



2020 AAMDSIF RESEARCH PROPOSAL APPLICATION GUIDELINES

Mission Statement:

AAMDSIF supports, educates and connects patients, caregivers and health professionals worldwide on bone marrow failure diseases. It promotes and invests in collaborative clinical research to accelerate the discovery of better treatments and cures for aplastic anemia, MDS, PNH and related bone marrow failure diseases.





Summary

The Aplastic Anemia and MDS International Foundation, Inc. (AAMDSIF) is pleased to help researchers dedicated to the study of bone marrow disorders, advance the understanding, prevention and treatment of the following bone marrow disorders:

- Aplastic Anemia (focus on acquired)
- Myelodysplastic Syndromes (MDS)
 - MDS transition to Acute Myeloid Leukemia (AML)
- Overlap between Myelodysplastic Syndromes and Myeloproliferative Neoplasms (MDS/MPN)
 - Includes Chronic Myelomonocytic Leukemia (CMML)
- Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Pure Red Cell Aplasia (PRCA)

Grants in the amount of \$60,000 over two years (30K each year) will be awarded to eligible national and international researchers, for qualified projects.

Background

For nearly thirty years, the AAMDSIF has provided financial support to research that leads to new insights into causes of bone marrow disorder diseases and to the development of new therapeutic approaches. The 2019 program will accept applications in any of these areas -

- Basic research
- Translational research
- Pre-clinical research
- Early stage clinical research
- Health services research

Applicant eligibility

To be eligible to apply for a research grant, an applicant must hold a Doctor of Medicine, Doctor of Philosophy, Doctor of Osteopathy, Doctor of Science, or other doctoral level degree (e.g., PharmD). There are no nationality restrictions. Research may be carried out in the United States or abroad but not at a for-profit laboratory in the private sector. The project must represent independent research conducted by the applicant. Members of the AAMDSIF Scientific and Medical Advisory Board are not eligible to apply for a grant, and may sponsor no more than 1 applicant during any given round.

*Preference will be given to **New Investigators** who propose a project that will contribute toward their professional development in bone marrow failure disease research.*

New Investigators are post-doctoral fellows, instructors, and those who have been assistant professors for fewer than five (5) years.

Project eligibility

The Foundation is interested in funding research across the spectrum of basic and clinical science, but highest priority is for studies that have the potential to impact prevention, diagnosis, risk-stratification, or treatment of these diseases **within three years**. Examples of this type of research include:

- Discovery of biomarkers predicting response or resistance to existing therapies.
- Correlative science on patient samples collected from clinical trials, to inform understanding of therapeutic mechanisms.
- Late-stage pre-clinical research, with a goal of establishing proof-of-concept for a novel therapy
- Basic research that reveals new mechanisms of disease likely to benefit patients

Investigators should be prepared to provide a rationale for the research as related to a gap in evidence for the proposed project. Applicants should clearly explain why the proposed research will address an important question that has not been answered by previous research.

Review Process

An NIH-model peer-review process is used to evaluate applications. Reviewers include members of the AAMDSIF Scientific Medical Advisory Board as well as ad hoc reviewers. It is the AAMDSIF policy not to reveal the names of the individuals who have reviewed the specific application.

Awards are based on the scientific quality of the research plan, the relevance of the proposed research to the AAMDSIF's goals, the applicant's qualifications, and the quality of the research institution and facility where the research will be conducted. The quality and support of the research mentor also will be considered.

In addition, the following criteria will be considered by reviewers:

- The likelihood that the goals of the proposal are attainable with the funding from AAMDSIF;
- The project is independent research and not part of a large, multi-funded study;
- The translational nature of the research and ***whether the findings can be rapidly applied to benefit patients in the near term (e.g. within 3 years)***;
- The likelihood that the project will contribute toward the professional development of the researcher.



APPLICATION INSTRUCTIONS

Important Dates

Letters of Intent Open: April 1, 2020
Letters of Intent Due: May 29, 2020
Applications Due: September 30, 2020
Notification of Award: December 1, 2020
Begins: January 1, 2021

Letters of Intent (LOI)

Letters of intent (LOI) will be accepted from potential applicants. The LOI deadline is Friday, May 29, 2020 and must be submitted via AAMDSIF's online grant portal housed on the organization's website.

