American Society of Hematology Statement to the
Senate Appropriations Subcommittee on Labor, HHS, Education, and Related Agencies
in Support of
Fiscal Year 2016 Funding for the National Institutes of Health (NIH) and
the Centers for Disease Control and Prevention (CDC)

April 3, 2015

The American Society of Hematology (ASH) thanks the Subcommittee for the opportunity to submit written testimony on the fiscal year (FY) 2016 Departments of Labor, Health and Human Services, and Education Appropriations bill.

ASH represents more than 15,000 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- and immunotherapy, 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).

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. Even modest investments in hematology research have yielded large dividends for other disciplines. Basic research on blood has aided physicians who treat patients with heart disease, strokes, end-stage renal disease, cancer, and AIDS. 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.
Future Promise

The era of precision medicine has arrived. The field of hematology has experienced a recent surge in progress thanks to novel technologies, mechanistic insights, and cutting-edge therapeutic strategies that have driven significant and meaningful advances in the quality of care. 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. These foundational insights are reframing modern research with the continued goal of improving outcomes and discovering cures for the most challenging hematologic diseases.

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. Yet today, a number of specific and critically important research questions must be answered to gain the insights that will launch the field into the next generation of care for hematologic conditions. A wide variety of blood-related diseases – from malignancies such as lymphoma and leukemia, to non-malignant diseases including hemoglobinopathies – continue to be associated with significant morbidity and mortality and demand attention to reduce their burden and improve the quality of care worldwide.

Sequestration Threatens Scientific Momentum

ASH is particularly concerned about the impact of continued cuts on biomedical research supported by the NIH. NIH's ability to continue current research capacity and encourage promising new areas of science is, and will be, significantly limited. At a time when we should be investing more in research to save lives, research funding remains in serious jeopardy. 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.

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. 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.

FY 2016 Requests

NIH Funding

ASH appreciates the welcome and much needed funding increase for the NIH that Congress provided in the FY 2015 Consolidated Appropriations Act. However, this increase did not give back all of the funds cut by sequestration in FY 2013 nor did it restore the purchasing power lost over the past decade. ASH supports the Ad Hoc Group for Medical Research recommendation that NIH receive at least $32 billion in FY 2016 as the next step toward a multi-year increase in our nation's investment in medical research. ASH also urges Congress and the Administration to work in a bipartisan manner to end sequestration and the continued cuts to medical research that squander invaluable scientific opportunities, discourage young scientists, threaten medical progress and continued improvements in our nation's health, and jeopardize our economic future.
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.

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 concerned that the Division of Blood Disorders was cut by nearly $6 million in the Consolidated Appropriations Act of 2014 and only $500,000 was added back to the Division in FY 2015. ASH respectfully requests that the Committee continue to restore funding for the Division of Blood Disorders to the FY 2013 levels to ensure that the programs funded by the Division for hemophilia, thalassemia, sickle cell disease, and DVT/PE can be maintained. 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.

Additional Activities

In FY 2016, ASH also urges the Subcommittee to recognize the following activities impacting hematology:

- **Importance of Genome Editing and Gene Therapy for the Correction of Inherited Blood Disorders**

  Genome editing is currently at the forefront of genetic engineering. It has led to several transformative advances thanks to its simplicity, versatility, flexibility and ability to precisely manipulate cellular genomes and correct mutations. As an experimental tool, it has tremendous power to help researchers develop and manipulate experimental models designed to correct inherited genetic alternations in hematologic diseases such as sickle cell anemia, thalassemia and hemophilia. The correction of genetic defects that cause these disorders would allow for cure, rather than life-long palliation.

  While gene editing represents a highly promising area for potential treatment of hematologic disorders, several critical questions still need to be addressed in order to establish appropriate processes that will guide the safe and effective transfer of its use into the clinic. NHLBI is encouraged to further its research efforts in genome editing and gene therapy by focusing on the following priority areas:

  - **Establishing strategies to determine the efficacy, safety, and toxicity of genome editing techniques.** Basic science research and the development of proper clinical trial infrastructure is needed to further advance our understanding of the biology of genome editing. Preclinical research is essential to help determine the accuracy, safety and
efficiency of this technology in order to help minimize off-target mutations and to reduce toxicity. Once the preclinical efficacy of this technology is established, its transfer into a well-established clinical trial structure will be critical in helping to understand its application in humans.

- **Applying genome editing technology to correct hematologic disorders.** Single nucleotide variants that result in hemoglobinopathies like thalassemias or sickle cell disease are ideal platforms for initial research programs; however, studies are still needed to determine which other disorders are amenable to genome editing correction, whether certain disorders can be characterized by more complex mutations, and which gene alterations should be targeted.

- **Ensuring Coordination and Collaboration between Federal Agencies with an Interest in Sickle Cell Disease**

  Sickle cell disease (SCD) is the most common inherited red blood cell disorder in the United States, affecting 70,000-100,000 Americans (mostly, but not exclusively, of African ancestry). SCD causes the production of abnormal hemoglobin, a protein that attaches to oxygen in the lungs and carries it to all parts of the body. Healthy red blood cells are flexible so that they can move through the smallest blood vessels. In sickle cell anemia, the hemoglobin is abnormal, causing the red blood cells to be rigid and shaped like a "C" or sickle, the shape from which the disease takes its name. Sickle cells can get stuck and block blood flow, causing pain and infections. Complications of sickle cell anemia are a result of sickle cells blocking blood flow to specific organs, and include stroke, acute chest syndrome (a condition that lowers the level of oxygen in the blood), organ damage, other disabilities, and in some cases premature death.

  Although the molecular basis of SCD was established several decades ago, it has been challenging to translate this knowledge into the development of novel targeted therapies. New approaches in managing this disease have improved diagnosis and supportive care over the last few decades, but many patients still have severe complications to overcome. The future of care for SCD patients will be dependent on advanced and highly targeted approaches to research, discovery, and implementation of proven and new interventions.

  To ensure that individuals with SCD receive state of the art care, it is important that key stakeholders, including federal agencies, work together to invest in SCD-related research and initiatives that could truly move the field forward with the hope of curing SCD in the future. A multi-agency approach would deliver advances faster, more economically, and more efficiently to patients suffering from this debilitating disease in the United States and the world. The Department of Health and Human Services Interagency Working Group on SCD is essential to ensuring the coordination and collaboration between federal agencies with an interest in SCD.

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 2016 requests.