American Society of Hematology
Statement to the Senate Appropriations
Subcommittee on Labor, HHS, Education, and Related Agencies
FY 2020 Funding for NIH and CDC
April 8, 2019

The American Society of Hematology (ASH) represents more than 17,000 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. Hematologists have been pioneers in the fields of bone marrow transplantation, stem cell biology and regenerative medicine, and gene and immunotherapy.

FY 2020 Request: NIH Funding
ASH thanks Congress for the robust bipartisan support that has resulted in several consecutive years of welcome and much needed funding increases for the National Institutes of Health (NIH), including the $2 billion increase that Congress provided in fiscal year (FY) 2019. For FY 2020, ASH strongly supports the Ad Hoc Group for Medical Research recommendation that NIH receive at least $41.6 billion. This funding level, supported by more than 300 other stakeholder organizations, would allow for meaningful growth above inflation in the base budget that would expand NIH’s capacity to support promising science in all disciplines. It also would ensure that funding from the Innovation Account established in the 21st Century Cures Act would supplement the agency’s base budget, as intended, through dedicated funding for specific programs.

American biomedical research has led to new medical treatments, saved innumerable lives, reduced human suffering, and spawned entire new industries, none of which would have been possible without support from NIH. Hematology research, funded by 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), has been an important component of this investment in the nation’s health.

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. Basic research on blood has aided physicians who treat patients with heart disease, strokes, end-stage renal disease, cancer, and AIDS.
However, while some blood disorders have benefited from tremendous progress in clinical research and development of new therapies, other areas have continuing challenges and have evaded effective treatment to date. A wide variety of blood-related diseases – from malignancies such as lymphoma and leukemia to non-malignant diseases such as sickle cell disease, platelet and coagulation disorders, and orphan diseases of the hematopoietic system – continue to be associated with significant morbidity and mortality and demand attention to reduce their burden and improve quality of care nationwide.

**FY 2020 Request: Report Language Supporting Gene and Cellular Therapies**
ASH is encouraged that there are a number of gene and cellular therapies in clinical trials that hold tremendous promise for patients. The approval of the first chimeric antigen receptor T-cell (CAR-T) therapy by the Food and Drug Administration (FDA) in August 2017 marked an important shift in the blood cancer treatment paradigm. CAR-T therapy is an innovative treatment for certain patients with leukemia and lymphoma.

While these therapies are potentially curative in many patients who have typically exhausted all other treatment options, patient access is currently limited because Medicare reimbursement falls significantly short of covering the high cost of these therapies, leading to concern that if reimbursement for these therapies does not improve, it will threaten innovation in this field. To support continued innovation and ensure patient access, the Society urges the inclusion of report language requiring all of the Department of Health and Human Services (HHS) agencies involved in the research, approval, and reimbursement for gene and cellular therapies, including NIH, the FDA, and the Centers for Medicare and Medicaid Services (CMS), to proactively harmonize their policies to ensure patients will have access to medically appropriate, approved gene and cellular therapies.

**FY 2020 Request: Centers for Disease Control and Prevention (CDC)**
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.

Sickle cell disease (SCD) is an inherited, lifelong disorder affecting nearly 100,000 Americans. Individuals with the disease produce abnormal hemoglobin which results in their red blood cells becoming rigid and sickle-shaped, causing them to get stuck in blood vessels and block blood and oxygen flow to the body. SCD complications include severe pain, stroke, organ damage, and in some cases premature death. Though new approaches to managing SCD have led to improvements in diagnosis and supportive care, many people living with the disease are unable to access quality care and are limited by a lack of effective treatment options.

Surveillance is necessary to improve the understanding of outcomes and health care system utilization patterns, increase evidence for public health programs, and establish cost-effective practices to improve and extend the lives of people with SCD. With funding from the CDC Foundation, CDC has established a population-based data collection system to gather and analyze longitudinal data about people living in the U.S. with SCD. However, due to limited funding, implementation of the program has occurred in only two states – California and Georgia (representing approximately 10% of the U.S. SCD population).
CDC’s SCD Data Collection Program should be maintained and expanded to include additional states with the goal of covering the majority of the US SCD population over the next 5 years. In order for expanded data collection to become a reality, funding must be provided. The *Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention, and Treatment Act of 2018* (P.L. 115—327) authorizes CDC to award SCD data collection grants to states, academic institutions, and non-profit organizations to gather information on the prevalence of SCD and health outcomes, complications, and treatment that people with SCD experience. For FY 2020, the Society urges the Subcommittee to provide at least $2 million for SCD data collection within the CDC’s National Center for Birth Defects and Developmental Disabilities, Division of Blood Disorders. This funding would allow CDC to expand the Data Collection Program to include additional states, with the goal of covering the majority of the U.S. SCD population over the next 5 years.

Additionally, ASH supports the public health community’s request for at least $7.8 billion in funding for the CDC in FY 2020. This funding level would build upon the funding increase Congress provided CDC in FY 2019 and strengthen all of CDC’s programs. ASH also urges continued support of the Public Health and Prevention Fund which has supported many critical projects at CDC, including investments in health-care associated infections. Currently the fund comprises more than 10 percent of CDC’s budget. ASH is concerned about the repeated efforts to eliminate this fund because of the budgetary pressure this would place on other programs within the Subcommittee’s jurisdiction.

Thank you again for the opportunity to submit testimony. Please contact ASH Senior Manager, Legislative Advocacy Tracy Roades at 202-776-0544 or *troades@hematology.org*, if you have any questions or need further information concerning hematology research or ASH’s FY 2020 requests.