Massachusetts General Hospital
Corporate & Foundation Relations
Office of Development

PRIVATE FUNDING OPPORTUNITIES: MAY 6, 2016

Please contact Corporate & Foundation Relations in the Office of Development at devcfr@mgh.harvard.edu if you wish to submit a proposal in response to any of these opportunities. Note that proposals are still routed through the standard InfoEd/Research Management process.

1. Familial RUNX1 Grant Program: Familial RUNX1 Patient Samples Grant, Alex's Lemonade Stand Foundation for Childhood Cancer (ALSF)

ALSF is committed to creating opportunities for new and innovative research into treatments and cures for childhood cancers. The Babich Family Foundation is dedicated to advancing areas of research of particular importance to families affected by familial platelet disorders (FPDs) leading to acute myeloblastic leukemia (AML) due to germline RUNX1 mutations with the ultimate goal of developing effective therapies to prevent the onset of AML. ALSF and The Babich Family Foundation have partnered to raise awareness and accelerate research around familial RUNX1 disorders.

The purpose of this grant is to increase understanding of the disease pathogenesis of familial RUNX1 disorders by facilitating correlative analyses of longitudinal patient samples and outcome data. Identification of sources for the collection and annotation of samples from patients with familial RUNX1 disorders will be foundational to building the familial RUNX1 disorder research program.

Research Directions of Interest:

1. Documentation of the order, frequency and clinical significance of specific accumulated defects through prospective collection and analysis of samples from pre-leukemic subjects with familial RUNX1 disorders. Retrospective incorporation of samples and data from the cell banks of consortia and genetic screening centers by techniques that identify samples from patients not originally diagnosed with this disorder is also of interest. The goal is to understand the disease transition from a pre-leukemic to a leukemic state.
2. Identification of the conditions or secondary mutation(s) responsible for disease progression of familial RUNX1 disorders through retrospective analyses of samples from patients who have undergone myelodysplastic or leukemic transformations.
3. Proposals may also combine approaches #1 and #2.

Award Amount: Up to $500,000 paid over 2 years
LOI Deadline: Jun 30, 2016
Website: http://www.alexslemonade.org/grants/runx1

2. Familial RUNX1 Grant Program: Familial RUNX1 Research Grant, Alex's Lemonade Stand Foundation for Childhood Cancer (ALSF)

Alex's Lemonade Stand Foundation (ALSF) is committed to creating opportunities for new and innovative research into treatments and cures for childhood cancers. The Babich Family Foundation is dedicated to advancing areas of research of particular importance to families affected by familial platelet disorders (FPDs) leading to acute myeloblastic leukemia (AML) due to germline RUNX1 mutations with the ultimate goal of developing effective therapies to prevent the onset of AML. ALSF and The Babich Family Foundation have partnered to raise awareness and accelerate research around familial RUNX1 disorders.

Purpose of the grant is to fund research that will develop strategies leading to the development of therapies for the prevention of the transition from pre-leukemia to leukemia for patients with the familial RUNX1 disorder. Projects should focus on familial RUNX1 disorder rather than acquired RUNX1 mutations seen during the evolution of AML.

Research Directions of Interest:

- Studies that develop our understanding of the biological basis for disease progression in individuals carrying familial RUNX1 mutations.
- Studies to develop potential therapies to limit disease progression from individuals with the familial RUNX1 deficiency.
- Development of in vitro and in vivo model systems (including but not limited to human cell, mouse or zebrafish) that: increase our understanding of disease pathogenesis, test mechanistic hypotheses, or that provide a suitable evaluation platform for the discovery of potential therapies.

The grant is designed as seed funding for researchers with a novel scientific approach to investigating hereditary or germline RUNX1 disorder. This may represent a change in research direction and/or an innovative new idea that moves away from an investigator's prior research, but for which a strong case can be made for the potential impact on familial RUNX1 disorder.

Award Amount: $250,000 paid over 2 years
LOI Deadline: Jun 30, 2016
Website: http://www.alexslemonade.org/grants/runx1

3. Grant Opportunities, LAM Foundation

Since 1996, T he LAM Foundation has been funding significant research that has led to tremendous progress in the treatment options and a possible cure for lymphangioleiomyomatosis. Today, The LAM Foundation is the largest funder of LAM research, outside of the National Institutes of Health.

The LAM Foundation-funded research has led to many major discoveries, including:

- Evidence that LAM is genetic

- Identification of a LAM gene
- Molecular explanation for abnormal smooth muscle cell growth in LAM, which has led to new therapies for LAM
- FDA approved treatment for LAM

The LAM Foundation offers the following research awards and grants:

- Career Development Awards
- Established Investigator Awards
- Pilot Project Awards
- Other awards (The LAM Foundation also considers proposals for clinical trials, bridge funding and other special projects.)

