



2024 Grant Application Guidelines for RARE Grant Program – Research Accelerating RUNX1 Exploration

Application Deadline: December 4, 2023 (8:00 PM ET)



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About the *RUNX1* Research Program (RRP) and Alex's Lemonade Stand Foundation (ALSF)

About RRP

A research and advocacy venture with a mission to improve the quality of life and prevent cancer in patients with *RUNX1*-FPD, a heritable blood cancer syndrome. RRP works to promote awareness and fund world-class collaborative research with an empowered patient community.

About Alex's Lemonade Stand Foundation

A non-profit changing the lives of children with cancer by funding impactful research, raising awareness, supporting families, and empowering everyone to help cure childhood cancer.

RARE Grant Program – Research Accelerating *RUNX1* Exploration

The *RUNX1* Research Program and Alex's Lemonade Stand Foundation are delighted to continue their partnership this year and are excited to launch the Research Accelerating *RUNX1* Exploration (RARE) Grant.

Key Objective

Purpose of the grant is to fund research that will accelerate the discovery of druggable oncogenic pathways and deliver cancer interception and cancer prevention therapeutics for *RUNX1*-FPD children and family members.

Program Description

The Research Accelerating *RUNX1* Exploration Grant is a two-year award designed to fund research that will lead to the development of therapies for patients with *RUNX1*-FPD that will either intercept the transition from a precancer state (clonal hematopoiesis) to MDS/AML or prevention of cancer (before clonal hematopoiesis).

The *RUNX1* Research Program hosts an annual scientific meeting that brings together grant recipients and other scientists. Grant recipients are expected to present their progress as part of the annual review.

A Research Accelerating *RUNX1* Exploration Grant is **\$250,000 over 2 years** (maximum \$125,000 per year may be requested).

Application Timeline and Review

- A full proposal must be submitted that meets all guideline criteria as well as all eligibility criteria; otherwise, applications will be administratively rejected.
- Proposals will be reviewed by an independent panel of experts according to the NIH recognized peer-review process.
- Selection criteria include the following:
 - Training/Education record
 - Publication record
 - Grant awards and research awards/recognition

- Quality and overall ranking of research proposal based on the NIH 9-point scoring system
- Evidence of collaboration within project proposal - An important sub-goal is to stimulate research progress through collaborations between research groups
- Alignment to overall goal of preventing hematologic malignancies in *RUNX1*-FPD and the feasibility of translating findings into the clinic

APPLICATION TIMELINE

PROPOSALS DUE	December 4, 2023, 8 PM (ET)
AWARD NOTIFICATION	February 2024
PROJECTED START/END DATE	April 2024-April 2026

Applicant Eligibility: must be met at the time of application

- Applicant institutions may be based in the U.S. or outside of the U.S. Applicants need not be United States citizens. Funds must be granted to non-profit institutions or organizations.
- Applicants must have an MD, PhD, or MD/PhD (DO, MBBS or equivalent).
- Applicants *may* have research grants from other funding sources during the award period, but there must be clear documentation of mechanisms to avoid scientific and budgetary overlap.
- Applicants must have research experience working in, and a deep understanding of, normal or malignant hematopoiesis and/or immunology.

Scope of Proposal

- Aims of research proposals must be relevant to the goal of preventing hematologic malignancies in *RUNX1*-FPD. Proposals that seek to translate from bench to bedside will receive priority.
- The following areas of research are of priority and are **not** ranked according to their order:

Drug Repurposing and Clinical Translation

- Support for preclinical experiments and correlative clinical studies designed to test regulatory-approved agents for the rescue of *RUNX1*-FPD hematopoietic function and interception of high-risk clonal hematopoiesis.

RUNX1 Biology

- Characterize the normal function of *RUNX1* and the function of germline *RUNX1* mutant proteins. High interest in defining the role of *RUNX1* in DNA damage repair in HSCs and the potential role germline *RUNX1* mutations may play in acquisition of somatic mutations.
- Identify regulators of *RUNX1* expression and function to discover novel methods of correcting *RUNX1* function within the hematopoietic system.

Discovery

- Investigate feasibility of targeting RNA splicing for disease therapy in patients with splice-site mutations or nonsense suppression therapies for patients with nonsense mutations.

