

April 28, 2025

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Dear Dr. Bhattacharya,

As the newly confirmed Director of the National Institutes of Health (NIH), the American Society of Hematology (ASH) writes to you today, to share our commitment with you and the Administration to reduce the burden of chronic disease through the important work of your agency. ASH is grateful for the leadership of the NIH as the driving force behind many decades of advances that have improved health outcomes, alleviated suffering, provided hope, and saved countless numbers of lives of Americans and others around the globe.

ASH represents more than 18,000 clinicians and scientists worldwide who are committed to the study and treatment of blood and blood-related diseases. These disorders encompass malignant hematologic disorders such as leukemia, lymphoma, and multiple myeloma, as well as classical hematologic conditions such as sickle cell anemia, thalassemia, bone marrow failure, venous thromboembolism, and hemophilia. In addition, hematologists are pioneers in developing innovative approaches to advance medicine specifically in the fields of stem cell biology, regenerative medicine, transfusion medicine, and cell and gene therapy. Our mission is to foster high-quality care, transformative research, and innovative education to improve the lives of all patients with blood and bone marrow disorders.

NIH-supported hematologic research has helped pave the way for many discoveries both within and outside of hematology. Research in chronic myelogenous leukemia (CML) and specifically, analysis of the CML-specific chromosomal translocation, resulted in the development of ibrutinib, a genetargeting drug that allows patients to take a non-toxic oral drug instead of radiation treatment; more than 75 percent of patients diagnosed with CML achieve a durable remission with this therapy. Discoveries made by hematologists have also 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, research that led to new antithrombotic treatments has 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. These are the types of discoveries in hematology that have advanced our understanding of blood disorders and improved patient outcomes.

It is imperative that individuals with blood diseases and disorders have the promise of continued advancement in innovative therapies; biomedical research, funded by the NIH has been the foundation of breakthroughs that now benefit millions of Americans.

It is with this in mind that the Society shares our interest in working with you to protect and advance innovation in hematology research; promote the next generation of the research workforce; and increase expertise in the peer review process. The ASH leadership respectfully requests a meeting with you to further discuss these priorities.

Protecting and Advancing Hematology Research

Critical hematology research is currently supported by leading NIH institutes and centers, including the National Heart, Lung, and Blood Institute (NHLBI), National Cancer Institute, National Institute of Diabetes and Digestive and Kidney Diseases, and National Institute on Aging, among others. 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 Sickle Cell Disease (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, a blood and bone marrow transplant was the only curative option. Research conducted by NHLBI and the National Human Genome Research Institute played a critical role in developing 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 ASH shares your goal of recommitting the NIH to its mission to fund the most innovative biomedical research agenda. The <u>ASH Agenda for Hematology Research</u> highlights key emerging and transformative areas of research that can launch the field into the next generation of therapies for hematologic conditions.¹ We urge you to use our research agenda to advance these emerging areas in hematology that can increase the quality of life for the millions of patients who suffer from chronic illness.

Additionally, ASH implores you to protect and enhance the NIH's role and the world's leader in biomedical innovation. 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 you examine NIH's existing programs and strive to spend federal dollars more efficiently, ASH encourages you to partner with stakeholders to gain a full picture of how NIH's programs are working, or in some cases, not working to meet the agency's mission. Finally, any changes in the agency's structure should be undertaken with the goal of optimizing federal investment to enhance health, lengthen life, and reduce illness and disability. We urge you to ensure that the essential functions of the agency—investing in basic and clinical research and training the next generation of researchers across the existing institutes and center's areas of study—are preserved. Furthermore, the results and impacts on human health of funded research must be clearly communicated to the public so that they understand how continued investment in biomedical research is improving and extending American lives.

Promoting the Next Generation of the Research Workforce including Hematologists

ASH remains concerned about the future of hematology researchers, and particularly at risk is the physician-scientist workforce. The NIH has traditionally provided strong support for early investigators through its policy around Early Stage Investigators (ESI). This policy is critical to the Federal Government's role in supporting the entry of new investigators into the biomedical research pathway and for junior investigators who are trying to establish and sustain independent research careers. ASH supports prioritizing ESI applications, as well as other funding mechanisms that support junior

¹ https://www.hematology.org/research/ash-agenda-for-hematology-research

investigators in the research workforce including the Clinical and Translational Science Awards program (CTSA), and the T32 grant program.

The T32 program, for example, has allowed research fellows to stay on at institutions and gain the necessary experience to succeed in research careers. Mentorship is a critical component of these programs and given current research workforce shortages, it is important to be flexible and efficient in grant mechanisms like the T32, by allowing consortiums of sites or states to create collective T32 programs. A program created to allow for such a consortium is the <u>U2C-TL1</u> program (Institutional Network Award for Promoting Kidney, Urologic, & Hematologic Research Training award) funded by NIDDK. Programs like this will help support the next generation of researchers who rely on mentoring to become independent researchers.

Additionally, NIH should increase early-stage and translational funding to bridge innovation and stabilize research careers. Collaborative grants with established researchers could further support junior researchers by facilitating their transition to faculty roles. The Society looks forward to discussing these ideas and others as we believe the NIH must have policies and programs in place that support research as a viable career option.

Increasing Expertise in the Peer Review Process

NIH's research must be informed by stakeholders with scientific expertise and as such, ASH recommends that all principal investigators who receive NIH Funding participate in the peer review process. Not only will this increase the number of investigators needed to assist with peer review, it will also increase the expertise needed to evaluate research proposals in all areas of research, including hematology. Given that hematology research is spread among several institutes, centralizing the peer review process may be beneficial. Currently, most hematology RO1s are reviewed by the Center for Scientific Review while T32s, K awards, and clinical trials are reviewed by individual institutes. It is our understanding that all reviews for all grant programs will be centralized to help streamline the process. Encouraging and enticing researchers at all levels in their career to contribute to the grant review process is essential. In order for this to be successful for hematology research, hematology expertise as well as patients impacted by the disease must be at the table; special emphasis panels should be convened for certain reviews to ensure reviewers would have the necessary expertise. This is particularly important for rare conditions, of which there are many in hematology. ASH recommends that CSR develop a mechanism to bring this type of specialized expertise to the review process when appropriate and would welcome the opportunity to discuss instances when this extra step is needed and methods to operationalize it.

In closing, ASH recognizes that NIH's current programs and structure could be optimized to make the best use of the federal investment and welcomes the opportunity to work with you to be sure that NIH continues to support the next generation of researchers and a pipeline for innovative therapies that can be accessible for all patients. Please use ASH Chief Policy Officer, Suzanne Leous (sleous@hematology.org) or Director of Government Relations and Public Health, Stephanie Kaplan (skaplan@hematology.org) as your points of contact to arrange a meeting with the ASH leadership.

Sincerely, Yourda S. avalos, MAD

Belinda Avalos, MD

President