



Fiscal Year 2013 Statement of the American Society of Hematology

House Appropriations Subcommittee on Labor, HHS, Education, and Related Agencies

March 29, 2012

The American Society of Hematology (ASH) thanks the Subcommittee for the opportunity to submit written testimony on the fiscal year (FY) 2013 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.

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. 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. 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.

FY 2013 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 2013 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.

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.

For FY 2013, ASH Seeks Congressional Support for the Following Activities:

In FY 2013, ASH also urges the Subcommittee to recognize the following areas of hematology research that have shown impressive progress and offer the potential of future advances:

Stem Cells and Regenerative Medicine: Improving Current Technologies to Cure Blood Disorders

Hematologists have been at the forefront of research in stem cell biology by studying blood cell development and exploring stem cells' potential to repair damaged tissue, fight infections, and reduce autoimmune diseases. The techniques and principles used by hematologists in studying the blood system stem cells have been applied to stem cells from many other tissues with great success, spawning a huge research effort across all areas of medicine.

Researchers have made significant progress in developing re-programmed adult cells, called induced pluripotent stem (iPS) cells, which can subsequently develop into any tissue of the body. iPS cells can be generated and used in patients who have genetic blood diseases as well as other complex diseases because they will not be attacked by a patient's own immune system, they serve as a continuous source of cells, and they are amenable to genetic manipulation.

Recent research has suggested that iPS cells can be manipulated to become blood stem cells and can be used as a transplant source for patients who do not have a matched donor. This will greatly enhance bone marrow and cord blood stem cell transplantation for the treatment of blood cancers and other hematologic disorders and subsequently inform our understanding of transplantation-related morbidities for other organs. iPS-generated red blood cells from rare blood types also could be used in blood banking as reagents to identify patients and blood units suitable for transfusion.

Future stem cell advances are highly dependent on the ability to transplant stem cells at high efficiencies and then have them perform well once transplanted. However, several barriers remain that currently prevent the clinical translation of iPS cell technology. Compared to other sources of stem cells, iPS cells have slower growth kinetics, are more genomically unstable, and have decreased efficiency for differentiation. These barriers are also important areas for future research.

ASH applauds the efforts of the National Heart, Lung, and Blood Institute (NHLBI) to conduct further research in the development of blood stem cells from iPS cells and to address the barriers to the clinical translation of iPS cell technology.

Research in Sickle Cell Trait and Exercise-Related Illness

Sickle cell disease (SCD) is an inherited blood disorder that affects 80-100,000 Americans, mostly but not exclusively of African ancestry. SCD causes production of abnormal hemoglobin, resulting in severe anemia, pain, other devastating disabilities, and, in some cases, premature death.

Eight to 10 percent of African Americans have sickle cell trait. Individuals with sickle cell trait do not have SCD, but are carriers of one defective gene associated with SCD. Millions of Americans with sickle cell trait enjoy normal life spans without serious health consequences. At the same time, possible health risks have been reported for individuals with sickle cell trait including increased incidence of renal failure and malignancy, thromboembolic disorders, splenic infarction as a high altitude complication, and exertion-related sudden death.

In April 2010, the National Collegiate Athletic Association (NCAA) adopted a policy requiring Division I institutions to perform sickle cell trait testing for all incoming student athletes. This policy has been

controversial because there are no high quality (well-controlled, hypothesis-driven, prospective) studies on sickle cell trait and exertional collapse or evidence to justify it.

There is a need for increased biomedical and population-based research on sickle cell trait and its relation to exertion-related illness as well as other conditions. Based on its 2010 Consensus Conference on this topic, NHLBI has identified a research agenda and ASH, the American Academy of Sports Medicine, and the NCAA have met to discuss potential studies to pursue. It is important that the research agenda is moved forward collaboratively under the direction of the NHLBI.

Conclusion:

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.

ASH urges the Subcommittee to continue to be a champion for research and support at least \$32 billion in funding for NIH in fiscal year 2013. The American people are depending on you to ensure the nation does not lose the health and economic benefits of our extraordinary commitment to medical research.

Thank you again for the opportunity to submit testimony. Please contact Tracy Roades, ASH Research Advocacy Manager, at 202-776-0544 or troades@hematology.org, or Ulyana Desiderio, PhD, ASH Senior Manager for Scientific Affairs, at 202-776-0444 or udesiderio@hematology.org, if you have any questions or need further information concerning hematology research or ASH's FY 2013 funding request.