

ACQUIRED BONE MARROW FAILURE DISEASE RESEARCH  
AND TREATMENT ACT OF 2010

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SEPTEMBER 28, 2010.—Committed to the Committee of the Whole House on the  
State of the Union and ordered to be printed

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Mr. WAXMAN, from the Committee on Energy and Commerce,  
submitted the following

R E P O R T

[To accompany H.R. 1230]

[Including cost estimate of the Congressional Budget Office]

The Committee on Energy and Commerce, to whom was referred the bill (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, having considered the same, report favorably thereon with amendments and recommend that the bill as amended do pass.

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## AMENDMENT

The amendment is as follows:

Strike all after the enacting clause and insert the following:

**SECTION 1. SHORT TITLE.**

This Act may be cited as the “Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010”.

**SEC. 2. ACQUIRED BONE MARROW FAILURE DISEASE RESEARCH.**

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. ACQUIRED BONE MARROW FAILURE DISEASE RESEARCH.**

“(a) **IN GENERAL.**—The Secretary may conduct research on acquired bone marrow failure diseases. Such research may address factors including—

“(1) trends in the characteristics of individuals who are diagnosed with acquired bone marrow failure diseases, including age, race and ethnicity, general geographic location, sex, family history, and any other characteristics determined appropriate by the Secretary;

“(2) the genetic and environmental factors, including exposure to toxins, that may be associated with developing acquired bone marrow failure diseases;

“(3) approaches to treating acquired bone marrow failure diseases;

“(4) outcomes for individuals treated for acquired bone marrow failure diseases, including outcomes for recipients of stem cell therapeutic products; and

“(5) any other factors pertaining to acquired bone marrow failure diseases determined appropriate by the Secretary.

“(b) **COLLABORATION WITH THE RADIATION INJURY TREATMENT NETWORK.**—In carrying out subsection (a), the Secretary may collaborate with the Radiation Injury Treatment Network of the C.W. Bill Young Cell Transplantation Program established pursuant to section 379 to—

“(1) augment data for the studies under such subsection;

“(2) access technical assistance that may be provided by the Radiation Injury Treatment Network; or

“(3) perform joint research projects.

“(c) **DEFINITION.**—In this section, the term ‘acquired bone marrow failure disease’ means—

“(1) myelodysplastic syndromes (MDS);

“(2) aplastic anemia;

“(3) paroxysmal nocturnal hemoglobinuria (PNH);

“(4) pure red cell aplasia;

“(5) acute myeloid leukemia that has progressed from myelodysplastic syndromes;

“(6) large granular lymphocytic leukemia; or

“(7) any other bone marrow failure disease specified by the Secretary, to the extent such disease is acquired and not inherited, as determined by the Secretary.”.

**SEC. 3. MINORITY-FOCUSED PROGRAMS ON ACQUIRED BONE MARROW FAILURE DISEASES.**

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

**“SEC. 1707B. MINORITY-FOCUSED PROGRAMS ON ACQUIRED BONE MARROW FAILURE DISEASES.**

“(a) **INFORMATION AND REFERRAL SERVICES.**—

“(1) **IN GENERAL.**—The Secretary may establish and coordinate outreach and informational programs targeted to minority populations, including Hispanic, Asian-American, Native Hawaiian, and Pacific Islander populations, that are affected by acquired bone marrow failure diseases.

“(2) **PROGRAM ACTIVITIES.**—Programs under subsection (a) may carry out activities that include—

“(A) making information about treatment options and clinical trials for acquired bone marrow failure diseases publicly available; and

“(B) providing referral services for treatment options and clinical trials.

“(b) **DEFINITION.**—In this section, the term ‘acquired bone marrow failure disease’ has the meaning given such term in section 317U(c).”.