The LOI should be no more than 3 pages, 10-12 pt. font and 1.5 line spacing. A comprehensive LOI should contain:

- Introduction
- Evidence gap (Statement of Need)
- Project Description/Methodology
- Description of Research Site and Qualifications
- Mentor Qualifications
- Budget (based on 2 year award)
- Summary/Conclusion

Application

Select applicants will be invited to submit full proposals, based on review of the letters of intent. Applications will be due September 30, 2020, and must be submitted via AAMDSIF's online grant portal housed on the organization's website.

- Title page
- Applicant/PI information
- Institution and Contacts
- Abstract
- Budget Summary
- Organizational Assurances
- Sponsorship (Mentor) Information
- Proposal Attachments (Project description and methodology)
- Three letters of recommendation
- Signature Pages

Use of Funds

Successful applicants can be funded up to \$60,000 for two years (\$30,000 each year, with second-year funding pending approved progress in the first year). Grant funds may be used only to support the direct costs of research and indirect costs of up to 10% of the grant. Grant funds may not be used for purchase of permanent equipment (defined as equipment expected to last at least three years and computer hardware) or for repair or service contract costs for institutional equipment; the construction or renovation of facilities; furniture; salaries of collaborators at other institutions; honoraria; membership dues; subscriptions; books; or journals.

Allowable use of grant funds includes salary for the principal investigator and for scientific personnel directly engaged in the research project at the principal investigator's institution; consumable supplies, disposables, and animals necessary to fulfill the project's specific aims; and travel necessary to conduct research or to present the results of the research at appropriate scientific or medical meetings sponsored by non-profit associations. The maximum amount of funds expendable for travel is \$2,000 per year. Travel funded by this grant must be completed during the funding period.

Grantees are required to present a poster of their work during the AAMDSIF Scientific Symposium that occurs during the two year period of the grant. These symposia occur biannually, and are attended by leading researchers in the field of hematology/oncology. For non-US based investigators, a separate travel stipend may be available upon request.

Support from Other Sources

An applicant may not apply for, use, or accept AAMDSIF funds for a research project or part of a project already supported by the AAMDSIF. Accordingly, full disclosure of all available and pending funds for research support must be made in the budget included with the grant application. If funds from other sources become available to the

applicant during the review or tenure of an AAMDSIF research grant, the principal investigator must inform the AAMDSIF in writing. The AAMDSIF and its advisors will then make a decision about the continuation of the research award.

Grant Payments

Checks are made payable to the grantee's institution and are issued quarterly. The institution's financial officer should establish an account from which research expenses may be paid under the terms of the approved award. **No funds will be released before receipt by the AAMDSIF of a signed copy of the Notice of Award.** Additionally, the AAMDSIF reserves the right to withhold payments for failure to meet the Foundation's reporting requirements outlined in this document and the Notice of Award.

Expenditures beyond the Grant Period

Expenditures may not be made against a grant after its expiration date except with prior authorization in writing from the AAMDSIF.

At the termination of the grant unexpended funds may, under exceptional circumstances, be used for an approved period of time. The grantee must request in writing such an extension of the use of grant funds. The request must state the amount of unexpended funds and how those funds will be used during the extension period. In addition, the grantee must provide a detailed justification satisfactory to the AAMDSIF and its Scientific Medical Advisory Board. Such a request must be made no later than two (2) weeks after the termination date of the award.

Change in Status

The continued use of grant funds following any change in the status of the principal investigator requires approval in writing by the AAMDSIF. Such changes include, but are not limited to, a move by the investigator to a new institution, the institution's withdrawal from the project, the investigator's termination from the institution, or an absence of the grantee longer than six weeks. Because the grant is awarded to the investigator and not to the institution, upon approval by the AAMDSIF, grant funds remain with the principal investigator.

Should the Foundation not approve the use of funds following the change of status, a financial report and progress report must be sent via electronic mail four weeks following the Foundation's decision. The progress report must justify the spending to date and will be reviewed by the Scientific Medical Advisory Board. All funds not spent before the investigator's change in status must be returned within six weeks following the Foundation's decision not to approve the use of funds following the change of status. Funds that are found by the Scientific Medical Advisory Board to be spent without justification must also be returned.

Cancellation of Grant

If, for any reason the grantee must relinquish the award, s/he must notify the AAMDSIF in writing within two weeks of the decision to relinquish the award. The AAMDSIF reserves the right to discontinue a grant if circumstances render the grantee unqualified and/or unable to perform under the terms and conditions of this policy and the Notice of Award. Such circumstances include, but are not limited to, loss of license, conviction of a crime in relation to research or clinical activities, or withdrawal of insurance. Failure to submit a progress report by the specified deadline will result in withdrawal of funding.