Award Amounts:

- Career Development Awards provide a maximum of $50,000 per year, renewable for up to two additional years).
- Established Investigator Awards provide a maximum of $50,000 per year, renewable for up to two additional years. This award may be condensed to two years should the investigator request it.
- Pilot Project Awards provide up to $25,000 and are available for the initiation of innovative research projects.

LOI Deadline: Jun 15, 2016
Website: http://www.thelamfoundation.org/research/apply-for-lam-funding

4. Neuro-Habilitation Program, Rettsyndrome.org (International Rett Syndrome Foundation)
"To reset the neurology we must find methods to habilitate cognition, speech, occupational and physical activities."

Neuro-Habilitation HeART (Help Accelerate RTT Therapeutics) grant awards are designed to provide funding for research aimed at maximizing function and abilities in the boys and girls with Rett syndrome. The program specifically targets research for cognitive therapies, physical therapies, occupational therapies and speech therapies, which will directly impact the quality of life for those affected by Rett syndrome.

Award Amount: $150,000 for 2 years
LOI Deadline: May 31, 2016
Website: https://www.rettsyndrome.org/research/funding-opportunities

5. Basic Research Grant Program, Rettsyndrome.org (International Rett Syndrome Foundation)
Rettsyndrome.org offers Basic Research grants for international biomedical research to promote the understanding of MeCP2 in the pathogenesis of the neurobehavioral phenotype of Rett

syndrome (RTT). RTT seeks gain a better understanding of the underlying pathology of the disorder that will lead us into potentially new treatment avenues for the amelioration of the symptoms and a cure for RTT.

The Basic Research grant awards are meant to (1) provide seed money for basic research that will generate the new ideas that will lead us to innovative therapeutic approaches, (2) design to assist investigators establish careers in fields relevant to Rett syndrome research, and (3) lead to follow-on funding from other agencies.

Award Amount: $150,000 for 2 years
LOI Deadline: May 31, 2016
Website: https://www.rettsyndrome.org/research/funding-opportunities

6. Translational Research Grant Program (HeART (Help Accelerate RTT Therapeutics)), Rettsyndrome.org (International Rett Syndrome Foundation)
HeART (Help Accelerate RTT Therapeutics) grant awards are designed to promote the development and testing of therapeutics to treat and reverse Rett syndrome (RTT).

Awards will be provided for cell-based assay development, early-stage drug discovery and development, early stage medicinal chemistry efforts on high value candidate therapeutics, cell-based screening of candidate therapeutics and follow-on early-stage in vivo testing. Grant applications for exploratory studies towards development of biomarkers or objective clinical outcome measures will also be considered. The goal of this award mechanism is to provide seed funding for early stage drug discovery and development efforts.

The sponsor encourages novel research programs that broadly encompass the following areas of unmet need:

- Design, synthesis and testing of potential disease modifying therapeutics to treat or reverse RTT
- Testing of existing therapeutics both in vitro and in vivo to repurpose their use in RTT
- Development and/or validation of in vitro and in vivo models of RTT for therapeutic testing
- Development and/or validation of novel biomarkers for objective clinical trials outcome measures
- Testing of IND-ready therapeutics in pilot clinical trials

Award Amount: $150,000 for 2 years
LOI Deadline: May 31, 2016
Website: https://www.rettsyndrome.org/research/funding-opportunities

7. Mentored Training Fellowship Program, Rettsyndrome.org (International Rett Syndrome Foundation)

Rettsyndrome.org offers Mentored Training Fellowships to support scientists early in their career to in both basic and clinical research in the field of Rett syndrome so that they become successful, independent basic research scientists and clinical investigators. These fellowships are designed to assist post-doctoral and clinical scientist researchers in training to establish careers in fields relevant to Rett syndrome research.

Rettsyndrome.org encourages novel basic research programs for Mentored Training Fellowships within the following emphasis areas:

- Understanding the role of MeCP2 during normal brain development
- Characterizing the role of MeCP2, including MeCP2 target genes, in normal structure and function of the developing and adult nervous system
- Determination of the relationship between patterns of expression of MeCP2, FoxG1, CDKL5, and related proteins in the nervous system and the neurologic and behavioral phenotypes of patients with RTT and/or related animal models
- The investigation of neuronal abnormalities that result from MeCP2 dysfunction
- The role of microglial and macroglial cells in development as it relates to RTT
- Understanding mechanisms and systems leading to aberrant behavior in RTT

Award Amount: $100,000 paid over 2 years
LOI Deadline: May 31, 2016
Website: https://www.rettsyndrome.org/research/funding-opportunities