

Dr. Ralph and Marian Falk Medical Research Trust

Catalyst and Transformational Awards Programs

Office Hours

April 9, 2024



MEET OUR TEAM



Who We Are



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HRiA partners with individuals, organizations, and communities to transform the practices, policies, and systems that improve health and advance equity.



Agenda

- Program Goals
- Research Focus and Stage
- New in 2024
- Program Overview
- Eligibility
- Review Criteria
- Features of Successful Projects
- Pitfalls to Avoid
- Application Tips
- Q & A



The Falk Trust was created to support:

“medical research to improve treatments of the past and eventually find cures for diseases for which no definite cure is known.”

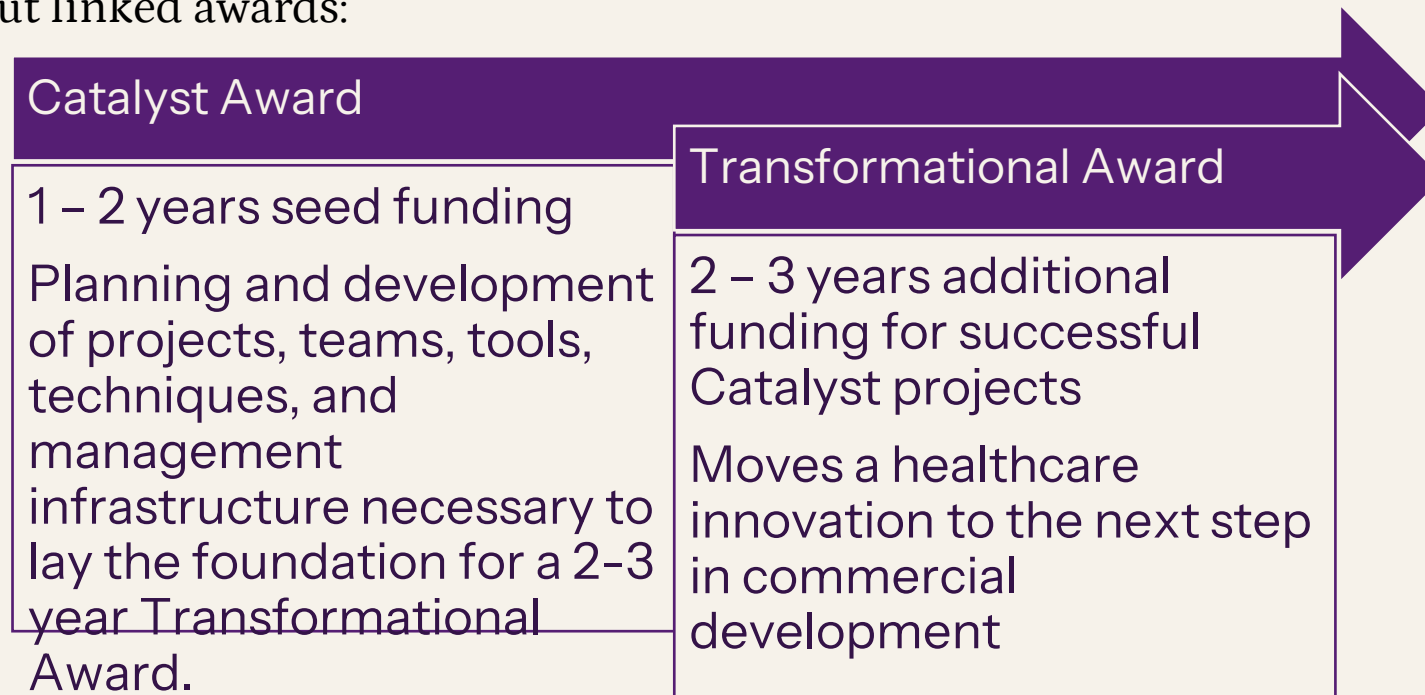


Program Goals

To move insights gained from basic science into clinical practice and support high-risk, high-reward projects that address critical scientific and therapeutic roadblocks.

If successful, these projects will have high impact outcomes that open new avenues for treating, curing, and improving the lives of individuals suffering from disease.

There are two separate but linked awards:



Research Focus and Stage

Principal Areas of Focus:

1. Identification of **biological markers** of disease and activity progression
2. Identification of **targets** for therapeutic interventions
3. 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

New in 2024

Only minor program changes:

- Commercialization potential: Applicant's should address current or future plans for licensing of technology if applicable.
- Investigator-initiated trials: Applicants should explicitly state the status of, or plan for obtaining FDA and/or IRB approvals
- Clarification on the eligibility window for Transformational applicants.

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 18th, 2024

Mid-October

Nov 30th, 2024

Nov 30th, 25/26/27

Award Notifications

Award End



Eligibility

Catalyst Award

Invited Institutions may nominate 2 projects each cycle

Transformational Award

Applicants may apply during the final year of their Catalyst Award or the year following. Can only apply once.

Must have 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 and clear rationale
- Feasible, 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 or the next step of commercial development.
- 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

- Impact/critical need are lacking
- Project is heavily mechanistic and far from clinical practice
- Unclear path forward (to Transformational Award and beyond to clinical practice)
- Idea lacks innovation (e.g. therapeutic in a competitive commercial space without a plan for differentiation)
- Lacking the correct expertise
- Lacking in preliminary data or alternative approaches
- Poor grantsmanship, proposals include much jargon and are not understandable to a general audience.

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)

Example Reviewer Feedback

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.”

Example Reviewer Feedback

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.”

Example Reviewer Feedback

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.”

Questions?

Contact Us:

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Catalyst Award:

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

Transformational Award:

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



View the Office Hours Recording
[HERE](#)