**SEC. 4. BEST PRACTICES FOR DIAGNOSIS OF AND CARE FOR INDIVIDUALS WITH ACQUIRED BONE MARROW FAILURE DISEASES.**

Part B of title III of the Public Health Service Act (42 U.S.C. 243 et seq.), as amended by section 2, is further amended by inserting after section 317U the following:

**“SEC. 317V. BEST PRACTICES FOR DIAGNOSIS OF AND CARE FOR INDIVIDUALS WITH ACQUIRED BONE MARROW FAILURE DISEASES.**

“(a) GRANTS.—The Secretary, acting through the Director of the Agency for Healthcare Research and Quality, may award grants to researchers to study best practices with respect to diagnosing acquired bone marrow failure diseases and providing care to individuals with such diseases.

“(b) DEFINITION.—In this section, the term ‘acquired bone marrow failure disease’ has the meaning given such term in section 317U(c).”.

Amend the title so as to read:

A bill to amend the Public Health Service Act to provide for research on acquired bone marrow failure diseases, minority-focused programs on such diseases, and the development of best practices for diagnosis of and care for individuals with such diseases.

PURPOSE AND SUMMARY

H.R. 1230, the “Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010”, was introduced on February 26, 2009, by Rep. Doris O. Matsui (D-CA) and referred to the Committee on Energy and Commerce.

The goal of H.R. 1230 is to support research on acquired bone marrow failure diseases, establish and coordinate outreach and informational programs on such diseases targeted to minority populations, and promote best practices for the diagnosis of and care for, individuals with these diseases.

BACKGROUND AND NEED FOR LEGISLATION

Bone marrow produces the blood cells that circulate in the human body. A bone marrow failure disease occurs when bone marrow stops functioning or begins to produce abnormal blood cells; it can be either inherited or acquired. These diseases, while rare, affect thousands of adults and children every year. They include aplastic anemia, myelodysplastic syndromes, paroxysmal nocturnal hemoglobinuria, pure red cell aplasias, and sideroblastic anemia.

While federal research is ongoing on both inherited and acquired bone marrow failure diseases, further efforts are needed to study these conditions.

COMMITTEE CONSIDERATION

H.R. 1230, the “Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010”, was introduced by Ms. Matsui of California on February 26, 2009, and referred to the Committee on Energy and Commerce. The bill was subsequently referred to the Subcommittee on Health on March 6, 2010. On September 15, 2010, the Subcommittee held a legislative hearing on the bill. The Subcommittee met in open markup session to consider H.R. 1230 on September 16, 2010. An amendment in the nature of a substitute (manager’s amendment) by Mr. Pallone of New Jersey was adopted by a voice vote. Subsequently, H.R. 1230 was favorably forwarded to the full Committee, amended, by a voice vote.

On September 23, 2010, the Committee on Energy and Commerce met in open markup session and considered H.R. 1230 as ap-

proved by the Subcommittee. There were no amendments offered in full Committee and subsequently the Committee ordered H.R. 1230 favorably reported to the House, as amended by the Subcommittee on Health, by a voice vote.

#### COMMITTEE VOTES

Clause 3(b) of rule XIII of the Rules of the House of Representatives requires the Committee to list each record vote on the motion to report legislation and amendments thereto. A motion by Mr. Pallone ordering H.R. 1230 reported to the House, as amended, was approved by a voice vote. There were no record votes taken during consideration of this bill.

#### COMMITTEE OVERSIGHT FINDINGS AND RECOMMENDATIONS

In compliance with clause 3(c)(1) of rule XIII and clause (2)(b)(1) of rule X of the Rules of the House of Representatives, the oversight findings and recommendations of the Committee are reflected in the descriptive portions of this report, including the finding that further efforts are needed to study acquired bone marrow failure diseases.

#### NEW BUDGET AUTHORITY, ENTITLEMENT AUTHORITY, AND TAX EXPENDITURES

Regarding compliance with clause 3(c)(2) of rule XIII of the Rules of the House of Representatives, the Committee finds that H.R. 1230 would result in no new budget authority, entitlement authority, or tax expenditures or revenues.

