

PROGRAM DETAILS  
Early Phase Clinical Trials: Canada  
(Rolling Applications)

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**NEW for 2018**

- The Institute has updated which **tools** are in scope. Please see Section 2 below or read our [What We Fund](#) webpage before applying to ensure your project is in scope.
- The Early Phase Clinical Trials: Canada 2018 program will now be run on a rolling basis.

### Background

The Weston Brain Institute (the “Institute”) supports neuroscience research that accelerates the development of therapeutics for neurodegenerative diseases of aging. To help achieve this, the Institute addresses gaps and inefficiencies in the funding market by supporting high-risk, high-reward translational projects, while leveraging world-class business and scientific expertise in a fast and flexible granting process.

The Institute was founded by The W. Garfield Weston Foundation, which supports the Institute’s Canadian programs. The Institute’s programs in the UK, the Netherlands, and Ireland are supported by the Selfridges Group Foundation. The Selfridges Group consists of Brown Thomas and Arnotts in Ireland, Holt Renfrew in Canada, Selfridges in the UK and de Bijenkorf in the Netherlands.

Neurodegenerative diseases of aging are among the least understood and most undertreated diseases today. Diseases such as Alzheimer’s and Parkinson’s are placing a large and increasing burden on society. If ignored, the social and economic costs to manage these diseases will rise significantly within a generation.

Meeting this challenge requires pioneering approaches to accelerating treatments. The Early Phase Clinical Trials program was launched to provide significant support for clinical trials and clinical trial sub-studies with excellent preliminary data.

### Important things to know about the Institute:

- **We do not fund basic research (also known as fundamental) research.** We only fund translational research.
- **We do not fund complementary approaches** (e.g., exercise)
- **Funds are provided contingent on meeting milestones.** If your project is awarded, funds are provided in tranches when experimental milestones are successfully completed.
- **Our application process is interactive.** You will likely receive feedback on your applications and may be asked to make modifications. We also encourage you to contact us if you have questions about our funding programs.

- **Many projects are declined at the Letter of Intent (LOI) stage.** Only ~15% of LOIs are invited to the Proposal phase, so that applicants and reviewers spend their time on Proposals that have an excellent chance of being funded. Proposal funding rates have ranged from 30-100%.
- **We provide more than funding.** Our grantees may also benefit from things such as expert advice from our scientific advisors, industry exposure, networking, and international collaboration opportunities.

## Section 1 Program Scope

The Early Phase Clinical Trials program provides funding to support translational research that accelerates the development of therapeutics for neurodegenerative disease aging.

Projects must meet these criteria to be eligible, following the institute definitions and scope in Section 2:

1. Be a clinical trial(s) and/or a clinical trial sub-study(s) that accelerates the development of therapeutics for neurodegenerative diseases of aging.
  - Projects that require only up to \$200,000 over up to 18 months may also be submitted to the Rapid Response program.
  - Translational research other than clinical trials and/or clinical trial sub-studies should be submitted to the Transformational Research program or the Rapid Response program.
2. Be the development of a therapeutic and/or tool.

You are encouraged to contact the Institute with any questions regarding the program, including whether a potential idea is in scope.

## Section 2 Institute Definitions and Notes on Scope

- **Neurodegenerative diseases of aging:** Alzheimer's disease, frontotemporal dementia, dementia with Lewy bodies, multiple system atrophy, Parkinson's disease, progressive supranuclear palsy, vascular contributions to these diseases (not stroke-mediated vascular disease), and prodromes to these diseases (e.g., mild cognitive impairment as prodromal to Alzheimer's disease; REM sleep behavior disorder as prodromal to Parkinson's disease).
- **Translational:** Applied research towards developing therapeutics for the prevention and/or treatment of human disease. For example, for small molecule drug development, this includes target validation to Phase IIa clinical trials. Basic/discovery research, including but not limited to understanding disease mechanisms and discovering genes implicated in disease, is not in scope.
- **Therapeutic:** A pharmacological approach (including small molecules, biologics, cell therapies and vaccines, including drug repositioning and repurposing), medical device, surgical intervention, or magnetic or electrical brain stimulation. Therapeutics can be for symptomatic relief, disease modification, or prevention. Complementary approaches such as exercise, acupuncture, music, dietary and nutritional supplements are not considered therapeutics. Identification of novel therapeutics is in scope (e.g., high throughput compound screens); however, identification of novel therapeutic targets, including genes implicated in disease, is not

in scope.

