TRANSLATIONAL AND CLINICAL PILOT STUDIES PROGRAM

Director: Brahm H. Segal, MD

Vision

Our vision is that the Pilot Studies Program will support studies and train more translational researchers to develop new approaches to engage people who experience health disparities in clinical research so that they may experience the many benefits of clinical research. This vision aligns directly with our CTSI vision to perform research to reduce health disparities and to improve the health of our community.

What we do:

- Review/fund pilot studies projects

- Conduct annual *Pilot Studies Colloquia*

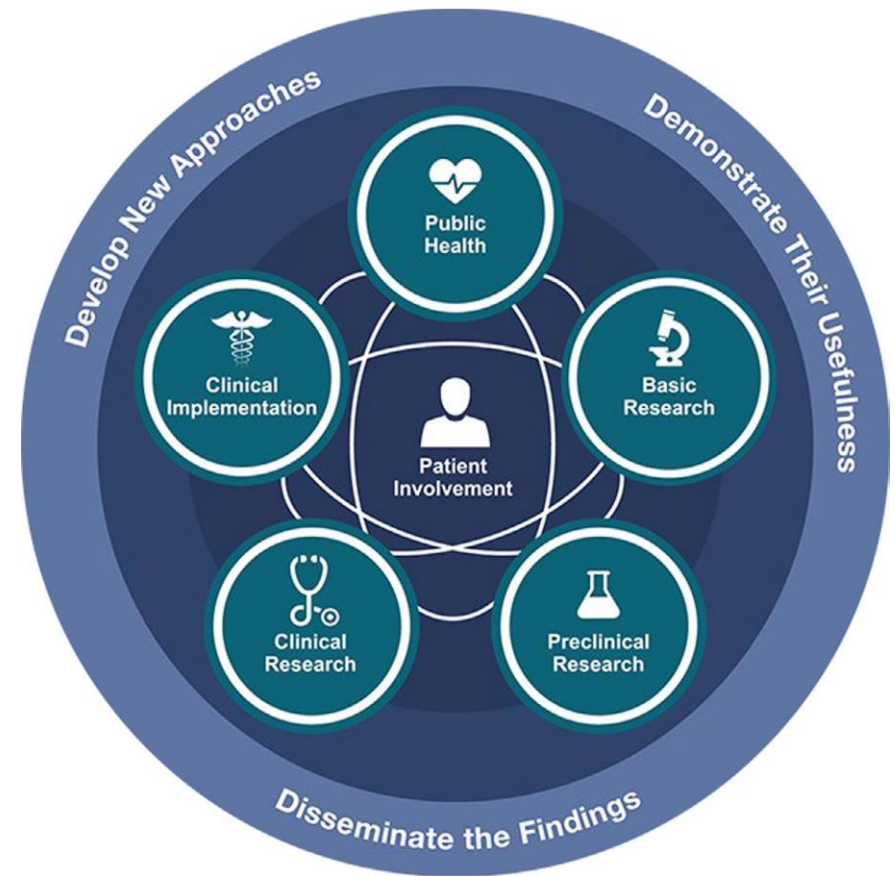- Conduct biennial *Workshops on Creativity & Innovation*

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.

NCATS Translational Science Spectrum

- **Mission of translational science**: "To bring predictivity and efficiency to the development and dissemination of interventions that improve human health."

- **Gap:** Successful fundamental discovery has not led to the expected therapeutic benefits to public health.

- Translational science attempts to address this gap.

Austin, CP. Opportunities and challenges in translational science. *Clin Transl Sci.* 2021

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

Michael LaMonte

Brian Tsuji

Patricia Diaz

Ranjit Singh

Brenda McDuffie

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

- Two stages: RFP $\rightarrow$ **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**

- Prioritize/fund pilot studies- typically $25-50K for 1 yr[§]

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

*Drs. Brahm H. Segal, Timothy F. Murphy, Albert Titus

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

Translational Pilot Studies Program*

Year	CTSA Funding	Institutional Funding	# of Awards
2015-16	$200,000	$400,000	8
2016-17	$200,000	$400,000	9
2017-18	$200,000	$400,000	13
2018-19	$200,000	$400,000	11
2019-20	NCE	$400,000	12
2020-21	$200,000	$400,000	13
2021	$200,000	$400,000	13
2022	$200,000	$400,000	13
2023	$200,000	$400,000	13
2024	$200,000	$400,000	12
Total	**$1,800,000**	**$4,000,000**	**117**

CTSA funding has <u>substantially enhanced</u> UB's ability to support and promote clinical-translational pilot studies within the Buffalo Translational Consortium (BTC)

Translational Pilot Studies Funding

- **$200,000 annual CTSA funding:** Going forward, will be dedicated to Translational Science projects

