CDMRP

Department of Defense

Bone Marrow Failure Research Program





U.S. Army Medical Research and Materiel Command

Congressionally Directed Medical Research Programs

HISTORY:

The Congressionally Directed Medical Research Programs (CDMRP) represents a unique partnership among the public, Congress, and the military. This partnership has grown to encompass multiple targeted research programs. The CDMRP seeks to fill research funding gaps by focusing on high-risk, high-gain research with an emphasis on innovative ideas, breakthrough technologies, and novel partnerships. Since 1993, funds for the CDMRP have been added by Congress to the Department of Defense budget annually to provide support for targeted research programs focused on a wide variety of diseases, conditions and injuries.

"Basic research is continuing to provide clues as to the extent and complexity of genetic alterations and the Bone Marrow Failure Research Program (BMFRP) can continue to help shape and direct the future of bone marrow failure research. The Integration Panel, charged with setting the vision and award mechanisms of the BMFRP, is agile and responsive to the changing needs of the field, ensuring that the focus on funding within the BMFRP is adaptive and able to meet the evolving needs of this important area of research. I have had the good fortune of serving with an exceptional group of individuals, encompassing a wide spectrum of expertise in bone marrow failure research. The members of the panel are experienced, intelligent, collegial, and truly united in their commitment to move the field of bone marrow failure research forward."

Lisa Minter, Ph.D.
University of Massachusetts
Integration Panel Chair

PROGRAM MANAGEMENT PROCESS:

Under the auspices of the U.S. Army Medical Research and Materiel Command (USAMRMC), CDMRP employs a flexible management process to maintain the individuality of each program while also meeting the needs of Congress, the Department of Defense, the research and advocacy communities, and the public at large. The CDMRP manages programs from receipt of funds, through competitive selection of proposals and individual project performance, to award closeout. The application review includes the two-tier review process for proposal evaluation recommended by the National Academy of Sciences' Institute of Medicine. Each tier of the review process is conducted by members of panels composed of scientists and clinicians (subject matter experts) and consumer advocates (persons affected by a specific disease, condition or injury) who provide a unique perspective and a sense of urgency to program processes. The first tier of application review is a scientific peer review against established criteria for determining scientific merit. The second tier of application review is programmatic review conducted by members of an Integration Panel who compare applications to make funding recommendations based on programmatic priorities and published criteria. The Commanding General of USAMRMC issues the final approval for funding.

Bone Marrow Failure Research Program

Bone marrow failure is a general term covering many diseases of the sponge-like tissue found inside bones. Disorders affecting cells of the bone marrow are generally rare, potentially life-threatening diseases in which the bone marrow stops functioning or produces abnormal blood cells. These diseases are classified into two major categories: acquired bone marrow failure and inherited bone marrow failure. Acquired bone marrow failure may be caused by a variety of factors, including exposure to certain chemicals, environmental toxins, and viruses, or by autoimmune responses. Inherited forms of bone marrow failure arise from specific alterations or abnormalities of genes passed on from parent to child. Current treatment options for bone marrow failure disorders include drug therapy and hematopoietic stem cell transplant.

The mission of the Bone Marrow Failure Research Program is to **encourage and support innovative research** that is committed to advancing the understanding of inherited and acquired bone marrow failure diseases, thereby improving the health of affected individuals, **with the ultimate goals of prevention and cure.** Through offering a variety of award mechanisms, the program funds a broad research portfolio of innovative basic, translational, and preclinical studies.

FY09 Exploration–Hypothesis Development Award: Supports the initial exploration of innovative, untested, novel, and potentially groundbreaking concepts

- **Molecular Mechanism of Bone Marrow Failure Associated with TINF2 Mutations**
  Alison Bertuch, M.D., Ph.D.; *Baylor College of Medicine*
- **In Vivo Imaging of Bone Marrow Regulatory T Cells and Their Role in the Prevention of Bone Marrow Failure**–Charles Lin, Ph.D.; *Massachusetts General Hospital*
- **Complementation of Myelodysplastic Syndrome Clones with Lentivirus Expression Libraries**–Daniel Lindner, M.D., Ph.D.; *Cleveland Clinic Foundation*
- **Redox Regulation in Bone Marrow Failure**–Shi Pan, Ph.D.; *University of Rochester*
- **Study of a Novel Genetic Regulator of Stress Hematopoiesis**–Archibald Perkins, M.D., Ph.D.; *University of Rochester*
- **Modelling the Epigenetic Changes Likely Underlying the Pathogenesis of Acquired Myelodysplastic Syndromes**–Linda Scott, Ph.D.; *University of Texas Health Science Center at San Antonio*
- **Induced Pluripotent Stem Cells and the Genetics of Aplastic Anemia**–Colin Sieff, M.B.B.Ch.; *Children's Hospital, Boston*
- **Epigenomic Analysis of Hematopoietic Progenitor and Stem Cells in Myelodysplasis**
  Amit Verma, M.B.B.S.; *Albert Einstein College of Medicine of Yeshiva University*

FY09 Idea Award: Supports innovative ideas and high-impact research approaches

- **Interferon-Gamma-Induced Proliferation and Premature Senescence Lead to Hematopoietic Stem Cell Dysfunction in Acquired Aplastic Anemia**–Margaret Goodell, Ph.D.; *Baylor College of Medicine*

FY09 Synergistic Idea Award: Supports innovative ideas through synergistic scientific collaborative partnerships

- **The Study of Bone Marrow Failure in Patient-Derived Induced Pluripotent Stem Cells (iPSCs)**
  Mitchell Weiss, M.D., Ph.D. and Monica Bessler, M.D., Ph.D.; *Children's Hospital, Philadelphia*
- **Correction of Human Fanconi Anemia-Induced Pluripotent Cells by Homologous Recombination**–Bruce Blazar, M.D.; University of Minnesota, Twin Cities and Jae Joung, M.D., Ph.D.; *Massachusetts General Hospital*

FY08 Investigator-Initiated Research Award: Supports important scietific contributions

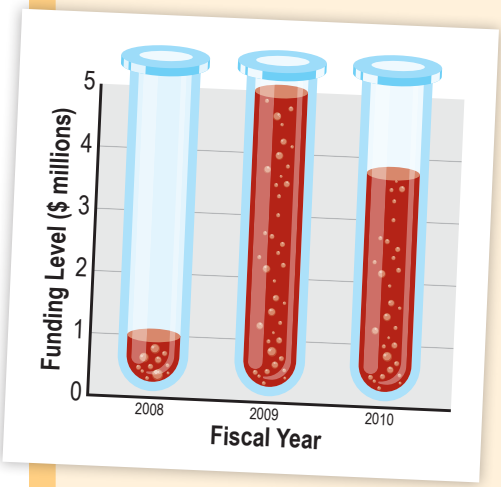
- **The Role of TAK1 in the Pathogenesis of Bone Marrow Failure Syndromes**
  Jiwang Zhang, M.D., Ph.D.; *Loyola University, Chicago*

"I am honored to participate in the BMFRP as a Consumer Reviewer and am very impressed by the quality of the proposals as well as the efficient and fair review process. The scientific community has many innovative and exciting ideas on the underlying causes of bone marrow failure, better ways to diagnose bone marrow failure, and better treatment options."

Cheryl Heisey
Leukemia and Lymphoma Society
Consumer Peer Reviewer

VISION

To understand and cure bone marrow failure disease



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