Statement for the Record from the American Society of Hematology for the Senate Committee on Appropriations Hearing: Biomedical Research: Keeping America's Edge in Innovation

April 30, 2025

The American Society of Hematology (ASH) thanks the Senate Appropriations Committee ("the Committee") for holding the hearing "Biomedical Research: Keeping America's Edge in Innovation" to examine the importance of U.S. biomedical research for the nation's health and economy and for the opportunity to provide this statement for the record.

ASH represents more than 18,000 clinicians and scientists committed to the study and treatment of blood and blood-related diseases, including malignant disorders such as leukemia, lymphoma, and myeloma, as well as classical (non-malignant) conditions such as sickle cell disease (SCD), thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. Hematologists have been pioneers in advancing understanding and treatment of various diseases and continue to be innovators in the fields of stem cell biology, regenerative medicine, transfusion medicine, and gene therapy. ASH membership is comprised of basic, translational, and clinical scientists, as well as physicians providing care to patients. The Society's mission is to foster high-quality care, transformative research, and innovative education to improve the lives of patients with blood and bone marrow disorders.

Medical research funded through the National Institutes of Health (NIH), the largest source of public funding for medical research in the world, has been a driving force behind many decades of advances that have improved the health of people in every state and community, providing cures and hope for patients and caregivers. NIH-supported hematologic research has also helped pave the way for many discoveries both within and outside of hematology. Discoveries made by hematologists have led to extraordinary advances in other fields of medicine, including new and better treatments for some of the world's deadliest and costliest diseases such as heart disease and stroke. For example, new antithrombotic treatments have lowered the risk of blood clots in leg veins by more than 70 percent and deaths from heart attacks have been reduced by around 50 percent. Critical hematology research is supported across NIH by many of its 27 institutes and centers, including the National Heart, Lung and Blood Institute (NHLBI), the National Cancer Institute, and the National Institute of Diabetes, Digestive and Kidney Diseases. This work is essential to advancing our understanding and treatment of blood disorders and improving patient outcomes.

The practice of hematology has benefited from NIH investments which have led to the development of cutting-edge therapies in the field of hematology, including gene therapies for SCD, thalassemia, and hemophilia, and chimeric antigen receptor (CAR) T-cell therapies for certain blood cancers. Few treatments are available to help individuals with SCD manage the pain crises associated with this genetic disease. Prior to the 2023 approval of two gene therapies for SCD, a blood and marrow transplant was the only curative option. However, research conducted by NHLBI and the National Human Genome Research Institute played a critical role in developing these two groundbreaking gene therapies offering patients a new curative option. Additionally,

CAR-T therapy, first approved for children with acute lymphoblastic leukemia (ALL) and certain adults with large B-cell lymphoma, has provided a vital treatment option for individuals with relapsed or treatment-resistant cancers. The first pediatric patient to receive CAR-T to treat ALL was at the NIH Clinical Center in 2012, with NIH support helping advance the clinical trials that made this therapy a reality.

These innovations are changing the practice of classical and malignant hematology (and in many other areas of medicine). The <u>ASH Agenda for Hematology Research</u> highlights key emerging and transformative areas of research that will launch the field into the next generation of therapies for hematologic conditions.

NIH-supported research occurs in every state and nearly every congressional district¹ and NIH funding directly and indirectly supports hundreds of thousands of jobs nationwide, including nearly 408,000 jobs supported in FY 2024.² Should there be cuts to the NIH, many institutions would no longer be able to afford the costs necessary to perform research or would be forced to significantly scale back their efforts. Across institutions jobs would be lost, laboratories would close, and the research workforce capacity would be cut. Cuts to NIH will result in fewer clinical trials, less fundamental discovery research, slower progress delivering new innovations and lifesaving advances, and erosion of U.S. leadership in biomedical research.

Recent executive orders and administration policies, as well as the threat of cuts to NIH's budget, are already significantly impacting clinical, translational, and basic research, in addition to clinical care. Since the start of the year, NIH has already scaled back new grant awards by \$2.3 billion.³ Delayed grant reviews and reductions in research funding in any area will cause years of damage and prevent patients from seeing the impacts of improved diagnostic and treatment options. Furthermore, the continued uncertainty is threatening both the future research workforce and the job security of Americans across the country, particularly those who provide administrative and laboratory support in research centers. The Society has already received examples from members highlighting the devastating impact this tumultuous and uncertain time has already had on hematology:

- A member at an institution working toward becoming an NCI-Designated Cancer Center noted that the institution is now facing a \$12 million dollar funding cut, which will limit opportunities to open clinical trials and keep the institution fully operational.
- A research center in the Midwest has frozen hiring and rescinded offers to PhD researchers. Institutional support for research staff has also stopped, putting responsibility on the principal investigator to support staff or let them go. Researchers are concerned about funding for support staff and are having to focus on what projects have the greatest chance for funding. Lack of funding impacts growth in labs and decreases innovative projects that were in progress.

https://www.faseb.org/science-policy-and-advocacy/federal-funding-data

https://www.unitedformedicalresearch.org/annual-economic-report/

¹ Federation of American Societies for Experimental Biology. Federal Research Funding Data.

² United for Medical Research. NIH's Role in Sustaining the U.S. Economy.

