

# Efficacy to Effectiveness (E2E) Clinical Trial Design: *Call for CTSA Investigators to Participate in Innovative Design Lab*

## Background

The MIT's CBI NEWDIGS (Center for Biomedical Innovation NEW Drug Development ParadIGMs), in collaboration with the Johns Hopkins-Tufts Trial Innovation Center (JHU-Tufts TIC), is opening up a unique Design Lab forum to the CTSA Trial Innovation Network, for the first time, to develop and pilot a new approach to clinical trial design: efficacy to effectiveness (E2E) trials.

In the drug development pathway, *efficacy trials* assess treatments in optimally selected patients, under advantageous conditions, for short periods, and for regulatory approval and marketing. *Effectiveness trials* test treatments across the spectrum of patients ultimately treated, in real-world conditions, with follow-up that matches durations of typical treatment. However, appropriate effectiveness trials often are not performed, leading to lack of effectiveness and safety data for many new treatments. To address this issue, a study design was devised that *integrates efficacy and effectiveness trials into a single two-component "efficacy-to-effectiveness (E2E) trial."*<sup>1</sup> This innovative clinical trial design can more efficiently provide the required evidence to allow better decision making and accelerate the translation of research into clinical practice and policy.

## What is involved?

CTSA investigators will be selected to provide product specific case-studies for a unique, two-day interactive Design Lab that will address two therapies. The purpose of the Design Lab will be to explore approaches to integrated efficacy to effectiveness (E2E) evidence development with a focus on the production of relevant evidence for different stakeholders, including patients, payers, regulators, healthcare providers, and others, over the product lifecycle.

Each case will be introduced through a briefing book that is developed by the CTSA investigator with input from the CBI NEWDIGS team prior to the Design Lab and distributed under confidentiality agreements to all attendees for pre-Design Lab reading. The CTSA investigators will present the case at the Design Lab which is scheduled for March 7-8<sup>th</sup> 2018 in Boston, MA.

The cases will be evaluated during the Design Lab through the structured multi-stakeholder "scenario design" methodology facilitated by NEWDIGS. Post Design Lab summaries are provided to capture general and product specific learnings.

1 Selker HP, Oye KA, Eichler HG, Stockbridge NL, Mehta CR, Kaitin KI, McElwee NE, Honig PK, Erban JK, D'Agostino RB. A Proposal for integrated efficacy-to-effectiveness (E2E) clinical trials. *Clinical Pharmacol Ther*; 2014; 95(2) 147-153.

## Who is eligible?

CTSA Investigators who are preparing a protocol for an investigator-initiated, multi-center clinical trial of a drug (Phase 2 or 3) are invited to apply. The cases will be selected by an Advisory Group that is created for this Design Lab. The selected cases should address an unmet clinical need and include appropriate quantifiable study end points other than mortality. Thematic areas can include:

- Those cases for which heterogeneity of factors, such as patient response (safety, efficacy or effectiveness), practice settings, reimbursement, or future indications are anticipated;
- Optimizing broadly inclusive populations;
- Adapting complex protocols to real-world healthcare delivery or adapting novel expensive treatments to universal healthcare and access;
- Bringing low-cost treatments to market.

## How to Apply?

- Interested investigators should complete the required application form and return via email to Sheeona Gorman at [sgorman@tuftsmedicalcenter.org](mailto:sgorman@tuftsmedicalcenter.org).
- Investigators are expected to provide a clinical product development plan, which will form the basis of much of the DL discussion. Assistance in assembling this material will be provided by the JHU-Tufts TIC and NEWDIGS staff.
- All applicants will be invited, via an initial phone call, to share their interest in E2E and to discuss how an E2E strategy might apply to the proposed study.

## Deadline

September 15<sup>th</sup>, 2017

## For more information please visit

[www.newdigs.mit.edu](http://www.newdigs.mit.edu)

[www.trialinnovationnetwork.org](http://www.trialinnovationnetwork.org)

*Note: The Design Lab is a stand-alone event. Participants who wish to leverage the services and resources of the Trial Innovation Network will be encouraged to apply to the Trial Innovation Network for an Initial Consultation. Please contact Sheeona Gorman at [sgorman@tuftsmedicalcenter.org](mailto:sgorman@tuftsmedicalcenter.org) with any questions.*