CTSA TRANSLATIONAL PILOT STUDIES PROGRAM

http://www.buffalo.edu/ctsi/cores/translational-pilot-studies.html

Program Overview and Tips for Success in Obtaining CTSA Pilot Studies Funding

Steven J. Fliesler, PhD
Brahm H. Segal, MD
Co-Directors, Translational Pilot Studies Program

(Sept. 2018)
Steven J. Fliesler, PhD, Co-Director
SUNY Distinguished Professor
Meyer H. Riwichun Endowed Chair Professor of Ophthalmology
Vice-Chair and Director of Research, Department of Ophthalmology
Professor, Department of Biochemistry and Neuroscience Program
Jacobs School of Medicine and Biomedical Sciences
and Research Career Scientist, VAWNYHS (Buffalo VAMC)
fliesler@buffalo.edu

Brahm H. Segal, MD, Co-Director
Chief, Division of Infectious Diseases and Professor of Oncology
Roswell Park Cancer Institute (RPCI)
Professor and Vice-Chair for Faculty Development
Department of Medicine
Jacobs School of Medicine and Biomedical Sciences
brahm.segal@roswellpark.org
PURPOSE OF CTSAs:

• To support high quality *translational and clinical* research locally, and nationally (integration into the CTSA network)

• To foster innovation in methods, training, and career development.

http://www.buffaloctrcc.org
No clinical trials beyond phase IIA will be supported by this CTSA program (but T3 and T4 research encouraged)
Themes and Priorities of our CTSA

• Engage the community of Buffalo and WNY in clinical and translational research.
• Involve underrepresented groups in clinical research: workforce and research participants.
• Develop and share innovative methods.
• Look for opportunities to collaborate with the CTSA consortium and other CTSA hubs.
• Work toward reducing health disparities.
Translational Pilot Studies Program

- Pilot grants (typically up to $50K, for 1 year) to support preliminary studies that will lead to garnering of substantive extramural federal funding (e.g., NIH, etc.)
- Support for innovative studies in clinical and translational research that are directly relevant to the themes and priorities of our CTSA, and that fulfill the purpose of CTSAs (see previous slides)
- Develop new methods and technologies to solve important clinical and translational research problems
- Foster cross-disciplinary collaborations & mentoring relationships
- Create a context for innovation in clinical and translational science within the Buffalo Translational Consortium (BTC)
The University at Buffalo CTSI Translational Pilot Studies Program, with local institutional support and an award from the National Institutes of Health, provides seed money to advance promising new technologies and therapeutics from the conceptual stage to clinical trials.

Advancing translational research

The program seeks proposals for innovative, high-impact, clinical-translational science projects. Translation, as defined by the National Center for Advancing Translational Sciences (NCATS), is the process of turning observations in the laboratory, clinic or community into interventions that improve the health of individuals and populations.
Pilot Translational and Clinical Studies Program

1. **RFA Pilot Studies**
   - Submitted by BTC investigators

2. **Letters of Intent**
   - Review and invite proposals

3. **Pilot Studies Proposals**
   - Submitted by BTC investigators

4. **Scientific Peer Review**
   - PENN STATE CTSI
   - Pilot studies Executive Committee

5. **Funding Priorities**
   - CTSI Board approval

6. **Fund Proposals**
   - Tracking

7. **Follow Metrics**

**Notes:**
- BERD “Research on a Napkin”
- Recommend collaboration
- Refer to CTSI Cores
- Identify Mentor
Requests for Proposals (RFPs)
https://www.buffalo.edu/ctsi/cores/translational-pilot-studies/pilot-study-rfp.html

PROPOSALS FOR FALL 2018 SUBMISSION

(Release date: August, 2018)

Two-tiered process:
1. Letters of Intent (LOIs), 2. Full proposals (if invited)

Timeline:

<table>
<thead>
<tr>
<th>Event</th>
<th>Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Letters of intent due</td>
<td>Friday, Sept. 14, 2018</td>
</tr>
<tr>
<td>Notification of applicants</td>
<td>Monday, Sept. 24, 2018</td>
</tr>
<tr>
<td>Full proposals due</td>
<td>Friday, Dec. 14, 2018</td>
</tr>
<tr>
<td>Full proposal selection, notification of applicants</td>
<td>Mid-February 2019</td>
</tr>
<tr>
<td>Funding start date</td>
<td>Monday, April 1, 2019</td>
</tr>
</tbody>
</table>

Proposals will be reciprocally reviewed by Penn State CTSA
Submit via Email as a PDF to: CTSA-Pilot-Studies@buffalo.edu
RFA for Fall, 2018, Pilot Studies Proposals

PURPOSE-- To develop:

- Novel methodologies and technologies that will yield generalizable solutions to research problems that can be translated to a clinical setting, and

- Innovative, multidisciplinary clinical and translational research at UB and its affiliated BTC partner institutions

