

111TH CONGRESS
1ST SESSION

H. R. 1230

To amend the Public Health Service Act to provide for the establishment of a National Acquired Bone Marrow Failure Disease Registry, to authorize research on acquired bone marrow failure diseases, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

FEBRUARY 26, 2009

Ms. MATSUI (for herself, Mr. BISHOP of Georgia, Mr. MURTHA, Mr. McDERMOTT, Mrs. EMERSON, Mr. GENE GREEN of Texas, Mr. ABERCROMBIE, Ms. SCHWARTZ, Mr. CONYERS, Ms. HIRONO, Ms. BORDALLO, Mr. LEWIS of Georgia, Mr. FRANK of Massachusetts, Mr. WOLF, Mr. KING of New York, Mr. VAN HOLLEN, Mr. MCGOVERN, Ms. DEGETTE, Mr. ARCURI, and Ms. ESHOO) introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To amend the Public Health Service Act to provide for the establishment of a National Acquired Bone Marrow Failure Disease Registry, to authorize research on acquired bone marrow failure diseases, 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 “Bone Marrow Failure
5 Disease Research and Treatment Act of 2009”.

1 **SEC. 2. FINDINGS.**

2 The Congress finds the following:

3 (1) Between 20,000 and 30,000 Americans are
4 diagnosed each year with myelodysplastic syndromes,
5 aplastic anemia, paroxysmal nocturnal hemo-
6 globinuria, and other acquired bone marrow failure
7 diseases.

8 (2) Acquired bone marrow failure diseases have
9 a debilitating and often fatal impact on those diag-
10 nosed with these disease.

11 (3) While some treatments for acquired bone
12 marrow failure diseases can prolong and improve the
13 quality of patients' lives, there is no single cure for
14 these diseases.

15 (4) The prevalence of acquired bone marrow
16 failure diseases in the United States will continue to
17 grow as the general public ages.

18 (5) Evidence exists suggesting that acquired
19 bone marrow failure diseases occur more often in
20 minority populations, particularly in Asian-American
21 and Hispanic/Latino populations.

22 (6) The National Heart, Lung, and Blood Insti-
23 tute and the National Cancer Institute have con-
24 ducted important research into the causes of and
25 treatments for acquired bone marrow failure dis-
26 eases.

1 (7) The National Marrow Donor Program Reg-
2 istry has made significant contributions to the fight
3 against bone marrow failure diseases by connecting
4 millions of potential marrow donors with individuals
5 and families suffering from these conditions.

6 (8) Despite these advances, a more comprehen-
7 sive Federal strategic effort among numerous Fed-
8 eral agencies is needed to discover a cure for ac-
9 quired bone marrow failure disorders.

10 (9) Greater Federal surveillance of acquired
11 bone marrow failure diseases is needed to gain a bet-
12 ter understanding of the causes of acquired bone
13 marrow failure diseases.

14 (10) The Federal Government should increase
15 its research support for and engage with public and
16 private organizations in developing a comprehensive
17 approach to combat and cure acquired bone marrow
18 failure diseases.

19 **SEC. 3. NATIONAL ACQUIRED BONE MARROW FAILURE DIS-**
20 **EASE REGISTRY.**

21 Part B of the Public Health Service Act (42 U.S.C.
22 311 et seq.) is amended by inserting after section 317T
23 the following:

1 **“SEC. 317U. NATIONAL ACQUIRED BONE MARROW FAILURE**
2 **DISEASE REGISTRY.**

3 “(a) ESTABLISHMENT OF REGISTRY.—

4 “(1) IN GENERAL.—Not later than 6 months
5 after the date of the enactment of this section, the
6 Secretary, acting through the Director of the Cen-
7 ters for Disease Control and Prevention, shall—

8 “(A) develop a system to collect data on
9 acquired bone marrow failure diseases; and

10 “(B) establish and maintain a national and
11 publicly available registry, to be known as the
12 National Acquired Bone Marrow Failure Dis-
13 ease Registry, in accordance with paragraph
14 (3).

15 “(2) RECOMMENDATIONS OF ADVISORY COM-
16 MITTEE.—In carrying out this subsection, the Sec-
17 retary shall take into consideration the recommenda-
18 tions of the Advisory Committee on Acquired Bone
19 Marrow Failure Diseases established under sub-
20 section (b).

