Mass General Brigham

Gene & Cell Therapy Institute

Award Program Guidelines FY2024

February 2024

Contents

Background and Purpose

The Gene and Cell therapy Institute (GCTI) Spark grants aim to accelerate the translation of gene and cell therapies and improve patient health. The program will enhance the innovative potential of the Mass General Brigham GCT research community, stimulate new inventive concepts, identify areas of commercially significant scientific strength, and accelerate commercialization of Mass General Brigham intellectual assets. For fiscal year 2024, the GCTI funding opportunity is open to all clinical areas and technology types that include cell or gene therapies. Multi-investigator collaborations are encouraged. The GCTI program will fund projects that can demonstrate tangible advancements towards clinical applications as well as commercialization outcomes, including licensing, commercial partnerships, or new spinoff companies.

For purposes of this RFA, the following technology categories are considered:

- Cell Therapy is defined as the delivery of human cells to replace or repair damaged tissue and/or cells. Many different types of cells may be used as part of a therapy or treatment for a variety of diseases and conditions.[1] Different types of cells are used for therapeutic purposes, including immune cells (e.g., T and NK cells), hematopoietic stem cells (HSC), embryonic stem cells (ESC), induced pluripotent stem cells (iPSC), and mesenchymal stem cells (MSC). Cell therapies can be modified (e.g., by a viral vector) or non-modified.

- Gene Therapy is broadly defined as the "use of genetic material in the treatment or prevention of disease. The transferred genetic material changes how a single protein or group of proteins is produced by the targeted cell. Gene therapy can be used to reduce levels of a disease-causing version of a protein, increase production of disease-fighting proteins, or to produce new/modified proteins"[2] via gene addition, replacement, inhibition, editing, or cell elimination. Either viral or non-viral vectors can be used as delivery vehicles.

- Platform Technology is a technology that enables the clinical translation of gene and cell therapies and can be used as a base upon which other applications, processes, or technologies are developed. Platform technologies in the gene and cell therapy space include genome editing, gene delivery modalities, manufacturing improvements, discovery techniques and processes.

Key Dates and Deadlines

- Pre-proposals are due no later than April 19th, 2024, 11:59pm EST
- Full proposals will be invited on June 3rd, 2024
- Full proposals are due no later than July 3rd, 2024, 11:59pm EST
- Full proposals selected for development will be announced in early September 2024
- The earliest possible start date is October 1, 2024

All pre-proposals and full proposals must be submitted through the electronic application site at GCTI Spark website.

At the applicant's request, Mass General Innovation can assist with questions related to the business opportunity.

Additional information and forms are posted at the GCTI Spark website.

Each applicant receiving an award will be expected to prepare interim progress reports and present project advancements to the GCTI Spark Advisory Committee in the following year.

Eligibility

This system-wide initiative is open to faculty members with PI privileges and a primary appointment at a Mass General Brigham affiliated institution. PIs may only submit one proposal application per cycle but may be co-PIs on multiple proposals.

Collaborators outside the Mass General Brigham community may be supported as vendors within the primary investigator's other costs. Collaborators at separate institutions within the Mass General Brigham community must submit individual budgets.

Evaluation Criteria

Pre-proposals will be evaluated by an internal team at Mass General Brigham and full proposals will be evaluated by an independent panel of academic and industry experts. ***Confidential information should not be included in the full proposal.***

Proposals will be evaluated in terms of the following criteria:

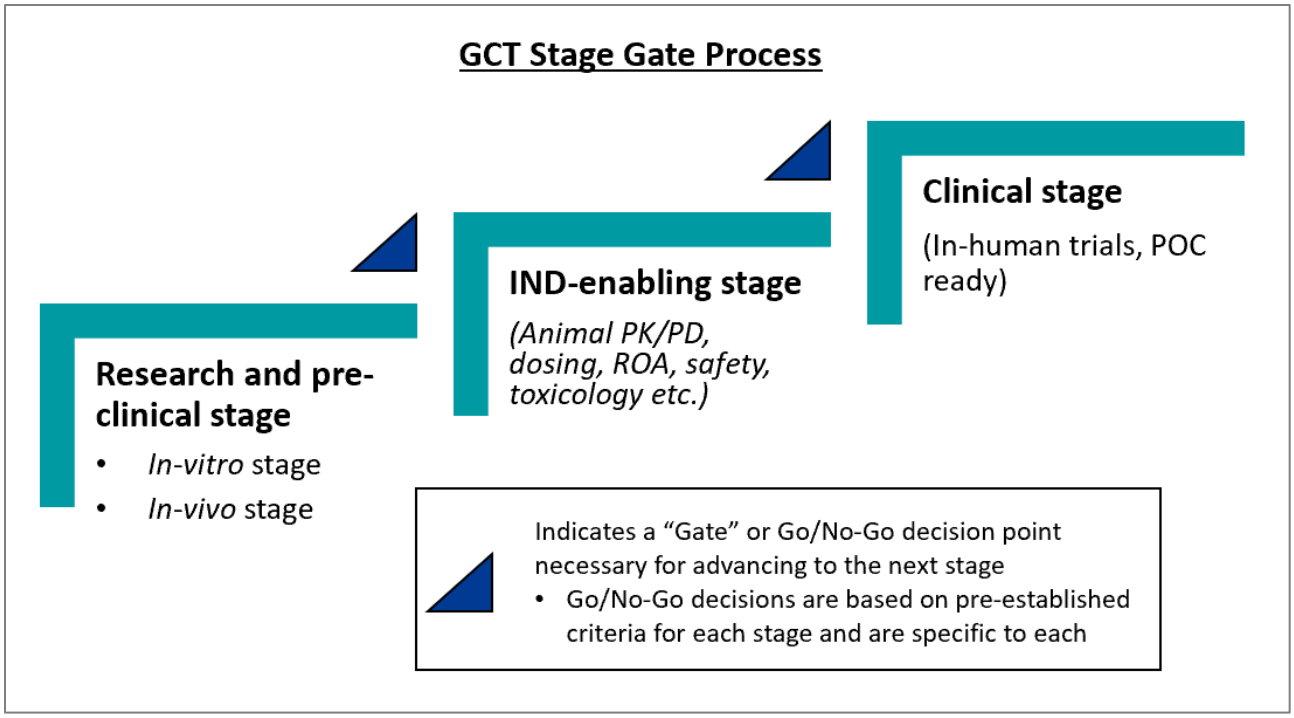
1. Leadership potential in the field of cell or gene therapies or platform technologies to support cell and gene therapies.
2. Magnitude of the stated unmet need addressed.
3. Technical quality of the proposal including definition of critical unsolved problems/issues
4. Quality and sufficiency of the team included in the proposal
5. Outcome of technical, execution and market risk assessment for the program
6. Commercial opportunity and likelihood of attracting future capital
7. Relevance of research interests
8. Ability to contribute to and advance MGB's mission and goals

