



**American Society of Hematology
Statement to the Senate Appropriations
Subcommittee on Labor, HHS, Education, and Related Agencies
FY 2019 Funding for NIH and CDC
June 1, 2018**

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. 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.

FY 2019 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 \$3 billion increase that Congress provided in the FY 2018 Consolidated Appropriations Act. For FY 2019, ASH strongly supports the Ad Hoc Group for Medical Research [recommendation](#) that NIH receive at least \$39.3 billion, including funds provided to the agency through the 21st Century Cures Act's Innovation Account for targeted initiatives. This funding level, supported by more than 200 other stakeholder organizations, would continue the momentum of recent years by enabling meaningful base budget growth above inflation to expand NIH's capacity to support promising science in all disciplines, including hematology, and also would ensure that the Innovation Account supplements the agency's base budget, as intended, through dedicated funding for specific programs. Securing a reliable, robust budget trajectory for NIH will be key in positioning the agency to capitalize on the full range of research in the biomedical, behavioral, social, and population-based sciences. Given the abundance of scientific opportunity, this recommendation represents a minimum investment to sustain progress that only would be amplified through an even more robust commitment.

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, none of which would have been possible without support from NIH. Funding for hematology research has been an important component of this investment in the nation's health. 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, including three therapies approved in 2016).

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.

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.

The approval of chimeric antigen receptor T-cell (CAR-T) therapy by the Food and Drug Administration in August 2017 marked an important shift in the blood cancer treatment paradigm. CAR-T therapy is an innovative new treatment for certain patients with leukemia and lymphoma. We now have proof that it is possible to eradicate cancer by harnessing the power of a patient's own immune system. This is a potentially curative therapy in patients who have typically exhausted all other treatment options, including chemotherapy, radiation, or stem cell transplant, and represents the latest milestone in the shift away from chemotherapy toward precision medicine. The FDA's approval of this groundbreaking therapy was the result of over a decade of hematology research, including research funded by the NIH.

However, while the importance of CAR-T cannot be overstated, this approval only pertains to a small population of patients. More research is needed to make this therapy more effective for a broader population, to reduce the severe side effects that patients experience during treatment, and ultimately to find a broader application beyond blood cancers. Continued research will also lead to improved manufacturing of large numbers of cells, which is necessary to make this therapy accessible to more patients.

ASH has created several [videos](#) highlighting the progress made, and the future promise, in areas such as immunotherapy, precision medicine, and genomic profiling.

FY 2019 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 and causing them to get stuck in blood vessels and block blood and oxygen flow to the body. SCD complications include severe pain, stroke, acute chest syndrome (a condition that lowers the level of oxygen in the blood), 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 understanding of the health outcomes and health care system utilization patterns, increase evidence for public health programs and to 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 surveillance system to collect and analyze longitudinal data about people living in the U.S. with SCD. Data is being collected from multiple sources (newborn screening programs and Medicaid) in order to create individual healthcare utilizations profiles. However, due to limited funding, implementation of the program has occurred only in two states – California and Georgia (approximately 10% of the U.S. SCD population).

CDC's SCD Surveillance 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. For FY 2019, the Society urges the Subcommittee to provide dedicated funding for SCD surveillance, outreach, and education programs to the CDC's Blood Disorders Division within the National Center on Birth Defects and Developmental Disabilities. Funding is needed for coordination and implementation of a training curriculum in the states with large SCD populations. CDC should develop a comprehensive, national public health awareness campaign for people with SCD and sickle cell trait (SCT, when a person carries a single gene for sickle cell disease and can pass this gene along to their children), their families, and the general public along with an educational campaign for the medical professionals who provide health care for people living with SCD or SCT. The goals of this effort would be to improve overall awareness of SCD and SCT and knowledge about health outcomes and to provide educational tools for healthcare professionals to help them understand the effects of medical interventions and inform best practices for SCD.

Additionally, ASH is supportive 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 approximately 12 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.

Finally, ASH supports the request recently made by 81 national medical, public health, and research organizations to provide funding for the CDC to conduct public health research into firearm morbidity and mortality prevention. Federally funded public health research has a proven track record of reducing public health-related deaths, whether from motor vehicle crashes, smoking, or Sudden Infant Death Syndrome. This same approach should be applied to increasing gun safety and reducing firearm-related injuries and deaths, and CDC research will be as critical to that effort as it was to these previous public health achievements. The foundation of a public health approach is rigorous research that can accurately quantify and describe the facets

of an issue and identify opportunities for reducing its related morbidity and mortality. Robust research on motor vehicle crashes and subsequent legislation has helped save hundreds of thousands of lives through public health interventions including seat belts and other safety features. The same approach can help reduce gun violence in our communities, including ensuring CDC is able to adequately fund and perform research into this public health priority.

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 2019 requests.