

## CONCEPT CLEARANCE

### ADVISORY COUNCIL FOR THE NATIONAL CENTER FOR ADVANCING TRANSLATIONAL RESEARCH

*September 2014*

#### **Innovative collaborations for the Clinical and Translational Science Award (CTSA) consortium**

##### **Division of Clinical Innovation, NCATS, NIH**

##### **Objective:**

The goal of this initiative is to stimulate collaborative research in the CTSA consortium. Building on the existing strengths at the CTSA hubs, and utilizing resources already funded by NCATS under the CTSA program, the collaborative projects under the proposed initiative will address important gaps in clinical and translational research, and will advance innovative solutions with the goal to get more treatments to more patients more quickly.

##### **Background and Specific Goals:**

Turning discoveries into clinical advances is a long and inefficient process. The proposed initiative aims to accelerate translational research in specific areas of research and at the same time to more broadly advance our understanding of translational science, the field focused on best scientific and operational methods. The focus of the innovative collaborations initiative is less on the development of technologies *de novo*, but more on the demonstration that innovative approaches can be applied to translational research problems, or can help address roadblocks. The projects should define the positive outcome, describe how it is measured, and include plans for next steps under different outcome scenarios. At project completion, the data generated should allow for a decision as to whether the approach should be abandoned in favor of alternatives, or whether it should be optimized and disseminated more widely in support of translational research. Therefore, data quality is critical, and robust experimental approaches are encouraged. When feasible, approaches might for example include the randomization or cluster randomization to different interventions that would allow for comparisons between contemporary arms or between pre- and post-intervention scenarios. Projects under this initiative are demonstration projects, and will be considered successful if they provide high-quality data on an innovative and generalizable approach so that the field moves forward. The projects are thus expected to have an impact almost regardless of their outcome by accelerating the uptake of an effective innovation, or by alternatively making it clear which approaches should be discontinued in favor of alternative investments. As appropriate, projects will include pre-defined milestones to monitor progress.

The innovative collaborations initiative will promote collaboration across the national consortium, or among CTSA investigators from a subset of sites. The initiative also encourages engagement of a combination of CTSA hubs and a variety of possible external entities and stakeholders such as federal partners (e.g., the Food and Drug Administration or others), commercial partners (e.g., biotechnology or pharmaceutical companies), or nonprofit partners (e.g., patient advocacy groups or other community groups). Projects funded under this initiative should always include NIH partners to make sure that the specific translational research problems that are being addressed are indeed of high priority to the NIH Institute overseeing research in the disease area of interest, to create co-funding opportunities, and to ensure that funded projects will have shared oversight by staff from NCATS and its NIH partner.

The following is a list of examples for potential focus areas. The examples should not limit the breadth of this initiative, which is deliberately broad in scope, and which aims to build on investigator-initiated innovative collaborations. 1) Advancing the use of telemedicine, mobile devices and remote, local enrollment and assessment of research participants so that access to research opportunities could be increased, including for example to rural or disabled populations. 2) Improving the research involvement of communities and the public, and participant recruitment to clinical research through new ways of engagement (e.g., including a broader range of stakeholders, entering into a partnership very early in the translational research process, or using innovative tools such as social media). 3) Evolving the consent process (e.g., developing best practices for the use of visual consent material, electronic consenting, highly interactive consenting, or finding ways to create shorter, more participant-friendly consent forms). 4) Testing trial designs that would accelerate the evaluation of promising novel therapeutics (e.g., adaptive designs, “n of 1” approaches, or other trial designs for rare diseases). 5) Strengthening approaches to promoting clinical research in special populations such as children (e.g., demonstrating the utility of shared resources and collaboration in multisite studies). 6) Promoting shared approaches to challenges in pre-clinical or early clinical translational research (e.g., in the area of medicinal chemistry, compound production under Good Manufacturing Practices, collaborations between human and veterinary researchers, or first-in-human studies). 7) Addressing critical roadblocks in the regulatory evaluation of novel therapeutics by identifying gaps that delay progress across therapeutic programs, and by using pre-competitive, collaborative models to generate the evidence needed in areas such as outcome measures, biomarkers, improved assays or robust natural history data. 8) Fostering innovative training methods such as online courses shared among CTSA hubs, or experiential learning opportunities that include exchanges with CTSA external partners, including but not limited to federal partners such as the FDA, or private partners such as nonprofit patient or research organizations, or the biotechnology or pharmaceutical industry.

**Current Portfolio Overview:**

The CTSA program is evolving from supporting infrastructure and unspecified pieces of all research, to a program that advances translational science, and that supports translational research by providing targeted support for specific, high-priority projects. Traditionally, funding in support of infrastructure was given to medical research centers in the U.S. Recently, NCATS has added collaborative initiatives to strengthen network capacity by providing for example central institutional review boards (IRBs), streamlined contracting and efficient participant recruitment resources. Currently, there is no mechanism for the CTSA program to promote the innovative and collaborative projects planned for the proposed initiative. Other NIH Institutes are funding innovative, multisite projects, but currently do not have a clear path to do so while utilizing the strength of the CTSA consortium. Because NCATS will collaborate with the NIH categorical Institutes and Centers for the proposed initiative, redundancies will be minimized, and synergies enhanced.

**Individuals and Groups Whose Input Was Used in Developing This Initiative:**

The initiative builds chiefly on the recommendations of the 2013 Institute of Medicine report entitled “The CTSA Program at NIH” that states: “The committee recommends that the CTSA Program establish an innovations fund to promote collaborative pilot studies and other innovative initiatives. The activities supported through this fund should engage a combination of CTSA institutions and a variety of possible entities and stakeholders.” In addition, the July 2014 CTSA PI meeting and the process leading up to it informed this initiative. Investigators had been invited to submit abstracts describing collaborative and innovative ideas; an anonymous vote was conducted to select abstracts to be presented and discussed at the July 2014 meeting; and all abstracts were made available to investigators and NCATS staff for consideration. The meeting helped gauge the landscape, interests, enthusiasm and priorities of the CTSA community.

**Alignment with NCATS Goals:**

This initiative is broadly aligned with the NCATS mission to catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

**References:**

- IOM report, <http://www.iom.edu/Reports/2013/The-CTSA-Program-at-NIH-Opportunities-for-Advancing-Clinical-and-Translational-Research.aspx>
- NCATS Advisory Council Working Group on the IOM Report: The CTSA Program at NIH, <http://www.ncats.nih.gov/files/CTSA-IOM-WG-Draft-Report.pdf>