Medicine by Design

Pivotal Experiment Fund

# GOAL

Medicine by Design’s vision is to harness the power of regenerative medicine to enable people around the world to live longer, healthier lives. Enabled by cutting-edge technologies such as single-cell analysis, computational modelling and synthetic biology, regenerative medicine aims to reverse disease by repairing or regenerating damaged cells, tissues and organs. It seeks to devise new approaches that will change the way disease is identified, treated and eventually cured, and block the processes that trigger disease. Increasingly, regenerative medicine uses a stem cell lens to identify critical interactions or defects that prepare the ground for disease, paving the way for new approaches to preventing disease before it starts.

The goal of Medicine by Design’s Pivotal Experiment Fund (PEF) is to build a robust, pre-clinical pipeline of regenerative medicine-based therapies, enabling technologies and ventures that have strong potential for clinical, socio-economic and/or commercial impact. A pivotal experiment is one that enables a “go/no go” decision on the merits of a product concept based on outcomes that drive a value inflection in the development plan

# PURPOSE

The PEF will bridge a critical gap within the innovation ecosystem by supporting early-stage regenerative medicine research discoveries to a point where follow-on investment from third parties is attractive. The PEF is a strategic and competitive program intended to advance translation of select Medicine by Design Cycle 2 projects.

**The PEF will:**

* Provide project funding of up to $250,000 over 12 to 18 months. In exceptional cases, consideration may be given to a larger investment, subject to discussions with Medicine by Design during the proposal development process.
* Support the generation of pivotal proof-of-concept and/or validation data, enabling the first steps towards demonstrating therapeutic potential and/or technological utility.
* Support projects that are defined by clear, milestone-driven (clear go/no go) decision points over a 12- to 18-month period.
* Encourage projects that leverage co-investment from ecosystem partners and programs (e.g. Connaught Innovation Award, UHN Innovation Acceleration Fund, CCRM, TIAP/LAB150, industry partners etc.).

**Project activities** can include but are not limited to:

* Generation of pre-clinical, pivotal *in vivo* efficacy and/or proof-of-concept data.
* Demonstration of a technology for a regenerative medicine application.
* Critical, pivotal experiments to support the early stages of target validation.
* Drug screening on a validated target.
* Demonstrating feasibility of cell manufacturing scale-up.

# ELIGIBILITY

Applicants

* **PEF lead PIs must be a member of a current Medicine by Design Cycle 2 project team. Cycle 2 co-PIs are eligible to lead PEF proposals.**
* All investigators must have a faculty appointment at the University of Toronto.
* Applications must be directly linked to the primary aim of the Cycle 2 project.
* All funds are to be spent at the academic institution(s) except as noted under expert services.
* NOTE: Only two PEF applications per Cycle 2 project may be submitted in each submission round. Applicants may re-submit their proposal in a later round if they are not successful in their first submission. Re-submitted applications will be counted as one of the two allotted applications for each submission round.

Project stage

* **Intellectual Property**
  + The PEF supports projects for which intellectual property (IP) has been or is in the process of being filed at the technology transfer offices (TTO) of U of T or its affiliated hospitals.
  + In exceptional cases, Medicine by Design may consider PEF applications for technologies that have not yet been disclosed; however, applicants are expected to engage with their TTO in conjunction with the preparation of a PEF application to demonstrate a clear plan for filing IP.
* **Technology Readiness Level (TRL)**
  + Product concepts are expected to be at a TRL 3 (“Identification and Characterization of a Product Candidate”) as defined by the NIH TRL Scale (Appendix A).
  + Project plans are expected to advance product concepts to a TRL 4 (“Optimization and Initial Demonstration of Safety and Efficacy”) or higher).

Project Expenditures

* Eligible expenses include research expenses (salaries and benefits, equipment, materials and supplies) and expert services that are directly related to PEF project aims and deliverables.
* Funds are to be spent in accordance with the specific project approved budget and within Canada First Research Excellence Fund (CFREF) guidelines. Please see CFREF Financial Administration Guide at <http://www.cfref-apogee.gc.ca/program-programme/admin_guide-guide_administration-eng.aspx>.
* **Expert services** could include:
  + Key Opinion Leader consultations.
  + The services of a contract research organization, in cases where there is a demonstrated need for further support beyond what is feasible in an academic research lab. (e.g. independent data validation).
  + Regulatory expertise to inform pre-clinical strategy.
  + **Notes about expert services:** 
    - Budget for expert services may be capped. Applicants are encouraged to discuss expert service needs with Medicine by Design during the proposal development process.
    - Expert services cannot be performed by companies where team members are founders and/or shareholders.
    - Expert services such as those typically provided by TTOs (e.g. patenting costs, market research, standard due diligence etc.) will not be considered as eligible expenses.
    - Requested expert services should not duplicate previous efforts by partner organizations (e.g. CCRM) but may build upon those efforts.
    - Procurement of expert services must comply with the procurement guidelines of the institution where the granted PEF funds are held.

