University at Buffalo
Clinical and Translational Science Institute

TRANSLATIONAL PILOT STUDIES PROGRAM

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

Brahm H. Segal, MD, Director

Goals of Pilot Studies Funding

- 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.

Translational Science

Translational Research

- Takes scientific discoveries made in the laboratory, in the clinic or out in the field and transforms them into new treatments and approaches to medical care that improve the health of the population.

Translational Science

- Field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process.
- Expedites the translational research process and the time it takes for laboratory discoveries to become treatments for patients.

Strengthen Community Engagement

- Continue to prioritize Pilot Studies with community engagement with an emphasis on underserved populations and track the impact of Pilot Studies on public health within our community.
- To ensure that Pilot Studies research is aligned with the goals of the WNY community, we added Brenda McDuffie as a lay community leader to our review committee.

Leadership over Pilot Studies Review

Tim Murphy

Brahm Segal

Albert Titus

Mike LaMonte

Brian Tsuji

Patricia Diaz

Ranjit Singh

Brenda McDuffie

Pilot Studies: Process (*How* we do it)

- Two stages: RFP → **1)** LOIs; **2)** Full proposals
- Executive Committee* reviews/prioritizes LOIs
- Typically, 25 invited for full proposal submission
- Partner with Penn State CTSI and CTSA External Reviewer Exchange Consortium for reciprocal reviews
- [Proposals from partner CTSA hubs are reviewed by BTC constituent researchers, *including previous Pilot Studies-funded investigators*]
- Reviews evaluated by Executive Committee to prioritize/fund pilot studies - typically $25-50K for 1 yr[§]

[§ Some exceptionally promising projects selected for 2-phase funding, consecutive years]

Pilot Studies: Criteria

- Scientific merit and innovation
- Significance and impact on the field
- Impact on research on special populations, people across the lifespan and underserved populations
- Potential for reducing regional health care disparities
- Effective use of a team science approach
- Rationale and utilization of proposed budget
- Potential that the study outcomes will lead to substantive extramural funding

Pilot Studies RFA: Funding Priorities

- Address healthcare disparities (WNY ➡ Nation)
- Develop approaches to overcoming translational research obstacles
- Pair “early stage” investigators with established investigators with a history of substantive extramural research funding, thus providing a built-in mentoring system
- Promote multi-disciplinary collaborations (“team science”) across the BTC (inter-departmental, -school, -institution)
- Clear plan for future substantive funding (e.g., an NIH R01, R21, or comparable grant, including one of the NCATS/CTSA Program funding mechanisms)

What this mechanism is NOT for:

- “Bridge funding” (in-between grants)
- Support of fundamental (“bench”) research without a clinically translatable component

Tips for Success in Obtaining CTSI Pilot Funding

- Read and follow the RFP directions carefully!
- Make sure you are eligible for Pilot Studies funding.
- Make sure your proposal is consistent with the purpose and goals of our CTSI Translational Pilot Studies Program.

Translational Pilot Studies

Support to develop and test novel approaches to accelerate translation and advance clinical and translational science

About Us
See Application Process
Current and Past Awards
Pilot Studies Colloquium

About Us

Funding for original pilot projects allows applicants to generate preliminary data to secure future extramural funding, develop new methods, or make discoveries that will advance translational science.

Review Process

Applications will be rated using the following criteria:

- Scientific merit
- Clinical significance and translational impact
- Potential for securing substantive extramural funding (e.g., NIH or DoD)
  - Explain how accomplishment of Pilot Study aims will provide preliminary data that will strengthen a future NIH or equivalent grant
- Innovation
- Realistic milestones and feasibility of completion (1 year)
- Rationale and use of proposed budget
- [For revised proposals] Compelling arguments that address prior critiques

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 CTSI pilot funding must cite our award (UL1TR001412, NCATS/NIH) and must have a PMCID number.
- CTSI-funded investigators are expected to participate, if called upon, as reviewers of CTSA pilot proposals submitted by other investigators.

Expectations (continued)

- Following completion of pilot study, and annually thereafter, provide noteworthy milestones:
  - Grants received
  - Papers published
  - Clinical studies generated
  - Patents received
  - Relevant teaching activities
  - Websites generated
  - Honors/awards/promotions

Requirements for Human Subject Research and Animal Research

- For proposals involving human subjects, following submission of the full proposal, the PI must submit all necessary documentation to the CTSI Clinical Research Facilitators for pre-review. This includes:
  - IRB protocol and related materials
  - Human subjects education if applicable
  - ClinicalTrials.gov registration status if applicable
  - Conflict of interest
- PIs from Roswell Park Comprehensive Cancer Center should follow a similar process while using the Roswell Park Click and IRB.
- For proposals involving animals, the PI must submit their protocol to IACUC *prior to submission* of the full proposal.