- Develop therapeutic strategies to raise normal RUNX1 activity or RUNX1 protein levels to improve hematopoietic function and reduce HM risk.
- Identify druggable mechanisms, with a focus on drug repurposing, that drive clonal hematopoiesis, clonal expansion and ultimately malignant transformation in RUNX1-FPD patients.
 1. Projects should focus on targeting clones with common co-occurring mutations in RUNX1-FPD (i.e. BCOR, TET2, PHF6, SRSF2).

Budget

The requested budget should be in proportion to the scope of the proposed project and should be at or under \$250,000 USD in direct costs over 2 years. A maximum of \$125,000 in total costs may be requested per year.

Restrictions:

- RRP/ALSF adheres to the NIH salary cap for principal investigator(s).
- Indirect costs are not allowed.
- The grant may not be renewed; one no-cost extension request is allowed.
- Other budget items may include fringe, travel, supplies and small pieces of equipment. RRP/ALSF funds cannot be used for tuition remission. View ALSF's complete [budget policy](#).
- If utilizing a subcontract or subaward, you must include this expense in the budget.
 - No indirect costs will be paid.
 - The PI's institution is responsible for disbursing funds for subawards and/or subcontracts

Resource and Data Sharing

Grant recipients are expected to share unique resources developed under this funding award, and as part of the application, applicants are required to submit a Resource Sharing Plan using [ALSF's Resource Sharing Form](#). Applicants should describe their track record of generating resources that are broadly re-used, the specific resources that will be generated in this proposed project, and the mechanisms by which those resources will be shared. To demonstrate a commitment to sharing that will be actualized, applicants should provide information in their sharing plan that clearly states the type of resource that will be shared, the method, characterization and timing of such sharing, and the anticipated resources (budget, personnel, etc.) required by the applicant and the resource user. Reviewers will consider the extent to which the dissemination of resources produced under the award will enhance or diminish the impact of the proposed work. For a full description and an example, see [the Resource Sharing Form](#). For the full Resource Sharing Policy visit our [Resources for Grant Applicants](#) page.

Restrictions

- **The RARE Grant is not transferable to another investigator.**
- RRP/ALSF does not fund proposals for research utilizing human embryonic stem cells or non-human primates. Research with human induced pluripotent stem cells is permissible.

Grant Policies & Reporting Requirements

- Please review the Grant Policies, including Budget Expenditures, Resource Sharing, found on the [Resources for Grant Applicants](#) page.
- Six month Interim and Annual Progress reports are required. Continued funding is contingent upon demonstration of satisfactory progress toward the completion of proposed research objectives and appropriate budget expenditures.
- Minor carry-over of funds (25% or less) is permitted each year with justification. Each year's budget will be approved subsequent to the review of project progress and milestones. RRP and ALSF may elect to partially fund, or to hold new funds, if a carry-over is excessive.
- A Final Report is required at the conclusion of funding. Report must state findings, expenditures, as well as publications and presentations which acknowledge RRP and ALSF funding. **The grant may not be renewed**; one no-cost extension may be requested in the Final Report.
- Publications, presentations, and posters featuring results of the experiments funded by this grant mechanism should acknowledge "The RUNX1 Research Program and Alex's Lemonade Stand Foundation (Grant #)". ALSF requests copies be sent via email to Grants@AlexsLemonade.org and RRP requests copies be sent to KEricson@Runx1-FPD.org.
- Investigators must present project progress at the annual RUNX1 Research Program scientific meeting.

Application Package Instructions

- All sections described below should be combined into one PDF (max 20 MB)
- All templates mentioned can be found at ALSF's [Resources for Grant Applicants](#) page.

Format Instructions

- PAGE HEADER: All pages of the application should be numbered; the name of the principal investigator should appear in the upper right-hand corner of each page.
- FORMAT: Follow NIH format guidelines: Arial, Helvetica, Palatino Linotype, or Georgia fonts with a font size of 11 points or larger with a minimum of ½ inch margins.
- ORDER & LENGTH: The order of the application should be followed, adhering to the maximum number of pages allowed for each subsection indicated in parentheses.

Section Descriptions

1. Project Information

- a. **Cover Page (1 page):** Download and complete the [Cover Page Template](#).
- b. **Table of Contents (1 page):** Provide a Table of Contents with page numbers to the corresponding sections.
- c. **Scientific Abstract (0.5 page):** Summarize the research objectives and rationale.

d. **Impact Statement (0.5 page):** How will this project impact patients, particularly children, with *RUNX1*-FPD

2. Budget/Justification (3 pages):

a. **Budget Template (1.5 pages):** Complete the [ALSF budget template](#). The signature from an institutional representative on the cover page of this grant application specifically acknowledges and accepts this budget and acknowledges that no indirect costs will be paid.