**Ineligible Expenditures**

* Costs not directly associated with PEF project aims and deliverables.
* Costs related to proposal development (including staff costs);
* Capital costs (e.g. equipment over $2,500, land, buildings, vehicles, etc.).
* Travel and publication costs

# APPLICATION PROCESS

Medicine by Design plans to run three PEF funding rounds, with submissions in March 2021, August 2021 and January 2022. Each round will commence with an expression of interest (EOI). Successful EOIs will be invited to submit full PEF proposals. Submission deadlines are listed in the table below.

**For each funding round only two PEF proposals may be submitted by each Cycle 2 team.** Applicants may re-submit their proposal in a later round if they are not successful in their first submission. Re-submitted applications will be counted as one of the two allotted applications for each submission round.

**Review Process**

PEF proposals will be reviewed by Medicine by Design’s Pivotal Experiment Fund Investment Committee. Please reference Appendix B for a list of committee members and their affiliations.

Teams that are invited to submit a full proposal will also be required to deliver a 10-minute pitch presentation, followed by Q&A, to the Committee.

The planned dates for each funding round are outlined in the table below. Please note that these dates may be subject to change.



# EQUITY, DIVERSITY, AND INCLUSION

The University of Toronto recognizes that diversity is essential to the creation of a vibrant intellectual community that allows our researchers to maximize their creativity and their contributions. Medicine by Design is therefore strongly committed to diversity in research and especially welcomes applications that engage racialized persons/persons of colour, women, Indigenous/Aboriginal Peoples of North America, persons with disabilities, LGBTQ+ persons, and others who may contribute to the further diversification of ideas.

**All applicants (both lead and co-PIs) are required to answer a self-identification survey if they have not already submitted one as part of another Medicine by Design award application.**In completing this survey, applicants may voluntarily self-identify in all applicable groups, or they may log a response indicating that they decline the survey. Self-identification data is important to the University’s ability to accurately identify barriers to inclusion and to develop strategies to eliminate these barriers. Any information directly related to you is confidential and cannot be accessed by the reviewers or the Medicine by Design team. *Aggregated* results as of the closing of this posting may be shared with only a small number of designated senior administrators on a need-to-know basis.

Medicine by Design

Pivotal Experiment Fund

# FULL PROPOSAL

# INSTRUCTIONS

Submission Deadlines:

**Round 1 – April 22, 2021**

**Round 2 – September 30, 2021**

**Round 3 – March 17, 2022**

Required submission components:

|  |  |
| --- | --- |
| Section | Submission method |
| 1. Application Package | Email to [awards.mbd@utoronto.ca](mailto:awards.mbd@utoronto.ca) |
| 1. My Research Application (MRA) | <http://aws.utoronto.ca/services/my-research-mr/> |
| 1. Self-identification surveys for new team members | Email to [amk.gill@utoronto.ca](mailto:amk.gill@utoronto.ca) |

1. **APPLICATION PACKAGE**

Email the completed application package to [awards.mbd@utoronto.ca](mailto:awards.mbd@utoronto.ca)by**5 pm on the submission deadline listed above.** Notification of receipt will be sent within one business day.

The application package contains 3 documents:

1. **Proposal**
   * Complete the attached Microsoft Word template and convert to a PDF document for submission
   * Formatting requirements: Arial size 11 font, with 1-inch (2.54 cm) margins, single spacing.

1. **CV document**
   * Attach a **single PDF file with CVs** of all investigators requesting funding from Medicine by Design.
   * CVs can be in any format (e.g. CIHR Biosketch), but each CV must be less than 8 pages.

1. **Budget**

* Complete the provided budget Excel spreadsheet, and submit in Excel format.