These Pilot Studies are funded by our Clinical and Translational Science Award (CTSA), as well as by institutional funds, with intended duration of one year, from April 1, 2019 through March 31, 2020.
This RFA is intended to solicit projects that:

- Allow clinical and translational researchers to generate preliminary data for submission of federal and private research grant applications;
- Seek to improve clinical research design, biostatistics, clinical research ethics, informatics, or regulatory pathways; and/or
- Support the design, development, and/or validation of novel, clinically applicable devices, biomarkers, or analytical methods, or other avenues (defined by the applicant) that will substantially advance clinical and translational research.
What this mechanism is **NOT** for:

- **“Bridge funding”** (in-between grants)
- Support of fundamental ("bench") research
- Studies that will not lead to a clear-cut clinically translatable outcome in the near term
Priority will be placed on applications that:

• Address healthcare disparities in under-served or under-represented populations in the Western NY region, and/or

• Pair “early stage” (NIH definition) investigators with established PIs (i.e., who have a history of substantive extramural research funding)– thereby providing a built-in mentoring system, and/or

• Promote multidisciplinary collaborations (inter-departmental, -School, -institutional (BTC partners))
Eligibility Criteria

In order to apply as PI for pilot studies funding, you MUST:

- Be a *full-time faculty member* at UB or at an affiliated BTC institution, and
- Agree, if called upon, to *serve as a reviewer* of proposals submitted for this or future rounds of pilot studies funding

**IMPORTANT:** Trainees (e.g., grad students, postdocs, residents, fellows) ARE NOT eligible to apply for CTSA pilot studies funding!

**ALSO:** While we *strongly encourage* multi-investigator collaborations, only one person can serve as the PI and submit an LOI or full application. [We track submissions by the PI’s name.]
Tips for Success in Obtaining CTSA Pilot Studies Funding

• READ AND FOLLOW THE RFA DIRECTIONS CAREFULLY!
• Make sure you are **eligible** for pilot studies funding
• Make sure your proposal is consistent with the **purpose and goals of our CTSA Translational Pilot Studies Program**

Applications will be rated using the following criteria:

• Scientific merit and innovation
• Clinical significance and translational impact
• Potential for securing substantive extramural funding
• Realistic milestones and feasibility of completion (1 yr)
• Rationale and utilization of proposed budget
• {for revised proposals} Compelling arguments that address the prior critiques (must include)
Submission of LOIs (2-page limit)

• **First page:** 1) Succinct **TITLE** for the proposal; and 2) Names, degrees, and institutional affiliations of the PI and all other investigators involved in the project. **One PI** (provide institutional email address) per project! **Clearly indicate junior vs. senior co-I’s**

• **Second page:** Provide a succinct **ABSTRACT** of the proposal, summarizing: a) what the project entails; b) a clear statement of the translational significance of the project, the expected outcomes, and the potential applications of those outcomes; and c) how the pilot study will lead to substantive extramural funding.

• **Submit as a PDF:** Arial 11pt font, single-spaced, 0.5-in. margins.

• **Email to:** CTSA-Pilot-Studies@buffalo.edu

• **Subject line:** CTSA Pilot Studies 2018 LOI
Submission of FULL PROPOSALS (by invitation ONLY)
DUE DATE:  Dec. 14, 2018, by 5:00pm
Email to:  CTSA-Pilot-Studies@buffalo.edu
Subject line:  CTSA Pilot Studies 2018 FULL PROPOSAL
Submit all documents as a single PDF
FOLLOW THE FORMAT EXPLICITLY PROVIDED in the RFA:

•  **Page 1: a)** Project TITLE;  **b)** Names/degrees/institutional affiliations of PI and all co-investigators;  **c)** Abstract (300 word max);  **d)** indicate use of (i) human subjects or (ii) vertebrate animals.

•  **Pages 2-5 (4-page limit): a)** Specific Aims;  **b)** Background and Significance (include clinical-translational relevance and potential impact on field);  **c)** Brief overview of Approach (avoid minutiae and jargon); and  **d)** Study timeline with specific milestones to be accomplished, and role of the pilot study in securing extramural funding for a larger, forward-going project
Submission of FULL PROPOSALS (cont’d): Additional Pages

- **References** (keep to minimum)
- **Budget**
  - Maximum allowable: $50,000 direct costs (no IDCs)
  - Must follow the *Uniform Guidance Cost Principles* and be allowable, reasonable, allocable, and consistent.
  - Faculty salaries/fringe and tuition are NOT allowable expenses.
- **An NIH biosketch** (current format, 5-page limit) for each Investigator. The *Personal Statement* in each should make clear the role of the Investigator in the project.
- **Appendix (if applicable):** A copy of critiques from the prior review of the submitted proposal (e.g., unfunded NIH grant application). *NOTE: Appendices other than funding agency critiques are not allowed, and will not be reviewed if attached.*
Expectations

• Pilot studies funding should result in one or more peer-reviewed publications, as well as one or more grant application submissions for *substantive* extramural *federal* funding.