- **Tools:** An item that accelerates development of therapeutics, e.g., imaging techniques or reagents, biomarkers, and diagnostics.
  - Tools must have direct impact on the translational development of therapeutics (as defined by the Institute, i.e., target validation to phase IIa clinical trials) for neurodegenerative diseases of aging and will be valued only on their ability to do this.
    - Any value the tools contribute to basic research will not be taken into consideration. For example, tools will not be valued for their ability to identify new targets or understand disease mechanisms.
  - Projects covering only the discovery/identification of a tool are out of scope.

**Notes about biomarkers:**

- Biomarkers must be being developed for human disease diagnosis, prognosis, for patient stratification to clinical trials or to predict response to therapies (surrogate for a clinical endpoint).
  - Biomarkers should measure pathology of the disease (e.g., fluid, imaging or tissue biopsy derived biomarkers) and not be based on behavioural phenotypes (e.g., gait or grip strength).
  - Genetic biomarkers including somatic mutations, SNPs, epigenetics and gene products are in scope if they meet the other eligibility criteria.
- If the project includes biomarker identification:
  - The project must also include experiments to validate the biomarker.
  - All the samples/data necessary for identification and validation must already be available/collected unless there is sufficient justification to collect new samples/data (e.g., samples cannot be stored).
  - Validation of biomarkers must occur in a well-characterized human subjects/samples/data. This validation must be in samples/data from different subjects than those used to identify the biomarker.
  - Post mortem tissue can only be used for validation of biomarkers previously identified in living subjects.

An identified biomarker is defined by the Institute as one that meets the following 4 conditions:

1. Specific item(s) or signature to be measured can be defined;
  - For e.g.,
    - Presence of a particular bacterium
    - Disease-specific EEG signature
    - Specific brain structure with reduced volume
    - Single protein increased
    - Precisely defined fingerprint
      - If the biomarker is a fingerprint of a family of proteins or a signature of brain volume changes, the precise fingerprint or signature to be replicated must be

previously determined. For e.g., omics studies for the purpose of identifying biomarker patterns or signatures are out of scope.

- Exact identities of multiple individual factors that may be useful individually or as a specific composite
- It is not in scope to know that a family of proteins is affected or that brain volume is changed overall, if the specific item or signature that is the biomarker cannot yet be specified. For example, a single protein is not considered an identified biomarker if only the family of proteins were previously identified to be affected.
- 2. In what it will be detected (e.g., which tissue/fluid), using what assay, and for what disease, can be clearly stated;
- 3. Specific item(s) (or signature) to be measured has been shown to be detectable in humans or human-derived samples/data in the tissue/fluid to be tested;
- 4. Compelling data exists to justify moving to validation (as defined by the Institute).
  - The most compelling data is likely in humans or human-derived samples/data with a relevant disease
  - The most compelling data will likely allow for a power calculation
  - Data from pathophysiologically relevant animal models could be considered if those animal data are compelling

Biomarker validation is defined by the Institute as:

- Testing a previously identified biomarker in a sufficient number of appropriate, comparable, well-characterized human subjects/samples/data to determine whether it is a sensitive and/or accurate biomarker.
  - If the proposed assay is different than the one used for initial biomarker identification, or if the assay will be used in a different type of specimen (e.g., different tissue/fluid or different species) then preliminary data must be provided to demonstrate that the assay works appropriately. For example, if a biomarker was identified using an assay in CSF and you are proposing to use the same assay to validate a biomarker in blood, there must be preliminary data demonstrating the assay works in blood.
  - Replication studies are not considered to be validation, e.g., using subjects with a different disease stage, or subjects on different drug regime if that regime could affect the biomarker.

