BONE MARROW FAILURE RESEARCH PROGRAM (BMFRP)

CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

VISION: To understand and cure bone marrow failure diseases

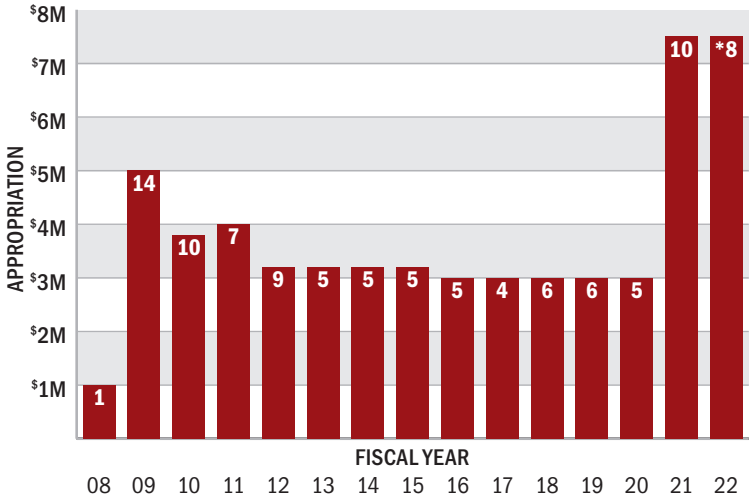
MISSION: To encourage and support innovative research that is committed to advancing the understanding and treatment of inherited and acquired bone marrow failure diseases, thereby improving the health of affected Service Members, Veterans, and the general public, with the ultimate goal of prevention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

FY23 Appropriation
$7.5M

BMFRP Background:

The BMFRP began in fiscal year 2008 (FY08) with an appropriation of $1 million (M). From FY08-FY21, the BMFRP has funded 92 awards through $49.05M in total appropriations. The figure below summarizes the annual appropriation breakdown of the BMFRP and associated funding history. The appropriation for FY22 is $7.5M, with an anticipated eight awards currently in negotiation.

FY08-FY22 BMFRP Appropriation and Number of Awards Funded



**Pending Negotiations*

Since FY08, the BMFRP has funded projects researching both acquired and congenital bone marrow failure (BMF) disease and syndromes. The program encourages applications designed to increase our understanding of all relevant bone marrow failure diseases and lead to eventual cures. Relevant disease and conditions include, but are not limited to:

- Aplastic Anemia
- Diamond-Blackfan Anemia
- Dyskeratosis Congenita/ Telomere Biology Disorders
- Fanconi Anemia
- GATA2 deficiency
- Induced BMF: Radiation/ Chemical
- Myelodysplastic Syndromes
- Paroxysmal Nocturnal Hemoglobinuria
- Pearson's Disease
- SAMD9/SAMD9L germline mutations
- Severe Congenital Neutropenia
- Schwachman-Diamond Syndrome

FY23 Funding Opportunity Summary

Idea Development Award (IDA)

- Early Career Investigator
- Established Investigator

To support innovative ideas and high-impact approaches based on scientifically sound evidence to move toward the BMFRP vision of understanding and curing BMF diseases.
Direct Cost Limit: **$530,000**
Max Performance Period: 3 years

Investigator-Initiated Research Award (IIRA) Funding Level 1

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

Direct Cost Limit: **$675,000**
Max Performance Period: 3 years

IIRA Funding Level 2

To support Investigational New Drug (IND)-enabling efforts that are empirical in nature, product-driven, and focused on the accumulation of data that will be used in support of an IND submission to the U.S. Food and Drug Administration.

