

**JDRF CCTN and the WB Family Foundation
Translational Program To A Cure Awards
Request for Applications**

Key Dates

Release Date: August 5, 2016

Letter of Intent Due Date: September 15, 2016

Notice of LOI Decision: October 27, 2016

Application Receipt Date: December 8, 2016

Earliest Anticipated Start Date: March,
2017

This RFA contains the following information:

- Goals and Research Objectives
- Eligibility
- Budget
- Instructions to submit an application
- Review of Applications
- Terms of Award
- Program Contacts

JDRF Canadian Clinical Trial Network (“JDRF CCTN,” www.jdrf.ca/cctn) is a platform that has been established for clinical research to test new treatments and technologies for type 1 diabetes (T1D). JDRF CCTN is pleased to announce a gift of \$3.5 million from the WB Family Foundation (WBFF) that is intended to support additional research opportunities in Canada to focus efforts aimed at curing T1D.

JDRF CCTN/WBFF Translational Program *To A Cure* Awards will be supported up to a maximum of \$1.5 million CAD total costs over 3 years. Emphasis will be placed on clinical translation efforts that pursue a cure for T1D. Applications may include more than one project, and may include pre-clinical research with the direct potential to lead to clinical trials. Collaborations among institutions, investigators, individuals and industry, especially those intended to take advantage of and build upon JDRF CCTN infrastructure, are encouraged. Programs that build upon ongoing JDRF CCTN activities are welcome.

This program is available to investigators who are based at Canadian institutions and may be from academic institutions or industry. Although principal investigators must be at a Canadian institution and of Canadian Nationality, collaborators from outside of Canada may be funded as part of an overall application.

Strategic Goals and Specific Objectives of the JDRF CCTN and the WB Family Foundation Translational Program To A Cure

The purpose of the JDRF CCTN-WB Family Foundation (WBFF) Translational Program *To A Cure* is to support the development of therapies to cure human type 1 diabetes (T1D), an autoimmune disease resulting in loss of functional beta cell mass and insulin dependence. Recent data suggests that there is extensive cross-talk between the beta cell and the immune system in the ongoing pathogenesis of T1D. After a diagnosis of T1D, a cure refers to treatments that have the potential to restore insulin independence through the restoration of normal beta cell function and protection of the restored beta cells from destruction. Thus, reversal of the disease may require combination therapeutics targeting the autoimmune process in addition to beta cell health/survival and regeneration.

Applications to the JDRF CCTN-WBFF Translational Program *To A Cure* should focus on immunomodulatory therapeutic approaches to restore and maintain immune tolerance in individuals who have been newly diagnosed for T1D or who have established T1D. Such immunotherapeutic approaches should include one or more of the following objectives:

1. Develop and/or evaluate novel immunotherapies in proof-of-concept (POC) trials that:
 - a. purge pathogenic autoreactive T cells
 - b. enhance immunoregulatory cells in a beta cell antigen-specific manner (*i.e.*, antigen-specific immunotherapy) or a polyclonal manner (antigen non-specific immunotherapy) to suppress pancreatic islet inflammation
2. Combination immunotherapeutic approaches that target the above pathophysiological processes are emphasized, and may include therapies that enhance β cell survival or regeneration in combination with an immunotherapy
3. Evaluate novel biomarkers and associated assays that are expected to reliably measure mechanism-of-action of proposed therapies*. These may include:
 - a. Biomarkers/assays that measure beta cell status pre- and post- therapy
 - b. Biomarkers/assays that measure relevant immune status pre- and post- therapy
 - c. Algorithms that involve multi-dimensional evaluation of biomarkers used to determine predictive value, mechanism-of-action, or efficacy-surrogate markers of therapies.

**Candidate biomarkers must be accompanied by robust preliminary data. Biomarker discovery efforts with trial samples will not be supported by this call.*

Additional Activities

Awardees supported by this program will become part of JDRF CCTN, with the same rights and responsibilities as other JDRF CCTN members.

Eligibility

Applicants must be Canadian and/or be based at a Canadian institution. Applicants should hold a D.M.D., D.V.M., M.D., Ph.D., or equivalent and have a faculty position or equivalent at a college, university, medical school, hospital, or other research facility. Collaborations among institutions, investigators, and industry, especially those intended to take advantage of and build upon JDRF CCTN infrastructure, are encouraged. Companies may also apply to this call, however demonstrable expertise/collaboration on the topic of T1D targeted immunotherapies will be required.