***For cognitive assessment tools and clinical assessment instruments:***

- If developing a cognitive assessment tool or a clinical assessment instrument, the tool must be being tested on patients with a relevant disease.
  - E.g., development of a questionnaire to assess cognitive decline.
- Requires discussion of why the new assessment would be better than existing ones
- **Clinical trial:** The Institute defines a clinical trial to be research in which one or more human subjects are prospectively assigned to one or more interventions to evaluate the effects of those

interventions on health-related biomedical or behavioral outcomes. A clinical trial sub-study is defined by the Institute to be a study investigating a question not addressed by the main trial, and which may involve obtaining additional measurements and data collection from a sub-group of all participants from the main trial.

### Section 3 Eligibility of Applicants

For this program, the Institute is only able to accept Letters of Intent (“LOIs”) and Proposals from institutions located in Canada (or individuals affiliated with and applying through or on behalf of institutions) that are designated as Canada Revenue Agency qualified donees. Funds can also be used to support the Canadian portion of collaborations with members from other geographies.

Eligible Principal Applicants must be at or above the level of Assistant Professor or equivalent. Co-applicants and Collaborators must be at the post-doctoral level or above, and can be working outside Canada.

Applicants may appear in any role on any number of LOIs.

Proposals submitted to this Program must be approved by the institution on whose behalf or through which the Proposal is being submitted. However, LOIs do not need to be approved in this manner.

### Section 4 Funding Specifications

**Overall funds:** The Institute is able to fund many grants through this program, and historically has not turned down Proposals due to the lack of funding. Grants are contingent on the receipt of high quality applications.

**Funds available per project:**

- Up to \$1,500,000 over up to 4 years is available per project.
- Funds will be granted only for direct costs that are appropriate and justifiable for the work proposed.
- Funds cannot be used for equipment purchases, computer purchases, administrative costs or indirect costs, unless prior written approval from the Institute has been obtained.
- Funds cannot be requested for personnel who have their salary paid for by their institution.
- Publication costs and travel expenses to scientific conferences/meetings to present work funded by the Weston Brain Institute can be included in the budget.
- Each item and its cost must be clearly described in the budget (provided at the Proposal stage only).
- The amount granted may not be for the full amount requested.
- Up to 35% of the funds can be used to bring unique international resources into work led by Canadian researchers.

Any grant provided by the Institute pursuant to this Program shall be directed to the institution and not to the individual affiliated with and applying through the institution. Responsibility for the planning, direction, and execution of the proposed project will rest solely with the Applicants. If the funded

project has significant commercial success, the Institute may request repayment or reallocation of grant funds at the discretion of the Institute to be used for further research funding. This option is included in the grant agreement.

**Multiple institutions:** In the event of collaboration between multiple institutions, it is the responsibility of the Principal Applicant to distribute/manage funds appropriately.

**Full or partial support of projects:** The Institute can support a full project or parts of a project. The grant can be used as matched funds. If the application is for part of a larger project, the criteria for granting will be applied only to the part of the proposed project. Applicants should make clear what part of the larger project the Institute funding would support.

**Conditional funding and milestones:** Grants are conditional on grantees meeting pre-determined milestones and providing deliverables, including submission of progress reports and participation in Institute sponsored assessment meetings. Continued support is not automatic and is contingent upon the progress reports being favourably reviewed by the Institute.

**Supplemental funding:** The Institute encourages grantees to seek additional funds to further their work once the term of the initial grant has expired. The Institute has no guaranteed policy for renewal or continuation of grants. The Institute may, at its discretion, seek to further support clearly successful projects. Grantees are also eligible to apply for funding through other Institute programs.

## Section 5 Application Process

The application process consists of two stages: LOIs and Proposals. To apply, applicants must submit an LOI to the Institute. Selected applicants will then be invited to submit a Proposal.