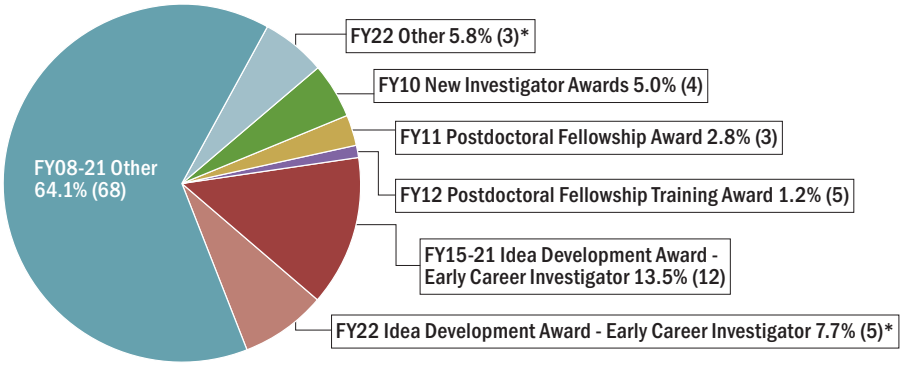
Direct Cost Limit: **$850,000**
Max Performance Period: 2 years

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

Support of Early Career Investigators

The BMFRP has a long-standing commitment to supporting Early Career Investigators (ECIs) who conduct research into bone marrow failure diseases. Twenty-four of the programs' 92 awards were to projects with New Investigators, Postdoctoral Fellows, and ECIs as the Principal Investigator (figure below). These awards represent 26% of the program's total research investment to date. For FY22, an additional five of the eight total awards recommended for funding were to ECIs, potentially increasing our total investment to over 30%. To ensure continued support, the BMFRP Idea Development Award currently offers an ECI Option that restricts eligibility to independent investigators at the level of Assistant Professor (or equivalent) within 10 years of their first faculty appointment.

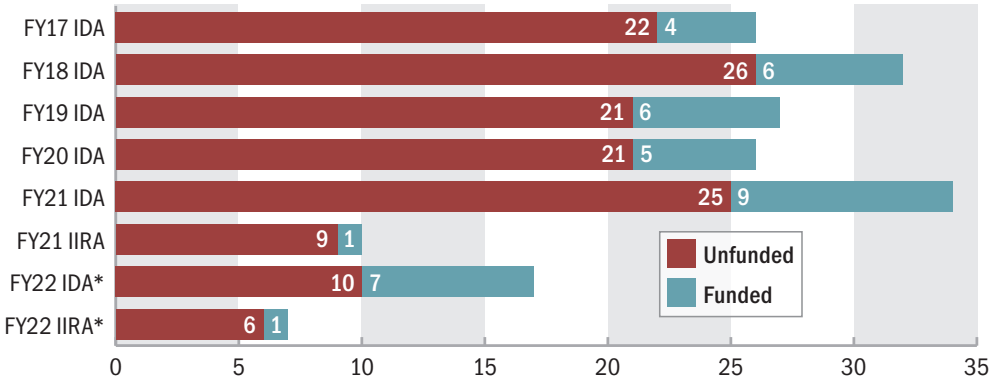
FY08-FY22* Percent Investment in New/Early Career Investigators (# of awards)



Success Rates for Applicants

Throughout its history, the BMFRP has offered several funding opportunities. The program has received 601 distinct research projects, of which 98 (including two partnering awards) were recommended for funding, representing an overall funding rate of 16.3%. As shown in the figure below, over the past six years, the primary funding vehicles of the program have been the Idea Development Award (IDA) and Investigator-Initiated Research Award (IIRA), with a combined applicant success rate of 21.8%*

Full Application Receipt for FY17-FY22* BMFRP Funding Opportunities and Percent Funded



BMFRP Outcomes

Funded investigators of the BMFRP have had a significant impact on the field of bone marrow failure research as evidenced by the number of highly cited publications, invited presentations, newly developed intellectual property, and research that has led directly to clinical trials. The program seeks to support the research accomplishments of existing and future investigators as we move toward our shared goal of improved disease understanding, treatments, and cures.

Outcomes	FY08-FY21
Awards	92
Publications	146
Total Publication Citations	8,994
Patent Applications	8
Presentations/Speaking Engagements	148
Clinical Trails Based on BMFRP Findings	5

**Pending Negotiations*

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.

https://clinicaltrials.gov/ct2/show/NCT03398824

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. Other study findings are currently being analyzed; enrollment continues.

https://clinicaltrials.gov/ct2/show/NCT02841540

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.

https://clinicaltrials.gov/ct2/show/NCT05024994

***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.*