³ https://www.statnews.com/2025/04/24/trump-100-days-nih-new-grants-

cut/#:~:text=This%20analysis%20does%20not%20include,clawed%20back%20by%20the%20NIH.

• For the past five years, a researcher at an institution in the Northeast has received funding from the NIH to study how to prevent blood clotting and bleeding in hospitalized patients. After successful completion of the initial grant, a competitive renewal was submitted in November. This next phase was designed to translate the initial research into improved patient care, in collaboration with colleagues in Vermont, Oklahoma, Texas, Michigan, Minnesota, and Pennsylvania. Although the grant renewal was scheduled for review in February, applicants were informed right before the scheduled review date that the study section review meeting had been postponed until late spring. Delays and cancelations are happening frequently as administrators who organize study sections have been dismissed or reassigned. These delays disrupt not only research but also the economy and healthcare innovation.

ASH urges the Committee to ensure that all current congressionally appropriated funds that support biomedical research are spent as Congress intended. Additionally, as the Committee considers funding for the upcoming fiscal year, ASH joins with the biomedical research community in noting that meaningful annual funding growth for NIH has been a steadfast bipartisan national priority over the last decade and beyond. In a time where global competitors continue to increase investments in medical research and scientific talent, it is crucial that the United States maintains its leadership on the global stage to deliver breakthrough advances for patients and economic growth for communities across the country. We must not lose the momentum of decades of progress in hematology. The Society urges the Committee to continue to support patients, discovery, and economic growth by providing adequate investment in NIH.

Additionally, ASH recognizes that changes are being made at the NIH as part of the Department of Health and Human Services (HHS) reorganization, including centralization of certain functions, like procurement and communications, and the potential consolidation and elimination of the 27 institutes and centers. We recognize that even successful agencies like NIH can be improved and recognize that new thinking may invigorate research on chronic diseases, cancers, and other topics as well as enhance the reliability of NIH-funded science. As the Committee examines NIH's existing programs and strives to spend federal dollars more efficiently, ASH encourages the Committee to partner with stakeholders to best understand how NIH's programs are working to meet the agency's mission. Changes to the agency's structure should undergo a bicameral, bipartisan authorization process, with the goal of optimizing federal investment to enhance health, lengthen life, and reduce illness and disability, while ensuring the essential functions of the agency – investing in basic and clinical research and training the next generation of researchers across the existing institutes and centers areas of study – are preserved. Furthermore, the results and impacts on human health of funded research must be clearly communicated to the public such that they understand why continued investment in biomedical research is essential.

ASH also recognizes the many other federal agencies that support medical research, including the research supported through the Department of Defense (DoD) Congressionally Directed Medical Research Program (CDMRP), which administers critical and highly successful defense health research programs, including biomedical research that supports basic, translational, and clinical research projects; research training; and research infrastructure. Over the years, numerous disease programs have been added, and CDMRP is currently a collection of programs that support cutting-

edge research across a broad range of diseases and conditions, including both classical and malignant hematologic conditions:

- The Peer Reviewed Medical Research Program (PRMRP) supports medical research projects of clear scientific merit that lead to impactful advances in military health care. Our understanding of and ability to combat SCD and understand sickle cell trait in the broader military community is already advancing due to inclusion in the list of eligible research areas for the program in fiscal year (FY) 2023. The U.S. military screens all members for sickle cell trait, demonstrating the importance of funding research to better understand sickle cell trait and SCD, and mitigate the impact of the disease on the children and family members of our nation's armed forces.
- The Peer Reviewed Cancer Research Program (PRCRP) promotes high-impact research in cancer prevention, detection, treatment, quality of life, and survivorship for military service members, their families, veterans, and the general public. The PRCRP has funded projects in myeloma, lymphoma, and pediatric, adolescent, and young adult blood cancers. A recently published <u>Case Review on Multiple Myeloma Patients with Military Services and Toxin Exposures</u> revealed a disproportionately high occurrence of multiple myeloma in patients with recorded history of military exposure to toxic agents when compared to other groups (9% vs 0.1% to 0.4%), demonstrating the need for and importance of further investigations.
- The Bone Marrow Failure Research Program (BMFRP) encourages and supports innovative research that is committed to advancing the understanding and treatment of inherited and acquired bone marrow failure diseases. This funding line includes research in aplastic anemia, myelodysplasia, paroxysmal nocturnal hemoglobinuria, and pure red cell aplasia.
- The Rare Cancer Research Program (RCRP) elevates rare cancer research to catalyze knowledge-building and enable clinically impactful discoveries for the benefit of service members, their families, veterans, and/or the American public. Meritorious research in hematopoietic cancers has been supported by the RCRP.

Unfortunately, CDMRP received a 57 percent cut in funding in the FY 2025 continuing resolution (CR). Cuts to the CDMRP of this magnitude will delay progress in understanding and treating blood and bone marrow disorders, and ultimately delay advancements in the health of affected service members, veterans, and the broader public. As the Committee begins work on FY 2026 funding bills, the Society urges the restoration of funding to the DoD CDMRP.

Thank you, again, for the opportunity to provide this statement for the record. ASH looks forward to continuing to work with you to support biomedical research and protect and enhance the nation's role as the world's leader in biomedical innovation.