The LOI stage of the application process is a significant stage of evaluation. Typically, only a small proportion of applicants are invited to submit full proposals, and of those, many will be funded. This ensures LOIs are easy to submit so that promising ideas are not missed, while ensuring applicants taking the time to write full proposals have a very good chance of being funded. In past programs, ~15% of applicants submitting LOIs were invited to submit Proposals, and at least 30-50% of submitted Proposals were funded.

Each LOI will be peer reviewed by a scientific review committee. Applicants whose LOIs meet the rigorous review criteria will be invited to submit a Proposal. Budgets are only required at the Proposal phase. Proposal instructions and feedback from our scientific review committee will be forwarded along with the invitation. Complete Proposals will be peer reviewed by the scientific review committee. No appeal process is currently available.

Applicants can expect to receive the outcome of their LOI application within ~2 months of submission. Applicants who are invited to the Proposal phase will have ~2 months to submit the full application after the invite notification. If different timelines are needed for Proposal submission, please contact the Institute.

The grant agreement must be completed within 6 weeks of notification of selection; otherwise, the

Institute reserves the right to cancel the grant.

## Section 6 Review Criteria

Criteria considered when reviewing LOIs:

- **Supporting data and rationale for the study:** Is there sufficient data from the previous stages of therapeutic development to warrant the next stage of testing and the proposed trial or sub-study? Is the preliminary data compelling?
- **Experimental approach:** Are the overall strategy, methodology and analyses well-reasoned and appropriate to accomplish the specific aims of the project? Are go/no-go criteria well-defined? Is the study designed with the proper considerations for key design elements such as the length of the trial (including regulatory and REB milestones), leadership structure, specific outcome measures/endpoints, inclusion/exclusion criteria, sample size, and subject recruitment and retention plan? How likely is the hypothesis to be proven or disproven?
- **Impact:** If successful, will the project accelerate the development of therapeutics for neurodegenerative diseases of aging in a significant and sustained way?
- **Team and environment:** How well-suited are the team and environment for this work? Does the work take advantage of unique features of either?
- **Fit:** Is the project, including its scale and scope, appropriate for this program?
- Other as needed.

Additional criteria considered when reviewing Proposals:

- **Experimental approach:** Are potential problem areas and common pitfalls adequately considered and addressed?
- **Development plan and future objectives:** Are there clear next steps after this study to continue development if successful?
- **Budget and timeline:** Are the proposed budget, milestones and length realistic yet aggressive for the research proposed? The budget and milestones are secondary considerations after the other criteria have been weighed.
- Other as needed

## Section 7 Reports and Assessments

Grantees must complete the following if a grant is awarded:

- *Milestone Reports.* Payments are tied to successful completion of project milestones mutually agreed upon by the Applicants and the Institute. A milestone report is due prior to each scheduled payment being made. Templates for the milestone report will be provided by the Institute.
- *Progress Reports.* A progress report includes a written report with budget and, if requested by the Institute, a telephone discussion with the Principal Applicant and/or data underlying the research (solely for use in assessing progress). Progress reports are due annually unless otherwise notified by the Institute. Templates for the progress report will be provided by the

Institute.

- *In-person meetings.* Grantees may be asked to come present the progress to date of their funded work to the Institute. Travel expenses required to attend in-person meetings will be covered by the Institute per Institute guidelines and does not need to be included in the budget.
- *Foundation Member Visits.* With prior consent of Applicants, Foundation members may wish to visit researchers to see project work underway. These visits are not mandatory, and the Institute hopes that grantees will welcome this opportunity.
- *Financial Accountability.* Grantees are expected to account for the moneys expended under any Institute grant; any moneys spent either not in accordance with the approved research project or prior to pre-approval of any material change in the project are both recoverable and subject to restitution by the grantees to the Institute and may be cause for immediate termination of funding. Any funding provided beyond what is needed for the agreed upon research must be returned to the Institute.

