

**Office of Discovery and Translation (ODAT)
2017 Translational Grant Program
Targeted Request for Applications in Rare Disorders**

PROGRAM OVERVIEW

The purpose of this funding program is to help drive the highest quality early stage translational research through the complex process of translating basic science discoveries into patient benefit. The overarching goal is to positively impact human health in Minnesota and the nation.

In 2017, this funding opportunity is targeted to rare diseases. In the USA, a rare disease or disorder is defined as a condition that affects fewer than 200,000 Americans at any given time. According to the National Center for Accelerating Translational Sciences (NCATS), less than 5 percent of rare diseases have a treatment. The goal of this year’s funding opportunity is to support the development of diagnostics and treatments for rare diseases. More information about rare diseases and a list of conditions classified as rare diseases can be found at Orphanet: <http://www.orpha.net/consor/cgi-bin/index.php>.

This funding program is intended to identify and fund early-stage projects in which the primary goal is to eventually develop a new therapeutic, diagnostic, medical device, or treatment approach. This program supports projects that may not yet have a defined product or treatment approach, but have transitioned past the point of researching the medical problem to developing a solution to an identified medical need. This program also supports the development of enabling technologies (e.g. novel testing or predictive models) to be used in the development of new therapeutics, medical devices, diagnostics, and treatment approaches.

For each funded project, a Project Development Team will be established with the appropriate expertise to determine critical project milestones, identify key gaps, and strengthen the likelihood for progress toward eventual development into a new product or treatment approach.

Submission of a brief letter of intent (LOI) is required. The purpose of the LOI is to confirm applicant eligibility, ensure that the project is at the appropriate stage to fit with the purpose of this funding mechanism, and enable the identification of reviewers with applicable expertise.

The CTSI anticipates funding up to 2 projects in 2017. Each award will be for up to \$50,000 direct costs for one year. No indirect costs are allowed.

SUBMISSION, REVIEW, AND AWARD DATES

LOI submissions due date	April 5, 2017 (5:00 p.m.)
Invitations to submit full proposals issued	On or before April 24, 2017
Full proposal due date	May 31, 2017 (5:00 p.m.)
Review period	June-July, 2017
Awards announced	August 2017
Anticipated award start date	October 2, 2017

INVESTIGATOR ELIGIBILITY

1. U.S. citizens, permanent residents, and non-permanent residents are eligible.
2. The principal investigator (PI) must have a faculty appointment at the University of Minnesota at the time of grant submission. Eligibility includes affiliated faculty at the VA, HCMC, Regions Hospital, and the Children's Hospitals of Minnesota. Faculty members holding adjunct appointments are ineligible.
3. The PI must hold a doctorate degree (e.g., MD, PhD, DVM, DO, DDS, PharmD).

PROJECT ELIGIBILITY

Eligible project types include but are not limited to:

1. Identification of a small molecule or biologic with potential for therapeutic effect
2. Moving from concept to initial design of a new medical device
3. Definition of differential expression of specific biomarkers for diagnosis or treatment purposes
4. Development of new models of human disease that could be used in the testing of therapeutics, diagnostics, devices or treatment approaches specific for rare diseases

Note: Clinical research using human subjects is outside the scope of this funding mechanism.

APPLICATION PROCESS

Note: Both the Letter of Intent and Full Proposal will be submitted using an online form by accessing the links identified below. If you have questions about this process, please contact Jodi Fenlon Rebuffoni (fenl0003@umn.edu).

Step 1 – LOI Submission

Mandatory letters of intent (LOIs) are due on April 5, 2017 at 5 p.m. and must be submitted in an online form (see link below). The information required in the LOI includes:

1. Project title
2. PI(s) name(s) and contact information
3. Co-investigator(s) name(s)
4. What is the rare disease being addressed?
5. What is the current standard of care, including available drugs, devices and diagnostics?
6. What specific population will benefit from this new proposed product or treatment approach?
7. Brief description of the proposed future product or treatment approach
8. Summary of specific work to be supported with funding

Letters of intent must be submitted through the following link:

<https://redcap.ahc.umn.edu/surveys/?s=K79N8C7XMY>

Note: You may save and return to complete your submissions at any time using the code provided when the "Save and Return Later" option is selected. To return to the form re-click the link identified above.

Letters of intent will be evaluated to confirm eligibility of the applicant and to ensure that the project is at the appropriate stage to fit with the purpose of this funding mechanism. We will notify applicants on or before April 24, 2017 to invite submission of a full proposal. At that time, further instructions for proposal submission will be provided.

Step 2 – Proposal Submission (by invitation)

A faculty member may apply for only one grant per cycle on which he/she would be the PI or co-PI. There is no limitation to the number of grants on which a faculty member would participate as a co-

investigator or collaborator. One resubmission of an unfunded application is allowed. Details about the full proposal content and submission process will be provided to those individuals invited to submit proposals. In brief, the following information will be required in full proposals:

1. Executive summary
2. Proposed product or solution
3. Project background
4. Work Plan
5. Collaborators and other sources of support
6. Intellectual property status
7. Strategic partnerships (if applicable)
8. Project team roles and responsibilities
9. Project timeline
10. Budget overview and personnel expenses
11. Biosketches
12. Letters of collaboration (if applicable)

REVIEW PROCESS

1. LOIs will be screened for eligibility criteria by program administration. Full proposals will be invited for those LOIs meeting eligibility requirements.
2. Full proposals will undergo scientific review *and* an assessment of the translational potential of the project.
3. Applicants whose proposals receive the highest scientific and translational potential scores will then be asked to interview with a review team to address issues raised in the proposal review.
4. Applicants selected for funding will be asked to make appropriate revisions to their work plan reflecting feedback from reviewers.

EVALUATION CONSIDERATIONS

Proposals will be evaluated based on the following criteria:

1. *Medical Need and Impact*: Does the project focus on a rare disorder and have the potential to significantly improve outcomes, reduce complications of care or improve quality of life?
2. *Translational Focus*: Has the project advanced to where the focus is predominantly on developing a solution to the identified medical need rather than further research on the medical problem?
3. *Feasibility*: Are the overall strategy, methodology, and analysis well-reasoned and appropriate to accomplish the goals of the project? Is the proposed work focused on translation of a research finding rather than further research?
4. *Expertise*: Will the scientific environment in which the work will be done contribute to the probability of success? Does the PI or investigative team have the necessary expertise to conduct the proposed work?
5. *Scientific quality*: Does the project have its origins in innovative, high-quality research conducted by the PI(s)?

FUNDING PERIOD EXPECTATIONS

1. A project development team (PDT) will be assigned to each funded application to assist and facilitate progress toward achieving specific milestones. The members of the PDT will be selected based on their expertise and capability to provide constructive input on the project. An initial meeting between program administration and grantee will be held at the time the grant is funded.
2. Grantees will agree to periodic meetings with the PDT following initiation of the award. The purpose will be to monitor progress on the project, and make any mid-course corrections that reflect unexpected results or the need to modify the experimental design. Advancement of the

projects toward the development of a new product or treatment approach will be tracked following the conclusion of funding.

PROGRAM CONTACT INFORMATION

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