March 4, 2016

Office of Science Policy, Engagement, Education, and Communications
National Heart, Lung, and Blood Institute
National Institutes of Health
31 Center Dr., Building 31, Room 4A10
Bethesda, MD, 20892-2480

RE: Request for Comments: NHLBI Strategic Visioning: Draft Strategic Research Priorities

Submitted electronically to: NHLBI_Vision@mail.nih.gov

Dear Sir or Madam,

The American Society of Hematology (ASH) is pleased to see the release of the National Heart, Lung and Blood Institute’s (NHLBI) draft strategic research priorities and is grateful for the opportunity to provide additional comments based on the ASH Agenda for Hematology Research.

ASH represents over 15,000 clinicians and scientists worldwide who are committed to the study of blood and blood-related diseases. These disorders encompass malignant hematologic disorders, such as leukemia, lymphoma, multiple myeloma, and non-malignant conditions, such as sickle cell anemia, thalassemia, aplastic anemia, venous thromboembolism, and hemophilia. In addition, hematologists have been pioneers in the fields of stem cell biology, regenerative medicine, bone marrow transplantation, transfusion medicine, gene therapy, and the development of many drugs for the prevention and treatment of heart attacks and strokes. ASH membership is comprised of basic, translational, and clinical scientists, as well as physicians working in diverse settings, including universities, hospitals, and private practices. The Society’s members have a vested interest in participating in NHLBI’s strategic planning process, in order to foster bench-to-bedside research, and to facilitate the timely and efficient delivery of necessary interventions to patients.

Hematologists have made vital contributions to the medical field at large, and specifically in the areas of stem cell biology and regenerative medicine. ASH strongly supports the NHLBI’s interest in standardizing protocols for functional studies needed to establish and maintain cultured cell lines, in order to better understand normal biological functions. Such studies could foster the effective production of clinically functional blood products, such as red blood cells derived from autologous induced pluripotent stem cells, which could potentially replace allogeneic products in highly immunized patients. In addition, ASH believes that the development and application of comprehensive single-cell biology analytics will also be needed to facilitate an integrated understanding of cellular diversity, and cell to cell interactions in blood diseases.
ASH is in accord with the NHLBI on the importance of investigating new pathobiological mechanisms in order to improve health and disease. In hematological diseases, such as venous thromboembolism (VTE), the development of improved animal models will be crucial in advancing our understanding of VTE biological mechanisms. This will allow for more accurate evaluation of emerging biomarkers and initial assessments of potential therapeutic interventions for this disease. Furthermore, the identification and prioritization of such novel biomarkers will also foster the understanding of molecular mechanisms underlying VTE, and will shepherd the development of novel therapies.

ASH also supports the NHLBI’s focus on advancing research in areas such as genomics, proteomics, and other “omics” to foster progress towards precise and individualized treatment of patients. Although there is diversity in the molecular abnormalities that underlie hematologic disorders, common threads, such as alterations in proteins that function in epigenetic mechanisms are now emerging. Additional research focusing on epigenetic alterations and emerging targets is needed to identify the role of such proteins in the development of hematologic disorders, in order to design potential targeted treatments to counter their effects. Furthermore, genetic and epigenetic alterations that drive hematologic diseases, and the extent to which normal cells are distinct from malignant cells, also needs to be more broadly elucidated, since many blood diseases, including hematopoietic cancers, disturb epigenetic regulators. ASH believes that the knowledge gained from research in these areas will lay the groundwork for precision medicine, and will help to provide more insight on potentially critical determinants of responsiveness to therapeutic regimens.

On the subject of novel diagnostic and therapeutic strategies, ASH believes that inherited monogenic hematologic diseases, such as hemophilia, beta-thalassemia, and sickle cell disease are prime targets for future application of genome editing technology. However, studies are still needed to advance our understanding of the biology of genome editing, in order to determine its accuracy, safety, and efficiency, as well as to identify other disorders that are amenable to genome editing correction. ASH believes that the gene correction strategies developed for inherited blood disorders will also be viable for other hematologic diseases and autoimmune disorders.

Finally, ASH strongly supports the NHLBI’s objective of developing a diverse and sustainable scientific and physician workforce. The Society has committed significant resources to career-enhancement awards and training programs to protect the field of hematology, and to those who have dedicated their careers to the specialty. Specifically, ASH has been a leader in promoting diversity within the scientific workforce through its long standing and highly successful Minority Recruitment Initiative. The Society will be expanding programming within the initiative over the next twelve months. Furthermore, the awards issued through the ASH Bridge Grant Program have made a significant and positive impact on the field and in the workforce, with more than 60% of the initial three cohorts successfully converting to an R01 or equivalent NHLBI Award. ASH looks forward to continuous collaboration with the NHLBI on this vital issue.

Additionally, ASH is aware of the National Institutes of Health’s (NIH) efforts to implement recommendations from the Physician-Scientist Workforce Working Group of the Advisory Committee to the Director. The Society is pleased that some of these efforts envision the creation of additional partnerships between the NIH and professional societies, as well as the creation of novel pilot programs. The Society encourages the NHLBI to engage fully in these efforts to implement the working group’s recommendations, and to explore with ASH opportunities for novel approaches to address this critical workforce issue.
ASH would like to thank the NHLBI for the opportunity to provide additional comments on its draft strategic research priorities. The Society looks forward to working with the NHLBI on these important priority areas, and to provide further information, and be a resource for the Institute. Please contact for any additional information the ASH Director for Scientific Affairs, Ulyana V. Desiderio, PhD, at udesiderio@hematology.org or ASH’s Scientific Affairs Specialist, Alice Kuaban, MS, at akuaban@hematology.org.

Sincerely,

Charles S. Abrams, MD
President