#### STATEMENT OF GENERAL PERFORMANCE GOALS AND OBJECTIVES

In accordance with clause 3(c)(4) of rule XIII of the Rules of the House of Representatives, the performance goals and objectives of the Committee are reflected in the descriptive portions of this report, including the goal of supporting efforts to research acquired bone marrow failure diseases.

#### CONSTITUTIONAL AUTHORITY STATEMENT

Pursuant to clause 3(d)(1) of rule XIII of the Rules of the House of Representatives, the Committee finds that the constitutional authority for H.R. 1230 is provided under article I, section 8, clauses 3 and 18 of the Constitution of the United States.

#### EARMARKS AND TAX AND TARIFF BENEFITS

H.R. 1230 does not contain any congressional earmarks, limited tax benefits, or limited tariff benefits as defined in clause 9 of rule XXI of the Rules of the House of Representatives.

#### FEDERAL ADVISORY COMMITTEE STATEMENT

The Committee finds that the legislation does not establish or authorize the establishment of an advisory committee within the definition of 5 U.S.C. App., section 5(b) of the Federal Advisory Committee Act.

APPLICABILITY OF LAW TO THE LEGISLATIVE BRANCH

Section 102(b)(3) of Public Law 104–1 requires a description of the application of this bill to the legislative branch where the bill relates to terms and conditions of employment or access to public services and accommodations. H.R. 1230 contains no such provisions.

FEDERAL MANDATES STATEMENT

Section 423 of the Congressional Budget and Impoundment Control Act of 1974 (as amended by section 101(a)(2) of the Unfunded Mandates Reform Act, Public Law 104–4) requires a statement on whether the provisions of the report include unfunded mandates. In compliance with this requirement the Committee adopts as its own the analysis of federal mandates prepared by the Director of the Congressional Budget Office regarding H.R. 1230.

COMMITTEE COST ESTIMATE

Pursuant to clause 3(d)(2) of rule XIII of the Rules of the House of Representatives, the Committee adopts as its own the cost estimate of H.R. 1230 prepared by the Director of the Congressional Budget Office pursuant to section 402 of the Congressional Budget Act of 1974.

CONGRESSIONAL BUDGET OFFICE COST ESTIMATE

With respect to the requirements of clause (3)(c)(3) of rule XIII of the Rules of the House of Representatives and section 402 of the Congressional Budget Act of 1974, the Committee has received the following cost estimate for H.R. 1230 from the Director of Congressional Budget Office:

U.S. CONGRESS,  
CONGRESSIONAL BUDGET OFFICE,  
*Washington, DC, September 27, 2010.*

Hon. HENRY A. WAXMAN,  
*Chairman, Committee on Energy and Commerce,*  
*U.S. House of Representatives, Washington, DC.*

DEAR MR. CHAIRMAN: The Congressional Budget Office has prepared the enclosed cost estimate for H.R. 1230, the Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010.

If you wish further details on this estimate, we will be pleased to provide them. The CBO staff contact is Lisa Ramirez-Branum.

Sincerely,

DOUGLAS W. ELMENDORF.

Enclosure.

*H.R. 1230—Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010*

Summary: H.R. 1230 would amend the Public Health Service Act to authorize the Secretary of the Department of Health and Human Services (HHS) to conduct research and outreach activities related to diseases involving the failure of acquired bone marrow. Additionally, the bill would authorize the Director of the Agency for Healthcare Research and Quality (AHRQ) to award grants to re-

searchers to study best practices with respect to diagnosing and providing care to individuals with such diseases.

Assuming the appropriation of necessary amounts, CBO estimates that implementing the bill would cost \$2 million in 2011 and \$26 million over the 2011–2015 period. Enacting H.R. 1230 would not affect direct spending or revenues; therefore, pay-as-you-go procedures do not apply.

H.R. 1230 contains no intergovernmental or private-sector mandates as defined in the Unfunded Mandates Reform Act (UMRA).