Reports

A financial report form will be mailed to the grantee's financial officer with a copy of the award letter. The completed form must be returned to the AAMDSIF within twelve (12) weeks after the termination date of the award.

A detailed mid-project progress report must be submitted to AAMDSIF by November 30, 2021; the report should explain how the research results have to date met the objectives established in the application. The report must be reviewed and approved by the AAMDSIF's Scientific Medical Advisory Board in order to receive the second year of funding. In addition, a one-page summary of the above information in lay language intended for patients must be submitted at the same time. The Foundation may use the summary in its website, newsletters, annual reports, social media venues and other material.

A final report is due to the AAMDSIF six weeks after the close of the award period (December 31 of the second year). The report will include an outline as well as a detailed discussion of the work performed, results (particularly as they compare to the objectives established in the application), and future plans for related research. Reprints of any publication written by the grant recipient and relating to the work performed during the award period must be included. Additionally, a one-page summary of the above information in lay language intended for patients must be submitted. The Foundation may use the summary in its website, newsletters, annual reports, and other material.

Publications and Oral Presentations

All manuscripts on findings produced with assistance from the research grant must be submitted to the AAMDSIF within one month of publication.

Recipients must cite support from the AAMDSIF in all published work and in all oral presentations that relate to findings produced with assistance from the research grant.

For a period of 10 years post-award, the grantee is responsible for forwarding to AAMDSIF, a copy of published material directly resulting from the funded work. AAMDSIF will perform a yearly follow-up with all grantees to establish a record of funded publications and additional funding awarded since receipt of AAMDSIF grant.

Grantees are strongly encouraged to publish material on open source venues when possible, to allow patients the greatest access possible to findings funded through their donations.

Human and/or Animal Subjects/Tissues

When human subjects or tissues are to be used in a research project, it is the responsibility of the grantee and the institution to ensure that the institution has on file:

- A complete copy of the research protocol approved by the Institutional Review Board with oversight on human subjects and a copy of that Board's current approval notice (or, for research conducted outside of the United States, the equivalent of an IRB), and
- A copy of the informed consent form(s) to be used by patients.

When animals and/or animal tissues are used, it is the responsibility of the grantee and the institution to ensure that the institution has a complete copy of the institution's animal care and use committee approval on file.

A copy of these documents must also be submitted with the application or delivered upon notice of award and before grant funds are transmitted.

AAMDSIF Policy on the Use of Animals in Research

All entities that receive funding from the AAMDSIF adhere to the following principles:

- Animals shall be used in biomedical research only when no other means of obtaining scientifically sound, valid, and useful results are available.
- The minimum number of appropriate animals required to obtain and to validate results shall be used.
- The acquisition, care, and use of animals must be in accordance with all applicable federal, state, and local laws and regulations, whether the research is conducted in the United States or abroad.
- Certifications must be received from research facilities prior to being approved for a research fellowship that the facility(ies), its researchers, and its employees adhere to the Animal Welfare Act, National Research Council Guide for the Care and Use of Laboratory Animals, and any appropriate U.S. Department of Agriculture and/or National Institutes of Health regulations and standards, whether the research is conducted in the United States or abroad.
- In research requiring the death of an animal, only the most appropriate and humane form of euthanasia shall be used consistent with the purpose of the research.

Conflict of Interest

Any real or potential conflict of interest on the part of the grantee or any collaborators/mentors in relation to the project must be revealed. It is expected that AAMDSIF grantees will observe the highest ethical standards while conducting research.

TERMS OF THIS POLICY ARE SUBJECT TO REVISION OR ALTERATION AT ANY TIME.

Inquiries

Contact Alice Houk, Senior Director of Health Professional Programs at hok@aamds.org or (301) 279-7202 Ext. 101.



A highly-respected patient-focused organization, AAMDSIF's mission is to serve its community in every cycle of these diseases. That includes providing the vital information and resources needed to adjust to the initial life-altering phase of diagnosis and get through the potentially life-threatening treatment phase that follows. We then help patients and families manage the life-long stage of having a chronic disease.

The Foundation also does substantive work with health professionals, including being administrator of the MDS Clinical Research Consortium - a unique collaboration of six major academic medical centers designed to maximize the results of MDS clinical trials, to the benefit of patients everywhere. AAMDSIF also organizes a two-day biennial event - The International Scientific Symposium for Bone Marrow Failure Disease. Sponsored by the National Institutes of Health, the meetings bring together clinical researchers and medical doctors who share and compare their knowledge of bone marrow failure. This scientific exchange helps to advance new theories and encourages collaboration in the search for the cures.