- i. The award amount is \$250,000 over 2 years.
- ii. ALSF and RRP adhere to the NIH salary cap for Principal Investigator(s)/Co-Investigator(s).
- iii. Indirect costs are not allowed.
- iv. If utilizing a sub-contractor, you must include their budget. No indirect costs will be paid to the sub-contractor.
- v. Reasonable travel expenses to national/international research meetings to disseminate findings may be budgeted.

b. **Budget Justification (1.5 pages):** Include a narrative for the following. Use N/A in sections as needed:

- I. Personnel
- II. Subcontractors/Subawards/Consultants
- III. Equipment (for equipment costs above \$5000)
- IV. Travel
- V. Computer and Software
- VI. Other

3. Biographical Sketch(es): Use the [NIH five-page biographical sketch](#) (SF424) format for the principal investigator and all key personnel.

4. Research Plan

- a. **Specific Aims (1 page):** List the goals, long-term objectives and what the specific research proposed in this application is intended to accomplish. State the hypothesis to be tested and relevance to *RUNX1*-FPD research and the stated objectives of this grant program.
- b. **Significance (0.5 page):** Describe the relevant background for the current research plan. State the significance and importance of your proposed project with respect to *RUNX1*-FPD research (must be aligned with *RUNX1* Research Program & ALSF mission). Relate the specific aims to the goals and long-term objectives.
- c. **Innovation (0.5 page):** Describe how the proposed research challenges and shifts paradigms or introduces a novel concept, approach, or technology.
- d. **Approach (4 pages):** Describe the experimental approach to the research question and how the research will be realistically accomplished within the proposed funding period. Images, graphs, and charts that are critical to the project should be included within this section, not in the appendix. They will count against the page limit. This section must include but is not limited to:

- i. Feasibility of the approach to reach project goals; if available, include PI's preliminary studies pertinent to the project.
 - ii. Key milestones with expected outcomes, details of potential problems, and plans to address these issues.
 - iii. A timeline in the form of a simple Gantt chart.
- 5. Resource Sharing (1-2 pages):** Use the [Resource Sharing Form](#) to complete this section of the application to describe the outputs from the proposed project and how they will be shared. Reviewers will be asked to consider the way outputs from this project will be shared and the extent to which this plan will increase or decrease the impact of the proposed project.
- 6. Plans for Clinical Translation (1 page):** If the specific aims described above are successfully met, state what steps you will take to move your research forward to a clinical therapy. Be specific and forecast what may be achievable within a 5-year horizon.
- 7. Human Subjects (0.5 page):** If approved, include the IRB approval letter or equivalent. If approval is pending, indicate the expected approval date. Any funds awarded will be held until the letter is received. If IRB approval is not applicable, include a note in this section.
- 8. Vertebrate Animals (0.5 page):** If approved, include the IACUC approval letter or equivalent. If approval is pending indicate the expected approval date. Any funds awarded will be held until the letter is received. If IACUC approval is not applicable, include a note in this section.
- 9. Literature Cited:** A maximum of 30 references are allowed. Use Vancouver format (numbered citations within text).
- 10. Letters of Support (no limit):** Include any appropriate letters from individuals confirming their roles in the project. Institutional letters of support are not required, but can be included, especially if there are issues of feasibility that can be addressed.
 1. **Appendix:** A brief appendix is allowed for RARE Grants. Appendices should be included only if essential to the understanding of the application. Appendices are limited to one accepted but not yet published manuscript OR two pages of additional information such as a summary of the protocol and supplementary figures. (Reminder: tables and figures noted in the text should be embedded in the body of the proposal.) Reviewers are not required to read the appendix.

Application Submission Instructions

- Details to follow soon! ALSF is migrating to a new portal for grant application submissions. In the meantime, we encourage researchers to prepare materials in anticipation of our portal launch in Fall of 2023. Once the ALSF grant application portal is live, ALSF will send an email announcement and update the 'Application Submission Instructions' to the guidelines for the 2024 RARE Grant (check back here).



Contact

- If you have any questions regarding your eligibility or your research proposal topic, we highly encourage you to reach out to Dr. Katrin Ericson, Executive Director at RRP at KEricson@Runx1-FPD.org.
- All other questions can be submitted to Margaret Poore at M.Poore@AlexsLemonade.org.