

REQUEST FOR PROPOSALS

Familial Platelet Disease (FPD)/RUNX1 Translational Research

LOI APPLICATION DEADLINE
January 13, 2017

The Leukemia and Lymphoma Society (LLS) and the Babich Family Foundation are partnering to launch a competitive grant to advance new therapies for, and obtain a greater understanding of, familial platelet disorders associated with RUNX1 mutations that lead to acute myelogenous leukemia. The program is focused on addressing translational research with a total of \$1.8 million; 3 grants will be awarded up to \$600,000 each.

About FPD/AML

Germline mutations in a one copy of RUNX1 (known as acute leukemia 1 protein [AML1] or core-binding factor alpha-2 [CBFA2]) can create a pre-leukemic state that typically manifests as a familial platelet disorder (FPD). Approximately 50% of patients with familial RUNX1 mutations will develop AML in their lifetime associated with acquisition of a second mutation. Beyond this, RUNX1 mutations are found in approximately 15% of all AML patients, with 11% and 31% of patients that have de novo and secondary AML, respectively. RUNX1 mutations are also found in patients with myelodysplastic syndromes.

It is currently poorly understood how RUNX1 can mediate platelet defects or lead to AML. This grant program aims to fund cutting-edge research with the potential to transform therapeutic options for pre- and post-leukemic FPD/AML patients as well as to contribute to current science's limited understanding of the role RUNX1 plays in leukemogenesis.



For application
information:

www.lls.org • lls.fluixx.io • researchprograms@lls.org

runx1
The FPD/AML Research Program

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Research proposals should include, but are not limited to:

Translational Research that includes, or can lead to, clinical trials

- Discover and develop novel therapeutics that re-activate RUNX1 or downstream pathways or provide synthetic lethality via collateral pathways to restore impaired (mutated) RUNX1 function
- Develop gene editing or gene therapy methods to correct RUNX1 mutations, thereby allowing allogeneic transplantations
- Develop new prognostic assays to determine if or when to treat with such new therapeutics

Laboratory research that supports Translational Research

- Develop a deeper understanding of the interaction of RUNX1 with other transcription factor regulators or other relevant biology related to disease progression
- Develop experimental systems either in vitro or in vivo that mimic FPD (or other RUNX1-mediated pre-leukemic conditions)

A detailed description of the LLS Translational Research Program and application instructions are available at <https://lls.fluxx.io> or <https://www.lls.org> under the Research tab. All applications are to be submitted online through FLUXX at <https://lls.fluxx.io>. The LLS and BFF seeks proposals responsive to the above requests for proposals, but will also consider other exceptional proposals with the near-term potential of clinical translation. Winners of the competition will be announced June 30, 2017. Grants will activate October 1, 2017.

For additional questions regarding LLS grant programs, eligibility and application processes, please contact researchprograms@lls.org.



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