## Section 8 Confidentiality

The Institute treats all LOIs, Proposals, research projects and associated research information (collectively, the “Confidential Information”) in confidence using reasonable care in protecting such Confidential Information from disclosure to third parties who do not participate in the grant review process and Institute assessments. All Confidential Information will be used by the Institute and its scientific review committee for the purposes of reviews and assessments, and will be shared only in accordance with the sharing policy as set out herein. Notwithstanding the foregoing, Confidential Information shall not include any information that:

- a) was generally known to the public prior to the effective date of this Program announcement;
- b) becomes generally known to the public through no unlawful or unauthorized act by any recipient of Confidential Information; or
- c) was independently developed by the Institute or its scientific review committee without reference to the Confidential Information.

If the Institute or any of its scientific review committee members is requested to disclose Confidential Information pursuant to a legal or governmental proceeding, the Institute shall give the Applicant or other owner(s) of such Confidential Information notice of such disclosure request as soon as is reasonably practicable.

## Section 9 General Information

Institutions and individuals affiliated with and applying through or on behalf of institutions (collectively, “Applicants”) should carefully discuss the Program announcement and the terms of this document with the appropriate office at their institution before submitting an application. The submission of an LOI or a Proposal (“Proposals” and each a “Proposal”) does not bind either the Institute or the Applicants by any



commitment to provide or receive funding, respectively. Successful Applicants will be required to agree to terms substantially similar to those contained in this document and the Institute reserves the right to alter, delete or add additional terms in the grant agreement between the successful Applicants and the Institute.

The Institute reserves the right to accept or reject any or all applications at its discretion and to negotiate the terms of the specific grant agreement with Applicants.

The Institute, at its sole discretion, may change the timeline of the application process.

## Section 10 Other

### ***Liability and Indemnity***

Each Applicant pursuant to this Program acknowledges and agrees in responding to the Program announcement that the Applicant shall have no claim against the Institute, and its respective representatives, related companies or affiliates, should such Program response be unsuccessful for any reason. Each Applicant hereby remises and releases the Institute, its representatives and affiliates, from any cause of action, complaint, or claim in connection with the RFA process and its outcome.

The Institute's role in grants awarded pursuant to this Program is that of a funder. The Institute is not the sponsor of funded projects. As such, the Institute will not assume any liability associated with funded projects and each Applicant who is ultimately awarded a grant pursuant to this Program releases the Institute from any and all liability with respect thereto and further indemnifies the Institute, and its respective representatives and affiliates, from any claim or loss whatsoever associated with the applicable grant.

### ***Intellectual Property Policy and Intellectual Property Agreements among Collaborators***

The Institute acknowledges that any intellectual property ("IP") that arises from research funded through this Program, including discoveries, is not the property of the Institute.

The Institute requires that researchers and collaborators agree on any material IP issues prior to submission of a Proposal.

### ***Publication and Sharing Policy***

Work funded by the Institute must be published as rapidly as possible in the open access scientific literature or other forms of publication that are readily available to the research community. Such publication should be consistent with high standards of scientific excellence and rigor, and provide sufficient detail so the research community can benefit from the findings from or in connection with the funded project.

A lay person abstract of the research proposal must be submitted prior to funding. A lay person abstract of the research results must also be submitted no later than 2 months from the date of grant expiration. These abstracts may be made available to the public by the Institute.



Any presentation, releases, papers, interviews, publication or other forms of communication dealing with the awarded project or the results from the awarded project must acknowledge the funding provided by the Institute, in a manner proportionate to the contribution of the Institute. Any other use of the Institute's intellectual property, including its name, logo or trademark requires prior written permission of the Institute.

All tools or reagents (i) funded by and (ii) that result from funded projects must be made readily available to the research community either freely or at reasonable prices within 3 months of study completion. If sharing of such tools or reagents will jeopardize the Applicant's right to secure patents or copyrights necessary to protect the Applicant's ownership, then they should be made available as soon these rights have been secured. The Institute may let the public know of these tools or reagents so other researchers know they are available.

The Institute encourages sharing of data and making raw data publicly available where possible.

The Institute requires any clinical trial awarded under any of its funding programs be registered with [clinicaltrials.gov](http://clinicaltrials.gov), [PDTrials.org](http://PDTrials.org), or other appropriate public registry. Note that the Transformational Research program excludes projects conducting a clinical trial or clinical trial sub-study.