EINSTEIN-MONTEFIORE CTSA: ADVANCING TRANSLATIONAL SCIENCE

PILOT PROJECT PROGRAM
WHAT IS THE CTSA?

- Clinical and Translational Science Award
- NIH grant from National Center for Advancing Translational Sciences (NCATS)
- CTSA has provided continuous funding for the Einstein-Montefiore ICTR since 2008
- 63 US medical research institutions receive CTSA funding, called “hubs”
NCATS’ Mission

Turn research observations into health solutions through translational science
THE PUBLIC HEALTH CHALLENGE

10,000 Diseases

and only

500 Treatments or Cures

Time from early development to the medicine cabinet takes 10-15 years

9 out of 10 that enter clinical trials fail
The NIH/NCATS define:

- **‘Translation’** as the process of turning observations in the laboratory, clinic and community into interventions that improve the health of individuals and communities – from diagnostics, preventions, and treatments to medical procedures and behavioral changes.

- **‘Translational research’** as the endeavor to traverse a particular step of the translational process for a particular target or disease.

- **‘Translational science’** as the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process.

**Translational science** is “disease universal”; it focuses on the scientific and operational bottlenecks that are common to translational research for most or all diseases.

[https://ncats.nih.gov/translation/spectrum](https://ncats.nih.gov/translation/spectrum)

Austin CP. *Clin Transl Sci* 2021
Examples of Bottlenecks

**Basic / Preclinical Research**
- Target qualification
- Predictive efficacy
- Predictive toxicology
- ‘Risky’ undruggable targets/untreatable diseases

**Clinical Research**
- Data interoperability
- Inconsistent diagnostic and outcome criteria
- Clinical trial participant recruitment and diversity
- Clinical trial operational efficiency
- Administrative burdens (IRB)

**Clinical Implementation/Public Health**
- Time of intervention adoption
- Access and adherence
- Comparative effectiveness

Bottlenecks in the Translational Research Pipeline

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RE-ENGINEERING THE CLINICAL TRANSLATIONAL PIPELINE

Examples of Solutions

**Operational**
- “One size fits all” approach
  - Adaptive and other novel trial designs
- Low enrollment and diversity in clinical trials
  - Enhance community and informatics efforts

**Administrative/Training**
- Administrative burden for study start-up
  - Streamline regulatory processes
- Shortage of qualified translational investigators and staff
  - Training and career development best practices

**Scientific**
- Insufficient tools and technologies to predict toxicity and efficacy of new drugs
  - Platform-based (WGS for rare diseases, mobile tech for multiple diseases)
- Incompatible databases to advance data science
  - Data, harmonization, interoperability and integration
EXAMPLES OF PROJECTS

NOT Translational Science

- Testing a compound that was efficacious in an animal model of disease in a human model of disease
- Evaluating the toxicity of a newly developed compound to treat Alzheimer’s disease
- Identifying early biomarkers of Multiple Sclerosis
- Recruitment of historically underrepresented groups into a clinical trial of breast cancer
- Generating a research database of patients with Diabetes in the Bronx

Translational Science

- Developing models/assays that can be better predictors of efficacy in humans than current cell/animal models
- Development of new assay types based on human cells that can identify potential toxicities more accurately and efficiently than current animal testing methods
- Development of framework/models to identify biomarkers that change early in the course of intervention to gauge intervention efficacy
- Systematically studying barriers to enrollment of historically underrepresented groups in clinical trials
- Developing systems to merge clinical datasets from different sources accurately and efficiently
QUESTIONS? CONTACT US!

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