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American Society of Hematology
Statement to the
Senate Appropriations Subcommittee on Labor, HHS, Education, and Related Agencies
in Support of
Fiscal Year 2014 Funding for the National Institutes of Health (NIH) and
the Centers for Disease Control and Prevention (CDC)
May 6, 2013

The American Society of Hematology (ASH) thanks the Subcommittee for the opportunity to submit written testimony on the fiscal year (FY) 2014 Departments of Labor, Health and Human Services, and Education Appropriations bill.

ASH represents approximately 14,000 clinicians and scientists committed to the study and treatment of blood and blood-related diseases. These diseases encompass malignant disorders such as leukemia, lymphoma, and myeloma; life-threatening conditions, including thrombosis and bleeding disorders; and congenital diseases such as sickle cell anemia, thalassemia, and hemophilia. In addition, hematologists have been pioneers in the fields of bone marrow transplantation, stem cell biology and regenerative medicine, gene therapy, and the development of many drugs for the prevention and treatment of heart attacks and strokes.

Funding for Hematology Research: An Investment in the Nation's Health

Over the past 60 years, American biomedical research has led the world in probing the nature of human disease. This research has led to new medical treatments, saved innumerable lives, reduced human suffering, and spawned entire new industries. This research would not have been possible without support from the National Institutes of Health (NIH). NIH-funded research drives medical innovation that improves health and quality of life through new and better diagnostics, improved prevention strategies, and more effective treatments. Federal funding of basic biomedical research through the NIH is crucial, as most of this discovery-based research is not supported by philanthropy or private industry. Discoveries gained through basic research yield the medical advances that improve the fiscal and physical health of the country.

Funding for hematology research has been an important component of this investment in the nation's health. Most of the research that produced cures and treatments for hematologic diseases has been funded by the NIH. The study of blood and its disorders is a trans-NIH issue involving many institutes at the NIH, including the National Heart, Lung and Blood Institute (NHLBI), the National Cancer Institute (NCI), the National Institute of Diabetes, Digestive and Kidney Diseases (NIDDK), and the National Institute on Aging (NIA).

With the advances gained through an increasingly sophisticated understanding of how the blood system functions, hematologists have changed the face of medicine through their dedication to improving the lives of patients. As a result, children are routinely cured of acute lymphoblastic leukemia (ALL); more than 90 percent of patients with acute promyelocytic leukemia (APL) are cured with a drug derived from vitamin A; older patients suffering from previously lethal chronic myeloid leukemia (CML) are now effectively treated with well-tolerated pills; and patients with multiple myeloma are treated with new classes of drugs.

Hematology advances also help patients with other types of cancers, heart disease, and stroke. Blood thinners effectively treat or prevent blood clots, pulmonary embolism, and strokes. Death rates from heart attacks are reduced by new forms of anticoagulation drugs. Stem cell transplantation can cure not only blood diseases but also inherited metabolic disorders, while gene therapy holds the promise of effectively treating even more genetic diseases. Even modest investments in hematology research have yielded large dividends for other disciplines.

The Future Promise of Hematology Research

The era of precision medicine has arrived. Insights into new genetic and biologic markers can be used to understand what causes a disease, the risk factors that predispose to disease, and how patients will respond to a particular treatment. Translating these new discoveries and technologies into personalized patient care offers the possibility of better survival, less toxicity, disease prevention, improved quality of life, and lower health-care costs. However, many patients still lack effective therapy for malignant and non-malignant hematologic diseases.

Research funding must increase to allow the major advances in understanding the molecular defects behind hematologic diseases to be translated into novel diagnostics and targeted therapeutics. Support for research in the areas listed below will be important for future progress:

- Stem Cells and Regenerative Medicine: Turn iPS cells into cures for human diseases
- Myelodysplastic Syndrome and Acute Myeloid Leukemia: Find an effective and personalized treatment for the elderly
- Hematopoietic Stem Cell Transplantation: Increase success rates by improving management of graft-versus-host disease
- Sickle Cell Disease: Reduce the barriers to care, burden of pain, end-organ injury, and premature death
- Deep-Vein Thrombosis and Venous Thromboembolism: Understand the risk factors and develop targeted therapies
- Childhood Leukemia: Improve cure rates by performing coordinated research that includes discovery and preclinical and clinical testing of novel targeted therapies
- Translating Laboratory Advances into the Clinic: Use novel genomic technologies to improve treatment of hematologic diseases

Sequestration Threatens Scientific Momentum

ASH is particularly concerned about the impact of continued cuts on biomedical research supported by the NIH. At a time when we should be investing more in research to save more lives, research funding is in serious jeopardy.

