BONE MARROW FAILURE RESEARCH PROGRAM

CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

Mission: To encourage and support innovative research that is committed to advancing the understanding, prevention, and treatment of inherited and acquired bone marrow failure diseases, thereby improving the health of affected Service Members, Veterans, and the general public, with the ultimate goal of cure

FOCUS AREAS

- Understanding the causes and progression of bone marrow failure diseases
- Finding effective bone marrow failure treatments and cures

DISEASES AND DISORDERS

The program addresses acquired and inherited bone marrow failure diseases and syndromes, including, but not limited to:

Acquired:

- Aplastic anemia
- Myelodysplastic syndroms
- Paroxysmal nocturnal hemoglobinuria
- Pearson's disease
- Radition- or chemical-induced disease
- VEXAS syndrome

Inherited:

- Diamond-Blackfan anemia
- Dyskeratosis congenita/telomere biology disorders
- Fanconi anemia
- Germline mutations, e.g., GATA2, SAMD9/SAMD9L
- Inherited neutropenia
- Schwachman-Diamond syndrome

FY24 Funding Mechanisms

Idea Development Award

Supports innovative ideas and high-impact approaches based on scientifically sound evidence to move toward understanding and curing BMF diseases

Maximum direct costs: $530,000
Maximum period of performance: 3 years

Encourage hypothesis-driven correlative studies associated with clinical trials

Pre-Application Submission Deadline: July 17, 2024
Application Submission Deadline: October 9, 2024

Early Career Investigator Option
Independent investigators <10 years from first faculty appointment or equivalent

Established Investigator Option
Independent investigators ≥ 10 years from their first faculty appointment or equivalent

Investigator-Initiated Research Award Level 1

Supports studies that further develop mature ideas, expand upon key discoveries and have the potential to make significant advances in research and/or patient care

Maximum direct costs: $675,000 | Maximum period of performance: 3 years

Encourage multidisciplinary collaborations
Partnering PI Option
Clinical Trials are not allowed

Pre-Application Submission Deadline: July 17, 2024
Application Submission Deadline: October 9, 2024

Investigator-Initiated Research Award Level 2

Supports Investigational New Drug application-enabling efforts

Must name at least one but no more than three lead therapeutic candidates

*Must address the Program's Focus Area, **"Find effective BMF treatments and cures"***

Maximum direct costs: $850,000 | Maximum period of performance: 2 years

Partnering PI Option
Clinical Trials are not allowed

Pre-Application Submission Deadline: July 17, 2024
Application Submission Deadline: October 9, 2024

https://ebrap.org/eBRAP/public/ProgramFY.htm?programFYId=603107

For more information, visit: **https://cdmrp.health.mil/funding/bmfrp**

June 2024

Support of Early Career Investigators

BMFRP remains dedicated to growing the field of bone marrow failure research and providing opportunities for research training.

~30% of awards since FY08 supported:

New Investigators
new to the field

Postdoctoral Fellows
recent doctoral graduates

Early Career Investigators
<10 years from first faculty appointment

High-Impact Preclinical Research

Exogenous RvE1

Resolvin E1 is a small pro-resolving lipid mediator for severe aplastic anemia. Using mouse models of the disease, researchers identified that treatment with RvE1 improves the ability of macrophages to resolve inflammation and has therapeutic benefits to alleviate disease symptoms. Ongoing BMFRP-funded research will address the precise mechanisms of RvE1 protection.

Hematopoietic Stem and Progenitor Cell Immune Privilege Site

Researchers identified the stem cell niche, a special compartment of the bone marrow where hematopoietic stem and progenitor cells conduct this process of self-renewal, as an immune privilege site where regulatory T cells are immune-suppressive and shield hematopoietic stem and progenitor cells from immune rejection. This scientific advancement identified new opportunities to improve care for bone marrow diseases.

Products on the Clinical Horizon*

Metformin

A single-institution phase 2 pilot study to treat non-diabetic Fanconi Anemia patients. Researchers observed no disease progression or relapse in study participants, along with no serious adverse events. Additional studies will provide greater clarity regarding metformin's role, efficacy, and utility in the treatment of Fanconi Anemia.

H3B-8800

A multicenter phase 1 trial to evaluate the safety, pharmacokinetic, and pharmacodynamic properties in subjects with myelodysplastic syndromes, acute myeloid leukemia, and chronic myelomonocytic leukemia. Preliminary results present a favorable safety profile under prolonged dosing; enrollment continues. Other study findings are currently being analyzed; enrollment continues.

E7820

A phase 2 trial initiated to evaluate safety and efficacy in subjects with relapsed/refractory acute myeloid leukemia, myelodysplastic syndromes, or chronic myelomonocytic leukemia. Results are pending and the study is currently enrolling.

*The BMFRP funded pre-clinical research that contributed to the opening of the clinical trials. Funding of the clinical trials came from follow-on/outside sources.

Feedback from Consumer Reviewers

"Changing the outcome of those living with bone marrow failure is critical for thousands of patients and their families. The BMFRP provides an important element to those impacted by these rare conditions by furthering a collective understanding of BMF disorders through impactful research. With an eye toward the future, the ultimate goal of finding a cure for BMF can be realized for those in the military, as well as the general public."

Melanie Marquez, BMFRP Programmatic Review Panel Member 2022-2024

"I have a rare disease called Fanconia anemia. At 9 years old, I received my life-saving bone marrow transplant in 2006. Survival rates were only 50% then. Now, thanks to research, an unrelated transplant is over 90% successful. I am grateful for the research the BMFRP has done, is doing, and will continue to do. I witnessed their impact firsthand. I can help others and make a difference with my life thanks to research like this."

Matt Pearl, Fanconi Anemia Research Fund