Budget

A maximum of \$1.5 million CAD total costs may be requested over a 3 year period. Funds requested must be used only for expenditures directly related to research. Costs for renovation, alteration or other infrastructure costs are not allowed. Allowable expenses include salaries for technicians, materials and supplies, and maintenance contracts. Requested funds should include 10% overhead costs built into the budget.

Budgets should be quoted in Canadian dollars and provided for the entire project period, broken down annually. Clinical trials should be budgeted and will be funded based on meeting established trial milestones including enrollment, patient follow-up, etc.

Interested parties should note the following:

JDRF is committed to the publication and dissemination of all information and materials developed using JDRF resources. All recipients of JDRF awards must agree to this principle, and must take steps in order to facilitate availability of data and samples. Awardees must commit to publishing their findings at the end of their funding timeframe so their work is in the public domain and available to other researchers.

Awardees supported by this program will become part of JDRF CCTN, with the same rights and responsibilities as other JDRF CCTN members.

Instructions to Submit an Application

Applicants should register and submit their Letter of Intent and application in response to this RFA in RMS360 (<http://jdrf.smartsimple.us>)

Letter of Intent

Prospective applicants should submit a Letter of Intent (LOI) 3 page maximum- on-line by September 15, 2016. LOIs will be competitively reviewed based on relevance to the goals of the RFA, strategic fit with JDRF's mission and portfolio and scientific merit/feasibility. Applicants will be notified approximately four weeks after the LOI deadline date if they have been approved to submit a full application.

LOI should include the following:

- A descriptive title of the proposed Program and a clear mission statement, the Program's priority goal, clinical trial plan, including the target patient group and the intervention tested; Projected timelines and milestones for each year of the proposal;
- A clear description of the deliverables and next steps if successful;
- The name, address, telephone number, and email address of the Principal Investigator;
- An estimated budget;
- The identities of other key personnel and participating institutions;
- The titles and a short description of component projects; and
- Research project descriptions indicating the translational significance, gap-filling nature and potential impact of the proposed studies for type 1 diabetes.

Application

Full applications will only be accepted from applicants with an approved Letter of Intent.

The full proposal should contain the following: a Table of Contents and a Program Overview.

The application for each component project should include a detailed research plan/description with milestones, biographical sketches and information about other support for the applicants, and a detailed

budget. The research plan for each project should not exceed 10 pages. Tables, charts, figures and references are not counted as part of the page limits. Individual projects may vary in timelines and duration.

Review and JDRF Approval

The Program application will be evaluated competitively by an external expert peer review committee based on the criteria described below.

Review of the Translational Aspects and Potential of the Project

The relationship and contributions of each individual research project and its translational potential will be evaluated by the review committee. The following criteria are among those considered:

Relevance: Does the proposed research meet the goals and objectives of the RFA?

Significance: Is the Program addressing a fundamental question or challenge in human T1D, that if addressed successfully, will have a major impact on understanding and curing human T1D?

Approach: Is the approach taken by the Program scientifically valid and technically feasible?

Innovation: Is the proposed research novel and addressing key gaps or major challenges using innovative approaches?

Translational Impact Potential: Will the Program move the field significantly and meaningfully closer to a cure of human T1D? What is the potential impact of the Program on the lives of those living with T1D?

Appropriateness of the proposed budget and duration in relation to the proposed research. Is the overall budget appropriate and efficient?

Institutional and other sources of support: What resources have been leveraged? What is the institutional commitment to the Program (financial support, time protection, and laboratory space and equipment, etc.)? Are there any other sources of financial support for the request, which could include: cost-sharing arrangements with the sponsoring institution(s), industry (keeping in mind that university-industry collaborations are strongly encouraged), or other agencies

Terms of Award

JDRF CCTN/WBFF Translational Program To A Cure Awards will be supported for a maximum of \$1.5 million CAD total costs over 3 years. Evidence of institutional commitment in the form of dollars or non-financial resources is also required.

Program Contacts

Please direct any scientific inquiries regarding this funding opportunity to :

David Alleva, Ph.D.

Email: dalleva@jdrf.org

Tel: 212-479-7547

Dr. Bob Goldstein

Email: gold@jdrf.ca

Tel: 301-651-1898

Please direct any administrative inquiries to Suvina Amin at samin@jdrf.ca.