21 “(3) PURPOSES OF REGISTRY.—The National
22 Acquired Bone Marrow Failure Disease Registry—

23 “(A) shall identify the incidence and preva-
24 lence of acquired bone marrow failure diseases
25 in the United States;

1 “(B) shall be used to collect and store data
2 on acquired bone marrow failure diseases, in-
3 cluding data concerning—

4 “(i) the age, race or ethnicity, general
5 geographic location, sex, and family history
6 of individuals who are diagnosed with ac-
7 quired bone marrow failure diseases, and
8 any other characteristics of such individ-
9 uals determined appropriate by the Sec-
10 retary;

11 “(ii) the genetic and environmental
12 factors that may be associated with devel-
13 oping acquired bone marrow failure dis-
14 eases;

15 “(iii) treatment approaches for deal-
16 ing with acquired bone marrow failure dis-
17 eases;

18 “(iv) outcomes for individuals treated
19 for acquired bone marrow failure diseases,
20 including outcomes for recipients of stem
21 cell therapeutic products as contained in
22 the database established pursuant to sec-
23 tion 379A; and

1 “(v) any other factors pertaining to
2 acquired bone marrow failure diseases de-
3 termined appropriate by the Secretary; and

4 “(C) shall be made available—

5 “(i) to the general public; and

6 “(ii) to researchers to facilitate fur-
7 ther research into the causes of, and treat-
8 ments for, acquired bone marrow failure
9 diseases in accordance with standard prac-
10 tices of the Centers for Disease Control
11 and Preventions.

12 “(b) ADVISORY COMMITTEE.—

13 “(1) ESTABLISHMENT.—Not later than 6
14 months after the date of the enactment of this sec-
15 tion, the Secretary, acting through the Director of
16 the Centers for Disease Control and Prevention,
17 shall establish an advisory committee, to be known
18 as the Advisory Committee on Acquired Bone Mar-
19 row Failure Diseases.

20 “(2) MEMBERS.—The members of the Advisory
21 Committee on Acquired Bone Marrow Failure Dis-
22 eases shall be appointed by the Secretary, acting
23 through the Director of the Centers for Disease
24 Control and Prevention, and shall include at least
25 one representative from each of the following:

1 “(A) A national patient advocacy organiza-
2 tion with experience advocating on behalf of pa-
3 tients suffering from acquired bone marrow
4 failure diseases.

5 “(B) The National Institutes of Health, in-
6 cluding at least one representative from each
7 of—

8 “(i) the National Cancer Institute;

9 “(ii) the National Heart, Lung, and
10 Blood Institute; and

11 “(iii) the Office of Rare Diseases.

12 “(C) The Centers for Disease Control and
13 Prevention.

14 “(D) Clinicians with experience in—

15 “(i) diagnosing or treating acquired
16 bone marrow failure diseases; and

17 “(ii) medical data registries.

18 “(E) Epidemiologists who have experience
19 with data registries.

20 “(F) Publicly or privately funded research-
21 ers who have experience researching acquired
22 bone marrow failure diseases.

23 “(G) The entity operating the C.W. Bill
24 Young Cell Transplantation Program estab-
25 lished pursuant to section 379 and the entity

1 operating the C.W. Bill Young Cell Transplan-
2 tation Program Outcomes Database.

3 “(3) RESPONSIBILITIES.—The Advisory Com-
4 mittee on Acquired Bone Marrow Failure Diseases
5 shall provide recommendations to the Secretary on
6 the establishment and maintenance of the National
7 Acquired Bone Marrow Failure Disease Registry, in-
8 cluding recommendations on the collection, mainte-
9 nance, and dissemination of data.

10 “(4) PUBLIC AVAILABILITY.—The Secretary
11 shall make the recommendations of the Advisory
12 Committee on Acquired Bone Marrow Failure Dis-
13 ease publicly available.

14 “(c) GRANTS.—The Secretary, acting through the
15 Director of the Centers for Disease Control and Preven-
16 tion, may award grants to, and enter into contracts and
17 cooperative agreements with, public or private nonprofit
18 entities for the management of, as well as the collection,
19 analysis, and reporting of data to be included in, the Na-
20 tional Acquired Bone Marrow Failure Disease Registry.

21 “(d) DEFINITION.—In this section, the term ‘ac-
22 quired bone marrow failure disease’ means—

23 “(1) myelodysplastic syndromes (MDS);

24 “(2) aplastic anemia;

1 “(3) paroxysmal nocturnal hemoglobinuria
2 (PNH);

3 “(4) pure red cell aplasia;

4 “(5) acute myeloid leukemia that has pro-
5 gressed from myelodysplastic syndromes; or

6 “(6) large granular lymphocytic leukemia.

7 “(e) AUTHORIZATION OF APPROPRIATIONS.—There
8 is authorized to be appropriated to carry out this section
9 \$3,000,000 for each of fiscal years 2010 through 2014.”.

10 **SEC. 4. PILOT STUDIES THROUGH THE AGENCY FOR TOXIC**
11 **SUBSTANCES AND DISEASE REGISTRY.**

12 (a) PILOT STUDIES.—The Secretary of Health and
13 Human Services, acting through the Administrator of the
14 Agency for Toxic Substances and Disease Registry, shall
15 conduct pilot studies to determine which environmental
16 factors, including exposure to toxins, may cause acquired
17 bone marrow failure diseases.

18 (b) COLLABORATION WITH THE RADIATION INJURY
19 TREATMENT NETWORK.—In carrying out the directives of
20 this section, the Secretary may collaborate with the Radi-
21 ation Injury Treatment Network of the C.W. Bill Young
22 Cell Transplantation Program established pursuant to sec-
23 tion 379 of the Public Health Service Act (42 U.S.C.
24 274j) to—

1 (1) augment data for the pilot studies author-
2 ized by this section;

3 (2) access technical assistance that may be pro-
4 vided by the Radiation Injury Treatment Network;
5 or

6 (3) perform joint research projects.