- **$400,000 annual institutional funding**
  - Provost
  - VP for Research and Economic Development
  - VP for Health Sciences
  - Roswell Park Comprehensive Cancer Center
  - Deans of the 5 Health Sciences Schools
    - Jacobs School of Medicine and Biomedical Sciences, Dental Medicine, Pharmacy and Pharmaceutical Sciences, Public Health and Health Professions, School of Nursing
  - Dean, School of Engineering and Applied Sciences

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"

Pilot Studies: Publications*

Year Funded	# of Pilot Awards	Publications in Peer-reviewed Journals
2015	8	12
2016	9	41
2017	13	40
2018	11	10
2019	12	7
2020	13	11
2021	13	4
2022	13	1
2023	13	0
2024	12	0
Total	**117**	**126**

*Projects initiated and supported <u>since</u> obtaining CTSA funding

Pilot Studies: Return on Investment*

Year Funded	# of Pilot Awards	Total Awarded	Extramural Funding
2015	8	$600,000	$7,372,295
2016	9	$600,000	$11,492,234
2017	13	$600,000	$2,513,906
2018	11	$600,000	$6,016,721
2019	12	$400,000	$4,139,835
2020	13	$600,000	$13,111,229
2021	13	$600,000	$791,965
2022	13	$600,000	$589,183
2023	13	$600,000	n/a
2024	12	$600,000	n/a
Total	**117**	**$5,800,000**	**$46,027,368**

Strategic investment by UB in support of clinical-translational research has been highly successful.

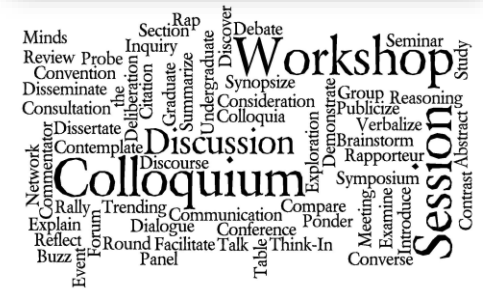*Projects initiated and supported <u>since</u> obtaining CTSA funding

Translational Pilot Studies 2024

- Request for Proposals released (March 31, 2023)

- Information Session held (May 1, 2023)

- **33** letters of intent received (May 15, 2023)

- **21** invited to submit full proposals (July 28, 2023)

- Exchange of reviews with Penn State CTSI and CEREC II

- Reviews to be completed, and proposals prioritized for funding (early-November)

- Award start date: January 1, 2024

Pilot Studies Colloquium

- Held December 14, 2023 (in-person)
- Oral reports from 2021 pilot study and 2023 community seed grant awardees
  - Progress posters were provided by 2022 pilot study awardees during the break
- Presentation by Pilot Studies leadership: "*Tips for Success in Obtaining Pilot Studies Funding*"*
- Next Scheduled: December 2024

Translational Pilot Program

Translational Pilot Program: Moving Forward

- Support pilot studies to develop innovative methods and technologies to solve significant clinical and translational research problems

- Emphasis on <u>underserved populations and healthcare disparities within Western NY</u> capture area

- Foster cross-disciplinary collaborations ("team science") and mentoring relationships

- Create a context for innovation in clinical and translational science across the Buffalo Translational Consortium (BTC)

Translational Pilot Program: Moving Forward

- <u>Build upon success</u>: Pilot studies program has been very successful in attracting investigators with diverse expertise to address important topics in improving health
  - Strong return on investment based on publications and grants
  - Further enhance pairing of senior/junior investigators
  - Expand research in patient and community engagement, e.g., small grants to increase participant accrual

Translational Pilot Program: Moving Forward

- **Translational Science**

- Novel pilot studies that target **inter-CTSA hub collaborations** (*e.g.*, focus on underserved populations and healthcare disparities), leading to NCATS applications (PAR-15-172, PAR-16-343)

- Continue reciprocal grant review exchange with Penn State and other CTSA hubs, as needed

- For those proposals *not* funded: provide investigators with referrals to "*Request a CTSI Service*"