Estimated cost to the Federal Government: The estimated budgetary impact of H.R. 1230 is shown in the following table. The costs of this legislation fall within budget function 550 (health).

	By fiscal year, in millions of dollars—					
	2011	2012	2013	2014	2015	2011–2015
CHANGES IN SPENDING SUBJECT TO APPROPRIATION						
Estimated Authorization Level .....	6	6	6	7	7	32
Estimated Outlays .....	2	5	6	6	6	26

Note: Components may not sum to totals because of rounding.

**Basis of estimate:** For this estimate, CBO assumes that the legislation will be enacted near the beginning of fiscal year 2011 and that the necessary amounts will be appropriated for each year. Estimated outlays are based on historical spending patterns for similar federal programs and on information provided by HHS, AHRQ, and the Office of Minority Health (OMH).

H.R. 1230 would authorize the Secretary of HHS to conduct research on diseases involving the failure of acquired bone marrow in collaboration with the department's Radiation Injury Treatment Network. CBO does not anticipate that any costs would be incurred under this provision beyond what the National Institutes of Health will spend under current law to conduct similar research over the next several years.

H.R. 1230 also would authorize the Secretary to establish and coordinate outreach and informational programs targeted to minority populations that are affected by diseases involving the failure of acquired bone marrow. Based on information from OMH, CBO estimates that this provision would require appropriations totalling \$16 million over the 2011–2015 period. Assuming appropriation of those amounts, CBO estimates that implementing those programs would cost \$13 million over the 2011–2015 period.

The bill also would authorize AHRQ to award grants to researchers to study best practices with respect to diagnosing and providing care to individuals with diseases involving the failure of acquired bone marrow. Based on spending by AHRQ for similar activities, CBO estimates that implementing this provision would require appropriations totalling \$17 million over the 2011–2015 period. Assuming appropriation of those amounts, CBO estimates that implementing that grant program would cost \$13 million over the 2011–2015 period.

**Intergovernmental and private-sector impact:** H.R. 1230 contains no intergovernmental or private-sector mandates as defined in UMRA and would impose no costs on the budgets of state, local, or tribal governments.

Estimate prepared by: Federal Costs: Jamease Kowalczyk and Lisa Ramirez-Branum; Impact on State, Local, and Tribal Governments: Lisa Ramirez-Branum; Impact on the Private Sector: Sarah Axeen.

Estimate approved by: Holly Harvey, Deputy Assistant Director for Budget Analysis.

#### SECTION-BY-SECTION ANALYSIS OF THE LEGISLATION

##### *Section 1. Short title*

Section 1 designates that the short title may be cited as the “Acquired Bone Marrow Failure Disease Research and Treatment Act of 2010”.

##### *Section 2. Acquired bone marrow failure disease research*

Section 2 authorizes the Secretary of Health and Human Services (HHS) to support research on acquired bone marrow failure diseases and specifies factors that such research may address. In supporting this research, the Secretary may collaborate with the Radiation Injury Treatment Network of the C.W. Bill Young Cell Transplantation Program (established under Section 379 of the Public Health Service Act).

##### *Section 3. Minority-focused programs on acquired bone marrow failure diseases*

Section 3 provides authority for the Secretary to establish outreach and information programs targeted to minority populations affected by acquired bone marrow failure disease and provide referral services for treatment options and clinical services.

##### *Section 4. Best practices for diagnosis of and care for individuals with acquired bone marrow failure diseases*

Section 4 provides authority for the Secretary, acting through the Director of the Agency for Healthcare Research and Quality (AHRQ), to award grants to researchers to study best practices with regard to the diagnosis and treatment of individuals with acquired bone marrow diseases.

#### EXPLANATION OF AMENDMENT

During the Subcommittee on Health markup of H.R. 1230, Mr. Pallone of New Jersey offered an amendment in the nature of a substitute (manager’s amendment), which was adopted by a voice vote. The substance of the substitute amendment is reflected in the section-by-section analysis contained in this report.