Common Pitfalls in Applications

- Proposed research isn't **translational** or the translational application is unclear
- Lack of clearly stated gap in knowledge and **significance** of the proposal to the field
- Weaknesses in statistical design
  - **Consult with BERD early in the application process**
- Concerns about feasibility
- Lack of clear articulation about how the Pilot Studies will lead to substantive future funding (e.g., NIH or DoD)

Pilot Study Vignette

Jason Muhitch, PhD, Assistant Professor of Oncology, Department of Immunology at Roswell Park Comprehensive Cancer Center

“Development of Radiotherapy Regimens for Improved Antigenicity of Human Renal Cell Carcinoma”

- $23,000 pilot studies award (2019-20)
- Support work published in PNAS (Chow J et al. PMCID: PMC7519245)
- Based on this work, Dr. Muhitch was awarded a Kidney Cancer Research Program Idea Development Award from DoD Office of Congressionally Medical Research Programs

Pilot Study Vignette

John Leddy, MD, Clinical Professor, Department of Orthopaedics, Jacobs School of Medicine and Biomedical Sciences

“A Randomized Controlled Trial of Exercise Treatment for Concussion”

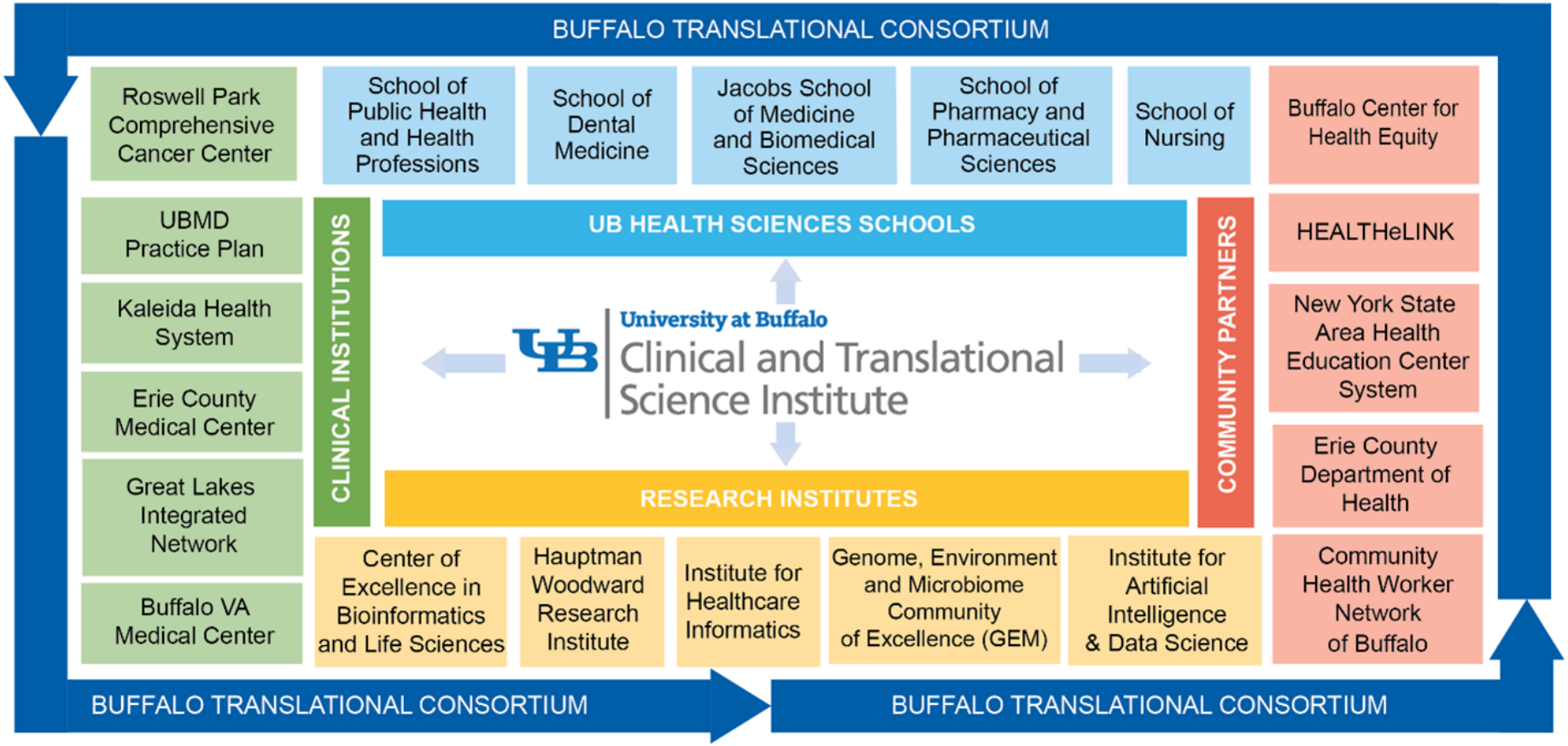
- $32,883 pilot studies award (2017-18)
- Conducted the first RCT to show aerobic exercise treatment prescribed to adolescents with concussion symptoms during first week after injury speeds recovery and may reduce incidence of delayed recovery.
- Results have been widely disseminated, particularly in the development of national and international concussion guidelines.

Pilot Study Vignette

Liise Kayler, MD, MS, Clinical Professor, Department of Surgery

“Feasibility of an eHealth Educational Intervention for African Americans with End-stage Kidney Disease”

- $45,000 pilot studies award (2020-21)
- Support work published in Transplantation (Kayler LK et al. PMID: 34873981)
- Provided preliminary data for an R01 “Increasing Live Donor Kidney Transplantation through Video-based Education and Mobile Communication”



Advancing research discoveries to improve health for all.