7 (c) AUTHORIZATION OF APPROPRIATIONS.—There is
8 authorized to be appropriated to carry out this section
9 \$1,000,000 for each of fiscal years 2010 through 2014.

10 **SEC. 5. MINORITY-FOCUSED PROGRAMS ON ACQUIRED**
11 **BONE MARROW FAILURE DISEASES.**

12 Title XVII of the Public Health Service Act (42
13 U.S.C. 300u et seq.) is amended by inserting after section
14 1707 the following:

15 “MINORITY-FOCUSED PROGRAMS ON ACQUIRED BONE
16 MARROW FAILURE DISEASES

17 “SEC. 1707A. (a) INFORMATION AND REFERRAL
18 SERVICES.—

19 “(1) IN GENERAL.—Not later than 6 months
20 after the date of the enactment of this section, the
21 Secretary, acting through the Deputy Assistant Sec-
22 retary for Minority Health, shall establish and co-
23 ordinate outreach and informational programs tar-
24 geted to minority populations affected by acquired
25 bone marrow failure diseases.

1 “(2) PROGRAM REQUIREMENTS.—Minority-foc-
2 cused outreach and informational programs author-
3 ized by this section—

4 “(A) shall make information about treat-
5 ment options and clinical trials for acquired
6 bone marrow failure diseases publicly available,
7 and

8 “(B) shall provide referral services for
9 treatment options and clinical trials,
10 at the national minority health resource center sup-
11 ported under section 1707(b)(8) (including by means
12 of the center’s website, through appropriate loca-
13 tions such as the center’s knowledge center, and
14 through appropriate programs such as the center’s
15 resource persons network) and through minority
16 health consultants located at each Department of
17 Health and Human Services regional office.

18 “(b) HISPANIC AND ASIAN-AMERICAN AND PACIFIC
19 ISLANDER OUTREACH.—

20 “(1) IN GENERAL.—The Secretary, acting
21 through the Deputy Assistant Secretary for Minority
22 Health, shall undertake a coordinated outreach ef-
23 fort to connect Hispanic, Asian-American, and Pa-
24 cific Islander communities with comprehensive serv-

1 ices focused on treatment of, and information about,
2 acquired bone marrow failure diseases.

3 “(2) COLLABORATION.—In carrying out this
4 subsection, the Secretary may collaborate with public
5 health agencies, nonprofit organizations, community
6 groups, and online entities to disseminate informa-
7 tion about treatment options and clinical trials for
8 acquired bone marrow failure diseases.

9 “(c) GRANTS AND COOPERATIVE AGREEMENTS.—

10 “(1) IN GENERAL.—Not later than 6 months
11 after the date of the enactment of this section, the
12 Secretary, acting through the Deputy Assistant Sec-
13 retary for Minority Health, shall award grants to, or
14 enter into cooperative agreements with, entities to
15 perform research on acquired bone marrow failure
16 diseases.

17 “(2) REQUIREMENT.—Grants and cooperative
18 agreements authorized by this subsection shall be
19 awarded or entered into on a competitive, peer-re-
20 viewed basis.

21 “(3) SCOPE OF RESEARCH.—Research funded
22 under this section shall examine factors affecting the
23 incidence of acquired bone marrow failure diseases
24 in minority populations.

1 “(d) DEFINITION.—In this section, the term ‘ac-
 2 quired bone marrow failure disease’ has the meaning given
 3 to such term in section 317U(d).

4 “(e) AUTHORIZATION OF APPROPRIATIONS.—There
 5 is authorized to be appropriated to carry out this section
 6 \$2,000,000 for each of fiscal years 2010 through 2014.”.

7 **SEC. 6. DIAGNOSIS AND QUALITY OF CARE FOR ACQUIRED**
 8 **BONE MARROW FAILURE DISEASES.**

9 (a) GRANTS.—The Secretary of Health and Human
 10 Services, acting through the Director of the Agency for
 11 Healthcare Research and Quality, shall award grants to
 12 entities to improve diagnostic practices and quality of care
 13 with respect to patients with acquired bone marrow failure
 14 diseases.

15 (b) AUTHORIZATION OF APPROPRIATIONS.—There is
 16 authorized to be appropriated to carry out this section
 17 \$2,000,000 for each of fiscal years 2010 through 2014.

18 **SEC. 7. DEFINITION.**

19 In this Act, the term “acquired bone marrow failure
 20 disease” means—

- 21 (1) myelodysplastic syndromes (MDS);
- 22 (2) aplastic anemia;
- 23 (3) paroxysmal nocturnal hemoglobinuria
- 24 (PNH);
- 25 (4) pure red cell aplasia;

- 1 (5) acute myeloid leukemia that progressed
- 2 from myelodysplastic syndromes; or
- 3 (6) large granular lymphocytic leukemia.