#### CHANGES IN EXISTING LAW MADE BY THE BILL, AS REPORTED

In compliance with clause 3(e) of rule XIII of the Rules of the House of Representatives, changes in existing law made by the bill, as reported, are shown as follows (new matter is printed in italic and existing law in which no change is proposed is shown in roman):

#### **PUBLIC HEALTH SERVICE ACT**

\* \* \* \* \*

TITLE III—GENERAL POWERS AND DUTIES OF PUBLIC  
HEALTH SERVICE

\* \* \* \* \*

PART B—FEDERAL-STATE COOPERATION

\* \* \* \* \*

**SEC. 317U. ACQUIRED BONE MARROW FAILURE DISEASE RESEARCH.**

(a) *IN GENERAL.*—The Secretary may conduct research on acquired bone marrow failure diseases. Such research may address factors including—

(1) trends in the characteristics of individuals who are diagnosed with acquired bone marrow failure diseases, including age, race and ethnicity, general geographic location, sex, family history, and any other characteristics determined appropriate by the Secretary;

(2) the genetic and environmental factors, including exposure to toxins, that may be associated with developing acquired bone marrow failure diseases;

(3) approaches to treating acquired bone marrow failure diseases;

(4) outcomes for individuals treated for acquired bone marrow failure diseases, including outcomes for recipients of stem cell therapeutic products; and

(5) any other factors pertaining to acquired bone marrow failure diseases determined appropriate by the Secretary.

(b) *COLLABORATION WITH THE RADIATION INJURY TREATMENT NETWORK.*—In carrying out subsection (a), the Secretary may collaborate with the Radiation Injury Treatment Network of the C.W. Bill Young Cell Transplantation Program established pursuant to section 379 to—

(1) augment data for the studies under such subsection;

(2) access technical assistance that may be provided by the Radiation Injury Treatment Network; or

(3) perform joint research projects.

(c) *DEFINITION.*—In this section, the term “acquired bone marrow failure disease” means—

(1) myelodysplastic syndromes (MDS);

(2) aplastic anemia;

(3) paroxysmal nocturnal hemoglobinuria (PNH);

(4) pure red cell aplasia;

(5) acute myeloid leukemia that has progressed from myelodysplastic syndromes;

(6) large granular lymphocytic leukemia; or

(7) any other bone marrow failure disease specified by the Secretary, to the extent such disease is acquired and not inherited, as determined by the Secretary.

**SEC. 317V. BEST PRACTICES FOR DIAGNOSIS OF AND CARE FOR INDIVIDUALS WITH ACQUIRED BONE MARROW FAILURE DISEASES.**

(a) *GRANTS.*—The Secretary, acting through the Director of the Agency for Healthcare Research and Quality, may award grants to researchers to study best practices with respect to diagnosing acquired bone marrow failure diseases and providing care to individuals with such diseases.



*(b) DEFINITION.—In this section, the term “acquired bone marrow failure disease” has the meaning given such term in section 317U(c).*

\* \* \* \* \*

TITLE XVII—HEALTH INFORMATION AND HEALTH PROMOTION

\* \* \* \* \*

**SEC. 1707B. MINORITY-FOCUSED PROGRAMS ON ACQUIRED BONE MARROW FAILURE DISEASES.**

*(a) INFORMATION AND REFERRAL SERVICES.—*

*(1) IN GENERAL.—The Secretary may establish and coordinate outreach and informational programs targeted to minority populations, including Hispanic, Asian-American, Native Hawaiian, and Pacific Islander populations, that are affected by acquired bone marrow failure diseases.*

*(2) PROGRAM ACTIVITIES.—Programs under subsection (a) may carry out activities that include—*

*(A) making information about treatment options and clinical trials for acquired bone marrow failure diseases publicly available; and*

*(B) providing referral services for treatment options and clinical trials.*

*(b) DEFINITION.—In this section, the term “acquired bone marrow failure disease” has the meaning given such term in section 317U(c).*

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