• Publications resulting from CTSA pilot studies funding **must** cite our CTSA (1 UL1 TR001412, NCATS/NIH), and **must** have a PMCID number.

• CTSA-funded investigators are expected to participate, if called upon, as reviewers of CTSA proposals submitted by other investigators (except for those proposals where a COI exists).
**NEW REQUIREMENTS**

On or by date of full proposal submission:

- Submission of the project for IRB and/or IACUC approval (if the project involves human or animal studies, respectively).
- Verification of completion of human subjects education
- ClinicalTrials.gov registration status
- Conflict of Interest (COI) and UB Sponsored Projects electronic approvals
REVIEW PROCESS:
Applications will be rated using the following criteria:

a. scientific merit and innovation
b. clinical significance and translational impact on the field
c. potential for securing extramural funding
d. realistic milestones and feasibility of completion within 1 year
e. rationale and utilization of proposed budget

No clinical trials beyond phase IIA will be supported by this CTSI program.
Translational Pilot Studies
Current and Past Awards

The pilot studies review process consists of an initial letter of intent which determines invitations for full RFPs (Requests For Proposals). The invited full proposals are then reviewed according to RFP criteria by faculty in a partner Clinical and Translational Science Award (CTSA) hub.

Research implementation and participation
An operational principle for the UB Clinical and Translational Science Institute is to maximize the impact of our initiatives through a catalytic approach to the development and testing of new approaches to
Pilot Studies Vignette

Mark Ehrensberger, PhD (Assistant Professor, Biomedical Engineering) & Anthony Campagnari, PhD (Professor, Microbiology & Immunology)
“A Novel Electrical Stimulation Technology Changing the Paradigm for the Treatment of Orthopedic-Related Infections”

• $75,000 pilot studies award (2015-16 and 2016-17)*
• Subsequently garnered a $500,000 grant from the Office of Naval Research (ONR)
• Developed a novel method of preventing chronic implant infections by disrupting bacterial biofilms on surgical implants in vivo, using electrical charge stimulation

*”Two-phase” pilot project funding: $25K (2015-16) and $50K (2016-17). Five other two-phase projects also were funded.
Pilot Studies Vignette

Andrew M. Gulick, PhD (Assistant Prof., Structural Biology (UB), and Research Scientist, Hauptman Woodward Institute) & Thomas A. Russo, MD (Professor, Dept. of Medicine)
“Development of a High-Throughput Assay for Inhibitors of Aerobactin Synthesis”

- $25,000 pilot studies award (2015-16)
- Subsequently garnered a $459,125 grant from the NIH/NIAID
- Developed a high-throughput screen for compounds that disrupt aerobactin synthesis by *Klebsiella pneumoniae* (cKP), a hyper-virulent “super bug” that has demonstrated increasing resistance to antibiotics
New Processes: Pilot Studies Investigators

• Finalize *Resource Guide* with FAQs and pre-/post-award guidelines
  • NCATS pilot studies requirements
  • Budget management
  • Publications citations
  • External funding
  • “Request a CTSI Service” portal

• Formalize 6-month Progress Report from PIs:
  Aims, accomplishments, and challenges
New Processes: Pilot Studies Investigators

- Following completion of pilot study, and annually thereafter: contact regarding noteworthy milestones:
  - Grants received
  - Papers published
  - Clinical studies generated
  - Patents received
  - Relevant teaching activities
  - Websites generated
  - Honors/awards/promotions
Advancing research discoveries to improve health for all
QUESTIONS?

Steven J. Fliesler, PhD  (fliesler@buffalo.edu)
Co-Director, CTSA Translational Pilot Studies Program
•Meyer H. Riwchun Endowed Chair Professor of Ophthalmology
•Vice-Chairman and Director of Research
•Department of Ophthalmology and Ira G. Ross Eye Institute Vision Research Center
•Professor, Department of Biochemistry
•Jacobs School of Medicine and Biomedical Sciences, University at Buffalo
•Research Career Scientist, VA Western NY Healthcare System, Buffalo VAMC

Brahm H. Segal, MD (Brahm.Segal@RoswellPark.org)
Co-Director, CTSA Translational Pilot Studies Program
•Professor of Medicine / Vice Chair of Faculty Development
•Jacobs School of Medicine and Biomedical Sciences, University at Buffalo
•Professor of Oncology, Department of Medicine
•Chief, Division of Infectious Diseases
•Member, Department of Immunology
•Director of Faculty Development
•Roswell Park Cancer Institute (RPCI)

Timothy F. Murphy, MD (murphyt@buffalo.edu)
Principal Investigator, CTSA
•Director, CTRC
•Senior Assoc. Dean for Clinical and Translational Research
•Jacobs School of Medicine and Biomedical Sciences, University at Buffalo