After a decade of flat funding, the NIH budget after inflation is about 20 percent lower than it was in 2003. ASH is deeply disturbed about the impact that this effective "un-doubling" of the NIH budget, combined with the more than 5 percent cut in NIH funding under sequestration in the current fiscal year and additional planned cuts in future fiscal years, will have on the ability

to sustain the scientific momentum that has contributed so greatly to our nation's health and our economic vitality. NIH's ability to continue current research capacity and encourage promising new areas of science is, and will be, significantly limited. Sequestration will result in cuts in extramural grants and slowing momentum for the development of new treatments, or even cures, for seriously ill patients with deadly diseases.

Additionally, perhaps one of the greatest concerns is the obstacle these continued cuts will present to the next generation of scientists, who will see training funds slashed and the possibility of sustaining a career in research diminished. NIH also plays a significant role in supporting the next generation of innovators, the young and talented scientists and physicians who will be responsible for the breakthroughs of tomorrow. The Society is especially concerned about the number of scientists who have abandoned research careers; continued cuts will exacerbate this exodus, forcing researchers to abandon potentially life-enhancing research, negatively affecting job creation, and seriously jeopardizing America's leadership in medical research throughout the world.

FY 2014 NIH Funding Request

ASH supports the recommendation of the Ad Hoc Group for Medical Research that the Subcommittee recognize NIH as a critical national priority by providing at least $32 billion in funding in the FY 2014 Labor-HHS-Education Appropriations bill. This funding recommendation represents the minimum investment necessary to avoid further loss of promising research and at the same time allows the NIH's budget to keep pace with biomedical inflation.

Hematology research offers enormous potential to better understand, prevent, treat, and cure a number of blood-related and other conditions. Recent investments have created dramatic new research opportunities, spurring advancements and precipitating the promise of personalized medicine that will yield far-reaching health and economic benefits. Trials to find new therapies and cures for millions of Americans with blood cancers, bleeding disorders, clotting problems, and genetic diseases are just a few of the important projects that could be delayed unless NIH continues to receive predictable and sustained funding.

It is critically important that our country continues to capitalize on the momentum of previous investments to drive research progress to develop new treatments for serious disorders, train the next generation of scientists, create jobs, and promote economic growth and innovation. Adequate funding is necessary for NIH to sustain current research capacity and encourage promising new areas of science and cures.

While ASH recognizes the deficit and the increasing debt the country faces will require difficult decisions, it is also important to understand that federal investment in research and public health programs saves lives, reduces health costs and strengthens the nation. Funding for hematology research is an investment in the nation's health. Research funding must increase to allow the major advances in understanding the molecular defects behind hematologic diseases to be translated into novel diagnostics and targeted therapeutics not only for blood disorders, but other life-threatening diseases. ASH urges the Subcommittee to continue to be a champion for

research and support at least $32 billion in funding for NIH in FY 2014. The American people are depending on you to ensure the nation does not lose the health and economic benefits of our extraordinary commitment to biomedical research.

Centers for Disease Control and Prevention (CDC) Public Health Response for Blood Disorders

The Society also recognizes the important role of the Centers for Disease Control and Prevention (CDC) in preventing and controlling clotting, bleeding, and other hematologic disorders. Blood disorders – such as sickle cell disease, anemia, blood clots, and hemophilia – are a serious public health problem and affect millions of people each year in the United States, cutting across the boundaries of age, race, sex, and socioeconomic status. Men, women, and children of all backgrounds live with the complications associated with these conditions, many of which are painful and potentially life-threatening.

Through the Division of Blood Disorders in the Center on Birth Defects and Developmental Disabilities (NCBDDD), CDC is working toward developing a comprehensive public health agenda to promote and improve the health of people with blood disorders. As a key component of this public health approach, CDC staff invest in identifying, monitoring, diagnosing, and investigating blood disorders to understand the prevalence and effect of these disorders. Charting the characteristics and outcomes of a disease population, such as those with sickle cell disease or hemophilia, can provide insight into these questions, as well as help identify the quality and cost of care issues that people who are affected face. Additionally, population-based studies can increase our understanding of risk factors that can result in severe complications for people with blood disorders.

CDC is uniquely positioned to reduce the public health burden resulting from blood disorders by contributing to a better understanding of these conditions and their complications; ensuring that prevention programs are developed, implemented, and evaluated; ensuring that information is accessible to consumers and health care providers; and encouraging action to improve the quality of life for people living with or affected by these conditions. The Society is supportive of maintaining the programs funded by the Division of Blood Disorders and supports the requested budget authority of $20,672,000 for the Public Health Approach to Blood Disorders in the President's FY 2014 budget request. This funding will allow CDC to improve health outcomes and limit complications to those who are risk or currently have blood disorders, by promoting a comprehensive care model; identifying and evaluating effective prevention strategies; and increasing public and healthcare provider awareness of bleeding and clotting disorders such as such as hemophilia and thrombosis, and hemoglobinopathies, including sickle cell disease and thalassemia.

Thank you again for the opportunity to submit testimony. Please contact Tracy Roades, ASH Legislative Advocacy Manager, at 202-776-0544 or *troades@hematology.org*, if you have any questions or need further information concerning hematology research or ASH's FY 2014 funding request.