

108TH CONGRESS
1ST SESSION

H. R. 2456

To require increased activities by the National Institutes of Health and the Centers for Disease Control and Prevention regarding Diamond-Blackfan anemia, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

JUNE 12, 2003

Mrs. MCCARTHY of New York (for herself and Mr. QUINN) introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To require increased activities by the National Institutes of Health and the Centers for Disease Control and Prevention regarding Diamond-Blackfan anemia, 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 “Diamond-Blackfan
5 Anemia Research and Care Act”.

6 **SEC. 2. FINDINGS.**

7 The Congress finds as follows:

1 (1) Diamond-Blackfan anemia is a rare genetic
2 bone marrow failure disorder, which develops in in-
3 fancy and results in severe anemia due to failure to
4 make red blood cells.

5 (2) Diamond-Blackfan anemia patients have an
6 increased risk of leukemia, solid tumors, and com-
7 plete bone marrow failure.

8 (3) Fifty percent of patients with Diamond-
9 Blackfan anemia are born with birth defects, includ-
10 ing abnormalities to the face, head, upper arm and
11 hand, genitourinary, and heart, and 21 percent of
12 the affected patients have more than 1 defect.

13 (4) Treatments for Diamond-Blackfan anemia,
14 including the use of blood transfusions and steroids
15 such as prednisone, have potential long-term side ef-
16 fects, including osteoporosis, iron overload (because
17 of the transfusions), and impaired growth (because
18 of the steroids).

19 (5) The only cure for Diamond-Blackfan ane-
20 mia is a bone marrow transplant, a procedure that
21 carries serious risks and, since most patients lack an
22 acceptable donor, is an option for only about 25 per-
23 cent of patients.

24 (6) Because Diamond-Blackfan anemia is a ge-
25 netic disorder of red cell production and a cancer

1 predisposition syndrome with a high rate of con-
 2 genital anomalies, the Federal investment regarding
 3 Diamond-Blackfan anemia must be expanded to
 4 allow the careful dissection of this disease, which will
 5 provide valuable insights into the biology of blood
 6 disorders and cancer predisposition and serve as an
 7 important model for understanding the genetics of
 8 birth defects.

9 **SEC. 3. DIAMOND-BLACKFAN ANEMIA.**

10 (a) DBA-RELATED ACTIVITIES OF NIH.—Part A of
 11 title IV of the Public Health Service Act is amended by
 12 inserting after section 404G (42 U.S.C. 283i) the fol-
 13 lowing:

14 “DIAMOND-BLACKFAN ANEMIA

15 “SEC. 404H. (a) IN GENERAL.—The Director of
 16 NIH, in coordination with the Directors of the National
 17 Heart, Lung, and Blood Institute, the National Institute
 18 of Diabetes and Digestive and Kidney Diseases, and the
 19 Office of Rare Diseases, shall expand and intensify re-
 20 search and related activities of the National Institutes of
 21 Health with regard to Diamond-Blackfan anemia.

22 “(b) COMPREHENSIVE RESEARCH INITIATIVE.—

23 “(1) IN GENERAL.—In carrying out this sec-
 24 tion, the Director of NIH shall make grants to, or
 25 enter into contracts with, public or private entities
 26 to support a comprehensive research initiative to

study, develop better treatments for, and ultimately find a cure for Diamond-Blackfan anemia.

“(2) RESEARCH.—The initiative supported under this subsection may include research on the following:

“(A) The links of Diamond-Blackfan anemia to chronic diseases.

“(B) Red cell differentiation.

“(C) The pathophysiology of Diamond-Blackfan anemia.

“(D) The relationship between Diamond-Blackfan anemia and predisposition to cancer.

“(E) Congenital anomalies in Diamond-Blackfan anemia patients.”.

(b) DBA-RELATED ACTIVITIES OF CDC.—Part B of title III of the Public Health Service Act (42 U.S.C. 243 et seq.) is amended—

(1) by moving section 317R so that it follows section 317Q; and

(2) by inserting after section 317R the following:

“SEC. 317S. REGISTRY AND CLINICAL CARE CENTER FOR DIAMOND-BLACKFAN ANEMIA.

“(a) REGISTRY.—The Secretary, acting through the Director of the Centers for Disease Control and Preven-

1 tion, shall maintain and expand the Diamond-Blackfan
2 Anemia Registry (in this section referred to as the ‘Reg-
3 istry’).

4 “(b) COMPREHENSIVE CLINICAL CARE CENTER.—

5 “(1) ESTABLISHMENT.—The Secretary, acting
6 through the Director of the Centers for Disease
7 Control and Prevention, shall establish a comprehen-
8 sive clinical care center for Diamond-Blackfan ane-
9 mia where a majority of the patients with the dis-
10 ease are examined, treated, and tracked through the
11 Registry, by experts in the disease.

12 “(2) DUTIES.—The center established under
13 this section shall—

14 “(A) gather and analyze extensive data on
15 Diamond-Blackfan anemia to be used for pub-
16 lic, non-profit, and government research initia-
17 tives involving gene discovery, ribosomal protein
18 function, genetics of birth defects, blood cell
19 formation (recovery from cancer chemotherapy),
20 cancer predisposition, red cell differentiation,
21 and a comparison of therapeutic treatments in-
22 cluding blood transfusion, steroids, and bone
23 marrow transplants;

24 “(B) provide thorough examinations of Di-
25 amond-Blackfan anemia patients by experts in

1 the disease to confirm diagnosis and provide ge-
2 netic typing along with a multi-system evalua-
3 tion; and

4 “(C) provide clinical care for Diamond-
5 Blackfan anemia.”.

○