H. Res. 524

In the House of Representatives, U. S.,

October 15, 2007.

Whereas Diamond-Blackfan Anemia (''DBA'') is a rare genetic bone marrow failure disorder affecting children and adults, 90 percent of whom are younger than 1 year of age when they are diagnosed, and results in severe anemia due to failure to produce red blood cells;

Whereas individuals and families suffering with rare diseases such as DBA not only face the challenges of their debilitating and life-threatening diseases, but must also confront the consequences of their rare disease status;

Whereas individuals suffering from rare diseases need access to treatment options and the potential for a cure;

Whereas research is proving the study of complex, rare diseases such as DBA yield tremendous advancements in other, larger disease areas that affect millions of Americans;

Whereas the children living with DBA have an increased risk of leukemia, solid tumors, and complete bone marrow failure, and 50 percent of patients with DBA are born with birth defects including abnormalities to the face, head, upper arm and hand, genitourinary, and heart with 21 percent of affected patients having more than 1 defect;

Whereas the study of DBA will yield the true incidence of aplastic anemia, myelodysplastic syndrome, leukemia, and the predisposition to cancer in DBA and will serve as an important model for understanding the genetics of birth defects;

Whereas treatments for DBA, including the use of steroids (such as prednisone) and blood transfusions, have potential long-term side effects, including osteoporosis, impaired growth because of the steroids, diabetes, and iron overload because of the transfusions;

Whereas the only cure for DBA is a bone marrow transplant, a procedure that carries serious risks and, since most patients lack an acceptable donor, is an option available for only about 25 percent of patients;

Whereas rare diseases, such as DBA, benefit greatly from well-established comprehensive care centers such as the DBA Comprehensive Clinical Care Center at Schneider Children's Hospital in New Hyde Park, New York (the ''Center''), which has become the multidimensional hub for the care and treatment of DBA patients across the country, as well as the home of the DBA Patient Registry which has become a valuable national resource for investigators utilizing the Center to accomplish research in a multitude of areas not specific only to DBA;

Whereas the successful establishment of the Center became a model for how to diagnose, treat, and improve the lives of patients with rare diseases, while learning from the disorder to yield advancements in other areas of disease research;

Whereas the success of the initial Center prompted the Centers for Disease Control and Prevention's DBA Public

Health Outreach and Surveillance Program to establish 3 additional DBA Centers in Texas, California, and Massachusetts to further patient access to information, treatment, and care by DBA experts, which has resulted in a doubling of patient care visits for DBA care and surveillance since their establishment;

Whereas the DBA Public Health Outreach and Surveillance Program at the Centers for Disease Control and Prevention (''CDC'') has resulted in the completion of the first CDC brochure for the DBA patient population, the introduction of a DBA hotline and dedicated DBA nurse, and has resulted in a 25-percent increase of enrollment of DBA patients into the DBA Patient Registry in the first 2 years of the program;

Whereas the collaboration between the National Institutes of Health and the Centers for Disease Control and Prevention and their close collaboration with the Daniella Maria Arturi Foundation and the DBA Foundation have driven the many recent successes in the DBA field and serve as a model for addressing rare disease research efforts through close public and private collaboration to achieve the highest levels of success in the areas of improved patient care and disease research;

Whereas the interagency collaboration achieved within the National Institutes of Health between the National Heart, Lung, and Blood Institute, the National Institute of Diabetes and Digestive and Kidney Diseases, the National Cancer Institute, and the Office of Rare Diseases to advance the research and understanding of DBA has resulted in significant advancements not only in the DBA scientific arena, but in understanding its many links to more prevalent disorders; and

Whereas the DBA research initiatives have already yielded tremendous success including the discovery of 2 ribosomal protein (''RP'') genes and the identification that DBA is the first human disease linked to a ribosomal protein problem which, as a fundamental unit of cellular function, has been implicated in a wide range of human disorders including cancer, making this discovery a profound example of the additional benefits that may result from the study of DBA: Now, therefore, be it

Resolved, That the House of Representatives—

(1) recognizes that the identification of Diamond-Blackfan Anemia (''DBA'') may advance the understanding of DBA, identify implications of cancer predisposition, and serve as an important model for understanding human development and the molecular basis for certain birth defects;

(2) recognizes the importance of comprehensive care centers in providing complete care and treatment for each patient, leading to an increase in correct and early diagnosis;

(3) commends Schneider Children's Hospital for providing the first DBA Comprehensive Clinical Care Center for patients across the country, for developing the DBA Patient Registry which has proven a robust surveillance tool to understand the epidemiology, biology, and treatment of DBA, and for proving a valuable resource for investigators at a national level, working to

understand DBA's link to more prevalent disorders facing Americans;

(4) commends the Daniella Maria Arturi Foundation and the Diamond-Blackfan Anemia Foundation for their efforts to facilitate the successful collaboration among the National Institutes of Health and the Centers for Disease Control and Prevention to achieve a successful multidisciplinary approach between clinical and scientific DBA efforts with the goal of shortening the life cycle of success realized between the laboratory and applied patient care; and

(5) encourages research efforts to further understand ribosomal protein deficiencies in rare inherited diseases and to advance the treatment options available to those with DBA.

Attest:

Clerk.