The American Society of Hematology (ASH) thanks the Subcommittee for the opportunity to submit written testimony on the fiscal year (FY) 2017 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. Much 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 such as sickle cell disease and thalassemia – continue to be associated with significant morbidity and mortality and demand attention to reduce their burden and improve the quality of care worldwide.

FY 2017 Requests

NIH Funding
ASH thanks Congress for the robust bipartisan support that resulted in the welcome and much needed funding increase for the NIH that Congress provided in the FY 2016 Consolidated Appropriations Act. ASH supports the Ad Hoc Group for Medical Research recommendation that NIH receive at least $34.5 billion in FY 2017 as the next step toward a multi-year increase in our nation's investment in medical research. If the nation is to continue to accelerate the development of life-changing cures, pioneering treatments, and innovative prevention strategies, it is essential to sustain predictable increases in the NIH budget. 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. This requested $2.4 billion increase represents 5 percent real growth above the projected rate of biomedical inflation, and will help ensure that NIH-funded research can continue to improve our nation’s health and enhance our competitiveness in today’s global information and innovation-based economy.

Additionally, the Society strongly supports the Administration’s proposed Moonshot Initiative, which seeks to accelerate progress across all cancers by supporting research in cancer prevention and vaccine development, early detection, immunotherapy and combination therapy, genomic analysis, data sharing, and pediatric cancer.
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 over $4 million in the Consolidated Appropriations Act of 2014. ASH respectfully requests that the Committee restore funding for the Division of Blood Disorders, by including increased funding to the public health approach to blood disorders account to enable CDC to meet growing needs for programs to address sickle cell disease and deep vein thrombosis/pulmonary embolism (DVT/PE). This funding will allow CDC to improve health outcomes and limit complications to those who are risk or currently have bleeding and clotting disorders, by promoting a comprehensive care model; identifying and evaluating effective prevention strategies; and increasing public and healthcare provider awareness.

Additional Activities

In FY 2017, ASH also urges the Subcommittee to recognize the following activities impacting hematology:

- **Centers for Disease Control and Prevention, National Center on Birth Defects and Developmental Disabilities**
  - **Report Language:**
    - Sickle Cell Disease – The Committee believes more can be done to educate patients and medical providers about sickle cell disease (SCD) and sickle cell trait (SCT). It is especially important that individuals know their sickle cell status, the potential for medical complications, and the implications when making reproductive choices and that providers be informed of the current recommendations (best practices) for providing medical care to individuals with SCD/SCT. The Committee asks that the Center’s Blood Disorders Division provide a plan on how to carry out a public health awareness and education campaign to meet these goals.
  - **Background:**
    - Sickle cell disease is the most common inherited red blood cell disorder in the United States, affecting approximately 100,000 Americans (mostly but not exclusively of African ancestry). SCD causes the production of abnormal hemoglobin, which can get stuck and block blood flow, causing pain and infections. Complications of sickle cell anemia include stroke, acute chest syndrome, organ damage, other disabilities, and in some cases premature death. Most SCD patients can expect to
live into adulthood, but the cost of care and the burden of pain, end-organ injury, and premature death remain high.

- **Centers for Medicare and Medicaid Services, Program Management**
  - **Report Language:**
    - *Sickle Cell Disease* – The Committee encourages CMS working through the Center for Medicare and Medicaid Innovation to explore with the interested provider and patient organizations, the development of model programs to provide integrated comprehensive care for adults with sickle cell disease (SCD). With an estimated 50% of the SCD population served under Medicaid and another 25% on Medicare, CMS has every incentive to assure that individuals with SCD are able to access specialized high quality services. Consideration should be given to funding of care coordinators/case managers for this population with incentives to reduce hospital admissions/readmissions and emergency department visits.

  - **Background:**
    - Sickle cell disease is the most common inherited red blood cell disorder in the United States, affecting approximately 100,000 Americans (mostly but not exclusively of African ancestry). SCD causes the production of abnormal hemoglobin, which can get stuck and block blood flow, causing pain and infections. Complications of sickle cell anemia include stroke, acute chest syndrome, organ damage, other disabilities, and in some cases premature death. According to the Agency for Healthcare Research and Quality (AHRQ) Healthcare Cost and Utilization Project – 2012, Sickle cell disease was the 5th most common discharge diagnosis for hospital “super users” for Medicaid patients under 64 and patients with SCD are high utilizers of emergency room services. Most SCD patients can expect to live into adulthood, but the cost of care and the burden of pain, end-organ injury, and premature death remain high.

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 2017 requests.