1. **MY RESEARCH APPLICATION (MRA)**

The lead PI on the RFA must register in My Research Application (MRA) at <http://aws.utoronto.ca/services/my-research-mr/> and submit the RFA document and budget sheet via this portal, in addition to sending them directly to Medicine by Design, by the deadline above. This will help Medicine by Design with the reporting/tracking to CFREF and ensures that the correct departmental or divisional approvals are in place. If you do not already have an account with MRA, please contact RAISE Help at 416-946-5000 or [raise@utoronto.ca](mailto: raise@utoronto.ca) as soon as possible to initiate this process. The program name in MRA for this competition is “MbD Pivotal Experiment Fund”.

1. **SELF- IDENTIFICATION SURVEYS**

 All applicants (both lead and co-PIs) who did not submit a survey at the EOI stage are required to answer a **self-identification survey**.

Please fill out the survey found at this link (<https://mbd.utoronto.ca/wp-content/uploads/2020/07/MbD_Self-ID_Form_July2020.pdf>) and either use the button in the form, or **send by email to Andrea Gill** ([amk.gill@utoronto.ca](mailto:amk.gill@utoronto.ca)), Research Equity and Diversity Strategist Research Services Office (RSO), Division of the Vice-President, Research and Innovation. **Do not copy** Medicine by Design staff or send this form as part of your application package**.**

In completing this survey, applicants may voluntarily self-identify in all applicable groups, or they may log a response indicating that they decline the survey. Self-identification data is important to the University’s ability to accurately identify barriers to inclusion and to develop strategies to eliminate these barriers. Any information directly related to you is confidential and cannot be accessed by the reviewers or the Medicine by Design team. *Aggregated* results as of the closing of this posting may be shared with only a small number of designated senior administrators on a need-to-know basis.

Medicine by Design

Pivotal Experiment Fund

FULL PROPOSAL

**Cycle 2 Project Title:**

**Pivotal Experiment Fund Project title:**

**Lead PI name:**

**Co-PI name(s):**

# EXECUTIVE SUMMARY

Provide a summary of your product concept and pivotal experiment plan in lay terms. For successful proposals, this summary may be used in press releases/website content.

*(Maximum 1/2 page)*

# SECTION 1: PRODUCT CONCEPT

*(Maximum 3 pages)*

* 1. **Product Concept**

Describe the product concept (technology, therapeutic, or service) that will result from this project.

* 1. **Unmet Need**

What unmet need will this product address? Who will be the end-user of this product?

Outline the current standard of care or technology gold standard.

What is the projected market size for the envisioned product concept?

If you have an available market analysis, please include this as an attachment (recommended but not required).

* 1. **Competitive Landscape**

Describe the competitive landscape for the product concept. Are there current technologies in existence or in development which meet the same need as the proposed product?

If you have an available competitive landscape analysis, please include this as an attachment (recommended but not required).

* 1. **Value Proposition**

Describe the unique features of the product concept compared to the competitive landscape. How does the product concept improve upon the current standard of care or currently available technologies?

Describe the potential for health and/or socio-economic benefit.

* 1. **IP status**

What is the IP?

Has the IP been published, or otherwise publicly disclosed?

Has IP been protected? If yes, provide details of the disclosure/ /patent filing. Contact your TTO for relevant details.

# SECTION 2: SUMMARY OF KEY RESULTS

*(Maximum 4 pages, including figures and tables)*

1. Summarize the key experimental results that have provided evidence that the product concept will behave as predicted and provide data in support of key results. For technologies that are new to regenerative medicine, include preliminary proof of concept data for the regenerative medicine application.
2. What is the current Technology Readiness Level (TRL)? Provide a rationale for the current TRL.  *The NIH TRL Scale can be found in Appendix B.*
3. Describe the status of engagement with institutional TTOs, local accelerators (e.g. CCRM, TIAP) and/or industry partners.

# SECTION 3: DETAILED PIVOTAL EXPERIMENT PLAN

*(Maximum 4 pages)*

1. Describe the proposed pivotal experiment plan. Clearly define the scope and deliverables of the project.

Each pivotal experiment should include a clear aim, approach and desired outcome(s). Include an explanation of how the proposed experiments will advance product development and increase the TRL.

Describe how sex and/or gender considerations will be integrated into your proposed research or explain why sex and/or gender are not applicable to your proposed research.

1. In the table below, list out the key milestones for this project. These milestones will act as checkpoints to chart progress throughout the course of the project. Milestones should be specific and measurable.