https://www.buffalo.edu/ctsi/cores/translational-pilot-studies.html
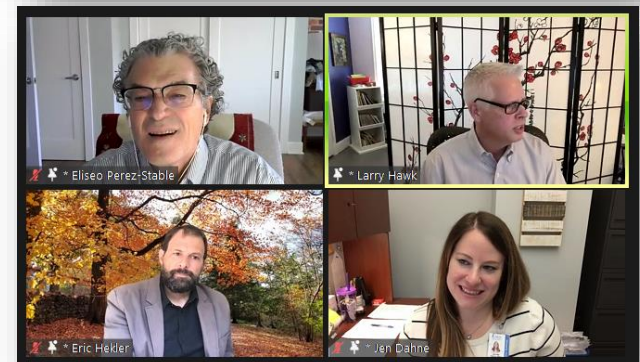
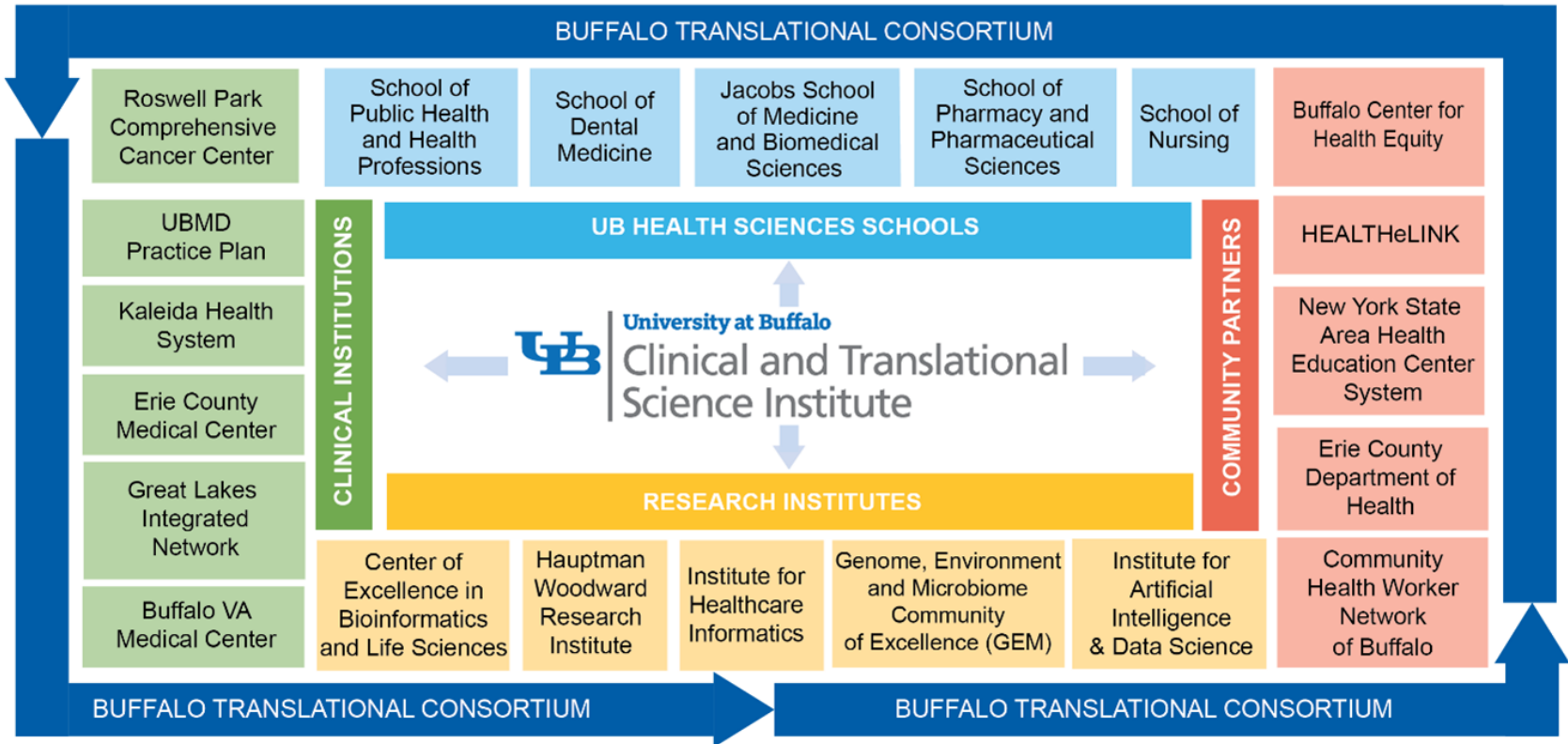## Biostatistics, Epidemiology, Research Design (BERD)

- BERD provides one-on-one consultations to deliver support for funding requests to sponsors.
- The involvement of BERD members results in applications for external funding which are <u>more competitive</u>.
- The development of materials is typically provided free of charge for grant applications that appropriately budget for BERD faculty/staff support.
- **Research on a Napkin**

Innovative approaches for recruitment and retention of study subjects

- ***Creative Scientist Workshops*** (CSW) address barriers in translational research and how to overcome them.

    - The 2021 CSW, "Remote Trials: Future or Fiasco" was co-hosted by the Medical University of South Carolina SCTR.

    - Featured "Inclusive Participation in Remote Trials" by Eliseo Pérez-Stable, Director of the NIH National Institute on Minority Health and Health Disparities.

    - The virtual nature brought almost 300 registrations (about 47% attended) and engaged a broad representation of the CTSA network including 32 hubs.

Advancing research discoveries to improve health for all