Intellectual Property

Awards will be governed by the Mass General Brigham Intellectual Property Policy (http://healthcare.partners.org/OGCpolicies/IPPolicy.pdf). Each applicant invited to submit a full proposal will be required to provide a signed Intellectual Property Acknowledgment form, assigning the applicant's intellectual property rights to Mass General Brigham or the appropriate system-affiliated institution.

Award Details

- No portion of the award may be subcontracted outside the Mass General Brigham community. However, other costs for CRO or other commercial vendor services and GMP manufacturing are allowable and can be negotiated by GCTI. All vendors and consultants must agree that all intellectual property rights will be assigned to the appropriate Mass General Brigham-affiliated institution.
- Full proposals must include an itemized budget along with a budget justification that describes the basis for all estimated costs.
- Salaries must include appropriate fringe benefit costs.
- Awards may not be used for capital equipment, travel expenses, subcontract expenses, or patent expenses.
- Award funding will be released in accordance with tranche plans developed by the PI in coordination with the GCTI Program Manager. Tranche plans will be requested once full proposals are selected for award. Tranche funding will be utilized to support GCTI awards in accordance with guidance provided by the GCTI Program Manager and progress achieved.
- Each stage in translational and clinical research has different costs depending on the technology developed. The following section outlines current thinking on standard academic costs for progressing technologies. The following information provides amounts up to which funds may be requested in accordance with the technology development stage. Indirect costs at 20% must be included in the budget as part of the funding limits provided below.



The above GCT stage gate process is intended to be implemented through Spark funding program by aiding development of projects in any of these stages.

Tiers of research support:

1. ***Research and Pre-clinical Discovery Stage*** refers to concept development research, including conducting *in-vitro* or preliminary *in-vivo* experiments (e.g., efficacy, mechanism of action) for candidate selection and to validate early-stage research ideas and hypotheses.
2. ***IND-enabling Stage*** refers to drug/platform development studies to predict safety concerns and estimate safe and efficacious starting doses for clinical trials. Projects in this stage are seeking to define the pharmacological properties of a gene/cell therapy and typically involved one or many of the following: animal pharmacokinetic/bioanalytical data, safety pharmacology studies, genetic toxicology, route of administration, dosing, duration of treatment etc. IND-enabling studies seek to support a submission of an Investigational New Drug (IND) application to the FDA.
3. ***Clinical stage*** refers to advanced stage projects that have initiated or are ready to initiate in-human trials and proof of concept. Assume treatment of 10 to 15 patients, depending on statistical significance. Any prior retrospective product and/or disease history studies can also be included as part of the Clinical Protocol draft and IND application.

Frequently Asked Questions (FAQs)

1. What is the funding period of the Spark grant?

The spark grant awardee will be funded for a period of one year. During this time, their progress will be evaluated by a board of reviewers on a regular basis (the GCTI Spark Advisory Committee), and the awardees will receive guidance periodically during the funding period on how to advance their technology most efficiently to the clinic and how to position their technology for further commercialization.

2. Can an investigator submit multiple Spark pre-proposal applications?

Investigators may only submit one pre-proposal application but may be co-investigators on multiple applications.

3. Will everyone be notified of the results?

Yes, everyone who submitted a proposal through the application process will receive communication on whether they were selected for full proposals. We will further send follow-up communications with those who were selected for full proposal submission until award announcements.

4. Can additional materials be submitted as attachments if there are formatting issues?

We highly recommend applicants to use the IDG online form for all submissions to ensure documents are intact and received on time. However, in exceptional circumstances, you can submit attachments to mgbGCTI@mgb.org and the attachments will be counted towards the given word-limit.

5. When will the results of final award be announced?

Finalists who are selected for the award will be notified in early fall through individual communication and announcements to public will be made in annual GCTI symposium.

6. Does the budget cap include indirect costs/overhead?

Yes, the budget cap includes an overhead/IDC of 20%.

7. Will sub-award be allowed through Spark?

No this will not be allowed.

Additional Information

Questions related to GCTI grants policy and procedures should be directed to Nandhitha Uma Naresh at mgbGCTI@mgb.org . Questions related to your technology commercialization strategy should be directed to your Innovation Licensing Manager at 857-307-2400.

References:
[1] Association for the Advancement of Blood & Biotherapies
FACTS ABOUT CELLULAR THERAPIES (AABB.ORG)
[2] American Society of Gene + Cell Therapy
GENE & CELL THERAPY FAQS | ASGCT - AMERICAN SOCIETY OF GENE & CELL THERAPY | ASGCT - AMERICAN SOCIETY OF GENE & CELL THERAPY