
11 tendency to form blood vessels that lack the cap-
12 illaries between an artery and vein. HHT often re-
13 sults in spontaneous hemorrhage or stroke from
14 brain or lung AVM. In addition to hemorrhagic
15 stroke, embolic stroke and brain abscess occur in ap-
16 proximately 30 percent of persons with HHT artery-
17 vein malformations in the lung (due to lack of cap-
18 illaries between the arterial and venous systems
19 which normally filter out clots and bacteria).

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

22 (3) Studies have found an increase in morbidity
23 and mortality rates for individuals who suffer from
24 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 \$9.9 million
22 in that screening can be limited to those persons
23 within the family groups who actually have the gene
24 defect, leading to early intervention in those found
25 to have treatable AVMs.

(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.

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. CENTERS FOR DISEASE CONTROL AND PREVEN-
13 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:

17 “SEC. 317U. HEREDITARY HEMORRHAGIC
18 TELANGIECTASIA.

“(a) IN GENERAL.—With respect to hereditary hem-
orrhagic 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 ‘Direc-
tor’) shall carry out the following activities:

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

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

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

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

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

18 “(b) COLLABORATIVE APPROACHES.—The Director
19 shall carry out this section through collaborative ap-
20 proaches within the National Center on Birth Defects and
21 Developmental Disabilities and the Division for Heart Dis-
22 ease and Stroke Prevention of the Centers for Disease
23 Control and Prevention for clotting and bleeding dis-
24 orders.

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

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

8 “(2) conduct surveillance by means of a popu-
9 lation study, supplemented by sentinel health care
10 provider or center surveillance, and by administra-
11 tive database analyses as useful, to accurately iden-
12 tify—

13 “(A) the prevalence of HHT; and

14 “(B) the prevalence of hemorrhagic and
15 embolic stroke, and brain abscess, resulting
16 from HHT;

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

23 “(4) provide data collected under paragraph
24 (2)(B) to the Paul Coverdell National Acute Stroke
25 Registry to facilitate—

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

3 “(B) development of screening and artery-
4 vein malformation treatment guidelines specific
5 to prevention of complications from HHT;

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

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

19 “(B) integration of such database with—

20 “(i) the universal data collection sys-
21 tem used by the Centers for monitoring he-
22 mophilia with the blood disorders; and

23 “(ii) the Paul Coverdell National
24 Acute Stroke Registry; and

1 “(C) inclusion of other medical providers
2 who treat HHT patients; and

3 “(6) use existing administrative databases on
4 non-HHT Treatment Centers of Excellence pa-
5 tients—

6 “(A) to learn about the natural history of
7 HHT and the efficacy of various treatment mo-
8 dalities; and

9 “(B) to better inform and develop screen-
10 ing and treatment guidelines associated with
11 improvement in health care outcomes, and re-
12 search priorities relevant to HHT.

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

18 “(1) The academic health center possesses a
19 team of medical experts capable of providing com-
20 prehensive evaluation, treatment, and education to
21 individuals with known or suspected HHT and their
22 health care providers.

23 “(2) The academic health center has sufficient
24 personnel with knowledge about HHT, or formal col-

1 laboration with one or more partnering organizations
2 for personnel or resources, to be able to—

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

5 “(B) coordinate evaluation, treatment, and
6 education of patients and their families in a
7 timely manner.

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

10 “(A) A medical director with—

11 “(i) specialized knowledge of the main
12 organ manifestations of HHT; and

13 “(ii) the ability to coordinate the mul-
14 tidisciplinary diagnosis and treatment of
15 patients referred to the center.

16 “(B) Administrative staff with—

17 “(i) sufficient knowledge to respond to
18 patient inquiries and coordinate patient
19 care in a timely fashion; and

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

23 “(C) An otolaryngologist with experience
24 and expertise in the treatment of recurrent epi-
25 staxis in HHT patients.

1 “(D) An interventional radiologist with ex-
 2 perience and expertise in the treatment of pul-
 3 monary arteriovenous malformations (AVM).

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

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

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

11 “(H) Established mechanisms to coordi-
 12 nate surveillance and outreach with HHT pa-
 13 tient advocacy organizations.”.

14 **SEC. 5. ADDITIONAL HEALTH AND HUMAN SERVICES AC-**
 15 **TIVITIES.**

16 With respect to hereditary hemorrhagic telangiectasia
 17 (in this section referred to as “HHT”), the Secretary of
 18 Health and Human Services, acting through the Adminis-
 19 trator of the Centers for Medicare & Medicaid Services,
 20 shall award grants on a competitive basis—

21 (1) for an analysis by grantees of the Medicare
 22 Provider Analysis and Review (MEDPAR) file to de-
 23 velop preliminary estimates from the Medicare pro-
 24 gram under title XVIII of the Social Security Act
 25 for preventable costs of annual health care expendi-

1 tures including items, services, and treatments asso-
2 ciated with untreated HHT furnished to individuals
3 with HHT, as well as socioeconomic costs such as
4 disability expenditures associated with preventable
5 medical events in this population, who are entitled to
6 benefits under part A of title XVIII of the Social Se-
7 curity Act or enrolled under part B of such title; and

8 (2) to make recommendations regarding an en-
9 hanced data collection protocol to permit a more
10 precise determination of the total costs described in
11 paragraph (1).

12 **SEC. 6. 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-

1 lating to HHT, including with respect to grants
2 for—

3 “(i) expanding understanding of HHT
4 through basic, translational, and clinical
5 research on the cause, diagnosis, preven-
6 tion, control, and treatment of HHT;

7 “(ii) training programs on HHT for
8 scientists and health professionals; and

9 “(iii) HHT genetic testing research to
10 improve the accuracy of genetic testing.

11 “(c) DEFINITIONS.—In this section:

12 “(1) The term ‘Committee’ means the HHT
13 Coordinating Committee established under sub-
14 section (b).

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

17 **SEC. 7. AUTHORIZATION OF APPROPRIATIONS.**

18 (a) IN GENERAL.—To carry out section 317U of the
19 Public Health Service Act as added by section 4 of this
20 Act, section 5 of this Act, and section 409K of the Public
21 Health Service Act as added by section 6 of this Act, there
22 is authorized to be appropriated \$5,000,000 for each of
23 fiscal years 2015 through 2019.

24 (b) RESOURCE CENTER.—Of the amount authorized
25 to be appropriated under subsection (a) for each of fiscal

1 years 2015 through 2019, \$1,000,000 shall be for car-
2 rying out section 317U(a)(4) of the Public Health Service
3 Act, as added by section 4 of this Act.

4 (c) OFFSET.—There is authorized to be appropriated
5 to the Department of Health and Human Services for sal-
6 aries and expenses of the Department for each of fiscal
7 years 2015 through 2019 the amount that is \$5,000,000
8 less than the amount appropriated for such salaries and
9 expenses for fiscal year 2014.

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