



AGENDA FOR HEMATOLOGY RESEARCH

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Over the past 60 years, American biomedical research has led the world in probing the nature of human disease. The fruits of this research have 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, other federal agencies, and research foundations. The field of hematology has made significant contributions to human health. With the advances gained through an increasingly sophisticated understanding of how the blood system functions, hematologists (both scientists and clinicians) have changed the face of medicine through their dedication to improving the lives of patients around the world.

Funding for hematology research is an investment in the nation's health. Research funding must increase to allow the major advances in understanding the molecular defects behind a hematologic disease to be translated into novel diagnostics and targeted therapeutics. Given the broad benefits that will be derived from this research, federal agencies should coordinate their hematology funding in order to produce the greatest impact on specific high-need areas. A multi-agency approach would deliver advances faster, more economically, and more efficiently.

Support for research in the areas listed below, co-sponsored by multiple agencies, will be important for future progress:

- Stem Cells and Regenerative Medicine: Turn iPS cells into cures for human diseases
- Myelodysplastic Syndrome and Acute Myeloid Leukemia: Find an effective and personalized treatment for the elderly
- Hematopoietic Stem Cell Transplantation: Increase success rates by improving management of graft-versus-host disease
- Sickle Cell Disease: Reduce the barriers to care, burden of pain, end-organ injury, and premature death
- Deep-Vein Thrombosis and Venous Thromboembolism: Understand the risk factors and develop targeted therapies
- Childhood Leukemia: Improve cure rates by performing coordinated research that includes discovery and preclinical and clinical testing of novel targeted therapies
- Translating Laboratory Advances into the Clinic: Use novel genomic technologies to improve treatment of hematologic diseases





Breakthrough therapies for blood disorders benefit those patients who suffer from leukemia, lymphoma, multiple myeloma, thalassemia, hemophilia, and sickle cell disease. As a result, children are routinely cured of acute lymphocytic leukemia (ALL); more than 90 percent of acute promyelocytic leukemia (APL) is cured with a drug derived from vitamin A; previously lethal chronic myeloid leukemia (CML) is effectively treated with well-tolerated pills; and multiple myeloma is yielded to entirely 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 inherited metabolic disorders, and 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.

The era of personalized medicine has arrived. 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. Translating these new discoveries and technologies into personalized patient care offers the possibility of better survival, less toxicity, disease prevention, improved quality of life, and lower health-care costs. However, many patients still lack effective therapy for malignant and non-malignant hematologic diseases.

Support for the field of hematology should be among the top priorities in health care because it will have a dramatic impact on the future of health in America and around the world in all areas of medicine.