

Dr. Ralph and Marian Falk Medical Research Trust

Catalyst and Transformational Award Programs

Office Hours

April 3rd, 2023, 12 – 1 PM ET

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Health Resources in Action
Advancing Public Health and Medical Research

Agenda

- New in 2023
- Program Goals
- Research Focus
- Program Overview
- Eligibility
- Review Criteria
- Pitfalls to Avoid
- Application Tips
- Q & A



New in 2023

- Catalyst Maximum Award Amount = \$350,000
- Research Focus: Will consider proposals for investigator-initiated trails under certain conditions
- Eligibility: Updated Catalyst Invited Institutions
- Minor updates to application materials

Program Goals

- To support “**medical research to improve treatments of the past and eventually find cures for diseases for which no definite cure is known.**”
- Move insights gained from basic science into clinical practice in the near term (*moving into commercial development by the end of the Catalyst/ Transformational award lifetime - i.e., 3-5 years from Catalyst proposal*)
 - **Catalyst Research Award**
 - 1 – 2 years seed funding to lay foundation for Transformational Award.
 - Planning and development of projects, teams, tools, techniques, and management infrastructure necessary to successfully compete for a 2-3 year Transformational Award.
 - **Transformational Research Award**
 - Must have completed a successful Catalyst project.
 - Moves a healthcare innovation to the next step in commercial development.

Research Focus

- To support high-risk, high-reward projects that address critical scientific and therapeutic roadblocks.
 - Identification of **biological markers** of disease and activity progression
 - Identification of **targets** for therapeutic interventions
 - Development of **therapeutic agents** that will disrupt, arrest, or prevent the disease process

Early stage	Early preclinical development from biomarker or target identification and validation to proof of concept, up to development of a lead therapeutic
Late stage	Lead optimization through drug candidate selection and IND-enabling studies
Pre-transition	Late-stage projects that need one or more critical experiments (e.g., lead candidate testing in non-human primates) to satisfy a transition requirement (i.e., FDA IND filing, venture capital investment, etc.)
Investigator-initiated trial	Proposals for human clinical studies that are initiated, managed, and sponsored by the investigator or investigator's institution

Program Overview

Catalyst Award

Transformational Award

Award Duration:

12 – 24 months

24 – 36 months

Maximum Award Amount:
(including 10% indirect costs)

Up to \$350,000

Up to \$1,000,000

Application Deadline

Award Start

June 15th, 2023

Mid-October

Nov 30th, 2023

Nov 30th, 2024/25/26

Award Notifications

Award End



Eligibility

Catalyst Award

- [Invited Institutions](#) may nominate 2 projects each cycle

Transformational Award

- For a limited time, following completion of Catalyst Award
- Successfully achieved milestones & benchmarks

- Investigative team may involve shared leadership and multiple PIs
- Administrative PI must be full time faculty and conducting research at nominating institution
- Nominating institution must be the primary funding recipient if awarded
- PI(s) must have institutional support
- Personnel may not have funding for similar project
- One application per PI

Review Criteria

Translational Potential

- Can be transferred to clinical practice in the near term

Impact

- Addresses a critical scientific roadblock and will have high impact on patient lives if successful

Investigative Team

- Right combination of expertise and infrastructure to successfully carry out the project

Project

- Based on sound precedents
- Clear rationale
- Realistic & powered methodology
- Appropriate budget and timeline

Common Features of Successful Projects

- **High impact:** high potential to improve the lives of those suffering from a disease
- Translational in the **near term** (typically within 3-5 years)
- **Fill an unmet need** or improve upon existing products
- **Clear strategic path** to a \$1M Transformational award.
- Combination of **expertise and infrastructure** in relevant areas (i.e. pharmacology, drug delivery, clinical trials, scaling & manufacturing, commercialization, specific techniques, etc.)
- **Solid project:** Sound precedents, preliminary data, and clear rationale; feasible and sufficiently powered; alternative approaches; appropriate and realistic budget and timeline.
- **Grantsmanship:** clear language and hypothesis understandable to a general audience and appropriate use of jargon and abbreviations.

Common Pitfalls to Avoid

Translational Potential

- “The work is at an early stage and it is unclear that efficient delivery will be feasible.”
- “Although innovative, this project is in its very early stages, without clear preliminary evidence that these treatments will be superior to current therapies, and thus transformational potential is somewhat limited. Somewhat limited by the lack of mechanistic understanding”
- “There are concerns with overlap with ongoing commercialization approaches.”
- “Commercialization potential or next steps for translation are not well addressed.”
- “I would expect more work directed to preparing for IND-enabling studies to be part of Aims for Transformational Awards especially given the 3 year duration.”

Impact

- “The likelihood that the proposed therapeutic will eventually have significant clinical impact is uncertain.”
- “Proposal is somewhat limited by the lack of mechanistic understanding and lack of data demonstrating benefit over conventional therapy”
- “This seems like a ‘too safe bet’ for Falk, a project that is very likely to succeed, likely to be funded by others, and not likely to create groundbreaking headlines when the results are known.”
- “Project focuses on an ultrarare disease with very low prevalence.”

Common Pitfalls to Avoid

Experimental approach

- “The proposed technique has been used in prior models with limited success and there is no discussion of how it might eventually prove to be a useful approach.”
- “This approach does not appear sufficiently well designed to impact outcomes in the near term”
- “Because of the previous clinical trial failures in this space (that are not always well explained/understood mechanistically) the authors should spend time in assessing the clinical trial applications in parallel with the development of the lead compounds. There appear to be potential differences in efficacy that relate to myriad factors in clinical trial design that are unrelated to the pharmacological properties of the compound.”
- “One would expect local patient communities to be guiding the development of the therapeutic such that it has patient centered properties (such as, targeted to specific quality of life challenges patients want to address, pill formulations that affect # of times taken per day (and medicine half-life) or pill size, side effects that are undesirable), as well as endpoints.”
- “It is not apparent how the proposed study is different from previous studies, including those that have utilized this disease model to identify biomarkers.”
- “It not apparent how the proposed project differs from funded, on-going projects.”

Common Pitfalls to Avoid

Investigative Team

- “This grant utilizes numerous novel techniques. While innovative, it is not clear that the PI has the resources, experience or collaborators for all of the novel techniques described.”
- “lack of understanding of what is needed to move to clinical development”
- “I am concerned that the team might not have the expertise to make the project work within the timeline suggested.”

Project and Grantsmanship

- “...focusing on a single therapeutic may not be the right approach.”
- “Densely written proposal with many crowded, small figures. It is not easy to read!”
- “the grant is not hypothesis-driven and there are concerns about feasibility that limit the transformational potential.”
- “contingencies and alternative plans should be addressed”
- “lack of experimental details and methods for validation/assessment of results”
- “Much of the proposal is early stage and unlikely to be ready for a Transformational award in 2 years.”
- “It is hard to determine the precedent and rationale from the description.”

Application Tips

- Recruit the right team: Include appropriate collaborations and/or shared leadership if it benefits the project
- Be realistic (in timeline, budgets, etc.)
- Avoid jargon and abbreviations; should be understandable to scientific generalists such as venture capital investors
- Be concise and clear (make it easy to read!)
- Include contingencies
- Know the competitive landscape
- Seek feedback (internally, externally, and across disciplines)

Questions?

Catalyst Award: <https://hria.org/tmf/falkcap/>

Transformational Award: <https://hria.org/tmf/falktap/>

Contact Us: FalkAwards@hria.org