Projects may run for a maximum of 18 months, with milestones completed within this timeframe.

|  |  |  |
| --- | --- | --- |
| **Milestone** | **Start date** | **Completion date** |
|  |  |  |
|  |  |  |
|  |  |  |
|  |  |  |

1. Outline why project milestones listed above are critical for de-risking the technology.
2. Identify any risks associated with the experimental plan that may arise over the course of this project, and planned mitigation strategies.
3. What is the proposed involvement of partners or external service providers (if any) in advancing the pivotal experiment plan?

# SECTION 4: NEXT STEPS AFTER PIVOTAL EXPERIMENT FUND

*(maximum 1 page)*

1. Once the proposed Pivotal Experiment Fund project has been completed, what are the next steps required for the product concept to reach the clinic and/or market (e.g. further pre-commercial development, license technology, company formation).
2. Outline any of the activities in part (a) that will happen concurrently with the Pivotal Experiment Fund project.

# SECTION 5: TEAM

*(maximum 1 page, excluding tables)*

1. Outline why your team is well positioned to effectively execute this project and describe the roles and responsibilities of each team member. Identifying a project manager is recommended.
2. Highlight equity, diversity, and inclusion (EDI) considerations that were relevant to team assembly.  Describe processes used to engage diverse team members, including those from under-represented groups, and/or describe how the team incorporates expertise in EDI. Please **do not**disclose demographic information about team members.
3. In the tables below, list all the team members involved in this project

*Note: Team members may have evolved from those identified in the EOI.*

LEAD PRINCIPAL INVESTIGATOR:

|  |  |
| --- | --- |
| Lead PI Name: | E-mail Address: |
| Department & Institution: | |

CO-PRINCIPAL INVESTIGATORS:

Please add rows as needed.

|  |  |  |
| --- | --- | --- |
| Name | Position & Institution | Email |
| 1. |  |  |
| 2. |  |  |

HIGHLY QUALIFIED PERSONNEL

Please add rows as needed.

|  |  |  |  |
| --- | --- | --- | --- |
| Name | Position & Institution | Supervisor | Email |
| 1. |  |  |  |
| 2. |  |  |  |

## SUPPORTING DOCUMENTATION

1. Attach a single PDF file with CVs for all investigators requesting funding from Medicine by Design. CVs can be in any format (e.g. CIHR Biosketch), but each CV must be less than 8 pages.
2. All applicants (both lead and co-PIs) **who did not submit a survey at the EOI stage** are required to answer a **self-identification survey**.

Please fill out the survey found at this link (<https://mbd.utoronto.ca/wp-content/uploads/2020/07/MbD_Self-ID_Form_July2020.pdf>) and either use the button in the form, or **send by email to Andrea Gill** ([amk.gill@utoronto.ca](mailto:amk.gill@utoronto.ca)), Research Equity and Diversity Strategist Research Services Office (RSO), Division of the Vice-President, Research and Innovation. **Do not copy** Medicine by Design staff or send this form as part of your application package**.**

In completing this survey, applicants may voluntarily self-identify in all applicable groups, or they may log a response indicating that they decline the survey. Self-identification data is important to the University’s ability to accurately identify barriers to inclusion and to develop strategies to eliminate these barriers. Any information directly related to you is confidential and cannot be accessed by the reviewers or the Medicine by Design team. *Aggregated* results as of the closing of this posting may be shared with only a small number of designated senior administrators on a need-to-know basis.

# SECTION 6: BUDGET

*(no page limit)*

1. How much funding is required, and for what time period?

*Note: Projects should be a maximum of 18 months in length. Typical proposals will be awarded up to $250,000. In exceptional cases, consideration may be given to a larger investment, subject to discussions with Medicine by Design during the proposal development process.*

1. Complete the budget table below. Please refer to the guidelines on pages 2-3 for a detailed list of eligible expenses.

|  |  |
| --- | --- |
| **BUDGET ITEM** | **AMOUNT (CAD)** |
| **RESEARCH EXPENSES** | |
| Salaries and benefits (students, technicians) | $ |
| Research Equipment | $ |
| Materials and Supplies | $ |
| **EXPERT SERVICES** | |
| Key Opinion Leader consultations | $ |
| Contract Research Organization (e.g. design and execution of relevant experiments required to obtain independent validation, scale-up and/or demonstration) | $ |
| Regulatory expertise to inform pre-clinical strategy | $ |
| Other:  \* applicants are encouraged to discuss eligibility of expert services with Medicine by Design prior to submission | $ |
| **TOTAL** | $ |

***Notes about expert services:***

* ***Exclusions****:* 
  + *Expert services such as those typically provided by TTOs (e.g. patenting costs, market research etc.) will not be considered as eligible expenses. Requested services should not duplicate previous efforts by other partner organizations such as CCRM, but may build upon those efforts;*
  + *Expert services cannot be performed by companies where team members are founders or shareholders.*
* *The services of a contract research organization are allowed in cases where there is a demonstrated need for further support beyond the capabilities an academic lab.*
* *Budget for expert services may be capped. Applicants are encouraged to discuss expert service needs with Medicine by Design during the proposal development process*
* *Procurement of expert services will be subject to the procurement guidelines of the institution where the granted PEF funds are held.*

1. Provide a budget justification for each line item above. If you are requesting funds for expert services, explain how these services go beyond the services typically performed by TTOs or cannot be done in an academic research lab.
2. In the attached budget sheet, provide a detailed budget breakdown by project PI. Research funds cannot be held by investigators outside of U of T and its affiliated hospitals. Travel and publication costs are not eligible. Funds are to be spent in accordance with the specific project approved budget and within Canada First Research Excellence Fund (CFREF) guidelines. Please see CFREF Financial Administration Guide at <http://www.cfref-apogee.gc.ca/program-programme/admin_guide-guide_administration-eng.aspx>.

# Pivotal Experiment Fund Full Proposal – Considerations for Review

**SECTION 1: PRODUCT CONCEPT**

* Product concept is well defined and addresses an unmet need
* Value proposition is compelling
  + Market opportunity is substantial
  + There are differentiating features which make the product concept innovative and novel compared to the competitive landscape.
  + Health and/or socio-economic benefit(s) of the proposed product concept is well defined
* IP exists and has been protected
  + If IP has not been protected, there is a clear plan in place for protection.

**SECTION 2: SUMMARY OF KEY RESULTS**

* Applicant has provided sufficient evidence that the product concept is at minimum a TRL 3.
* Proof of concept data is compelling and supports intended functionality/performance
* Preliminary proof-of-concept data is shown for technologies new to regenerative medicine applications.

**SECTION 3: DETAILED PIVOTAL EXPERIMENT PLAN**

* PE plan is appropriately designed to advance the product concept to a higher TRL level
  + Pivotal experiments are defined by clear go-no go milestones that are achievable within the defined timeline
* Desired outcomes will de-risk the technology and have strong potential to attract investment interest from third parties
* Potential challenges of the plan have been presented and planned mitigation strategies are appropriate.

**SECTION 4: NEXT STEPS AFTER PIVOTAL EXPERIMENT FUND**

* There is clear potential for commercialization and uptake
  + Applicant has engaged with institutional TTO(s) as well as prospective commercialization partners
* There is forward-looking plan, with a clear idea of next steps for technology development
* Where possible, activities are planned to progress concurrently with the PEF project

**SECTION 5: BUDGET**

* Proposed budget allocation is reflective of pivotal experiment plan and well justified
* Requested expert services go beyond what is offered by the institutional TTOs. Services do not duplicate previous efforts.

**SECTION 6: TEAM**

* Project team has the required expertise to tackle the aims of the PE plan
* Roles and responsibilities of team members are clearly defined
* Project manager has been identified
* Project teams took equity, diversity and inclusion (EDI) into consideration when recruiting members

Appendix A: Pivotal Experiment Fund Investment Committee Members

|  |  |
| --- | --- |
| Name | Affiliation |
| **Committee Chair:** Michael Sefton | Executive Director, Medicine by Design |
| Bharat Srinivasa | Co-Founder and Principal, Amplitude Ventures |
| Cynthia Lavoie | President and Chief Investment Officer, CCRM Enterprises Inc.  Co-Founder & Managing Director, AlloSteRx Captial |
| Jacques Sayegh | CEO and Managing Partner, Samuel Capital Partners |
| Jamie Stiff | Managing Director, Genesys Capital |
| Matthew Mistry | Market and Equity Analyst, CCRM Enterprises Ltd. |
| Parimal Nathwani | CEO, Toronto Innovation Acceleration Partners (TIAP) |