Massachusetts Institute of Technology

MIT CBI Center for Biomedical Innovation

PROSPECTUS

NEWDIGS WISDOM Project

Evidence from real world data sources beyond traditional Randomized Clinical Trials (RCT) is increasingly proposed to dramatically, even disruptively, change biomedical development and use.

New data sources from medical records to social media, along with new analytical approaches, may produce additional evidence for regulators, payers, physicians and patients as to a drug or treatment's effectiveness and safety. To be impactful, however, the new approaches to using this information must meet decision maker needs for actionable evidence. In other words, the new evidence must be 'Fit for Purpose' (FfP) as determined by stakeholders upon whose actions success will depend.

NEWDIGS WISDOM Project: Focus and Approach

The NEWDIGS WISDOM Project seeks to illuminate how new kinds of evidence integrated with that from traditional RCTs could impact specific decisions regarding biomedical innovation licensing, access and use. In so doing, it provides a structured framework for the planning and production of integrated evidence (RCT + real world) across the life span of products. It is designed to be applied prospectively, early in product development, collaboratively with input from key stakeholders, and iteratively, reassessed and adapted as evidence emerges. It further applies a systems engineering approach to characterize the policy and process innovations that may be needed to enable the generation and use of acceptable RWE in reliable and scalable ways.

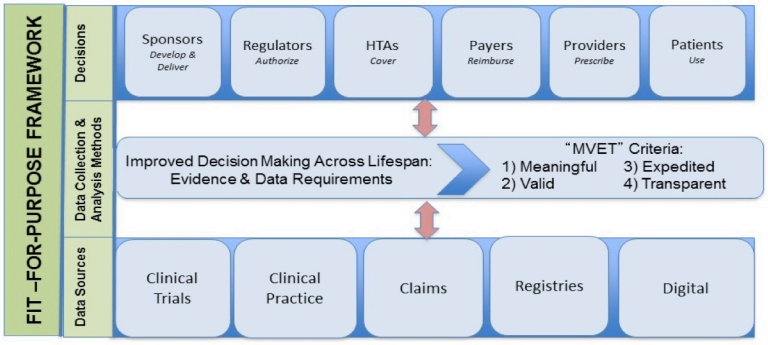

Figure 1: *Fit for Purpose (FfP) Framework designed to coordinate the planning and use of integrated evidence (i.e., from RCTs and real world data) to improve decision-making for all stakeholders across the product lifespan.*

Our vision is to inform the design of biomedical/healthcare innovation system that drive more value faster to patients, in ways that work for all stakeholders. WISDOM defines what it will take to generate evidence from emerging data sources, experimental designs, and analytic methods that is FfP for decision making by regulators, payers, physicians, and patients.

The FfP framework (*Figure 1*) centers on ensuring that evidence produced across the product life span is meaningful, valid, expedited, and transparent, as determined by parameters provided from the appropriate stakeholders.

It takes a systematic, question-based approach to defining qualification criteria as applied to specific product-focused use cases, such as:

1. What specific decision must be made, by whom, and in what (disease, product, or healthcare market) context?
2. What design considerations—e.g., end points, comparators, and timing/sequence of evidence development across the product life span—must be addressed in planning of evidence generation and use as relates to the question?
3. What are the critical characteristics of the data required to generate the evidence? Can existing data sources meet these needs? If not, what types of curation and de novo data sources might be considered?

Through case-based interactive design exercises involving all stakeholders, NEWDIGS prospectively explores a range of possible scenarios for the evidence generation and use plan across the product life span. It considers, for example, tradeoffs between the precision necessary for a given decision and the cost and timeliness of results, with a systematic exploration of theoretically appropriate methods for the associated generation of evidence. It then assesses the feasibility of executing on the proposed approaches by evaluating the degree to which existing data sources meet the requirements for acceptability as defined by the appropriate stakeholder/ decision maker. Lastly, it jointly considers various stakeholder concerns that may prevent certain approaches from being useful in practice, absent modifications to the original idea or to the systems in which the decision operates. The process thus enables a focus on exploring solutions that ultimately are methodologically sound and practically implementable to meet common goals.

The WISDOM Difference

Many initiatives are underway today that seek to develop new data collection capabilities from social media, wearable devices and the plethora of siloed medical data; to extract useful health information from unstructured raw data; to enhance the computer technology for exchanging and integrating data; and to develop algorithms for detecting correlations among those connected data elements. But moving the needle in the highly regulated pharmaceutical industry requires not only demonstrating exciting new technical capabilities, but also understanding requirements for acceptability by key decision-makers. This is currently particularly challenging with real world evidence due to the current lack of formal standards. This project addresses the more fundamental challenge of how to "pre-qualify" methods, data sources and, thereby, the resulting evidence so that it is established as FfP for making decisions regarding biomedical product authorization, access and use.

This project is also unique in its approach. "Pre-qualification" comes with an endorsement based on a multi-stakeholder deliberation. We bring together forward-thinking global stakeholders across the spectrum of licensing, access and use in a safe haven environment to examine the issues and develop creative solutions in the context of real product case studies. Evidentiary needs are assessed in the context of the global system that the evidence informs. This, now well-established, NEWDIGS "Scenario Design Lab" approach holistically explores and debates a product example from each stakeholder perspective to create solutions that meet everyone's needs, not simply those of a few. Design Labs create an interactive setting that combines qualitative discussions among all attendees with quantitative fact bases and simulations. The Lab insights are then further advanced with the participants over time to drive broader impact through the development of new methods and analytic frameworks; conduct pilot projects; inform policy designs; and disseminate emerging insights though high visibility publications and speaking engagements across the global industry.

Priority Activities in 2017-2018

Current and near-term activities in WISDOM center around a series of case-based "efficacy-to-effectiveness (E2E) design exercises. The goal of these activities is to develop a structured methodology & tools to help stakeholders reduce risk and uncertainty of drug development by:

- Identifying specific E2E gap drivers associated with the particular product & disease under consideration
- Exploring different evidence generation scenarios to reduce the gaps
- Evaluating the implications of each scenario for all stakeholders, in order to recommend the optimal path forward.

The E2E exercises in 2017 center around single product cases, while in 2018 we will focus on the scaling of E2E strategies and infrastructures in the design of next generation platform clinical trials and disease-focused innovation ecosystems.

In addition, an ongoing collaboration between the Johns Hopkins University-Tufts Trial Innovation Center and MIT NEWDIGS will target the design and real world implementation of E2E clinical trials, leveraging NIH's Clinical and Translational Science Awards (CTSA) national infrastructure for clinical trials.

The NEWDIGS Value Proposition

NEWDIGS functions as a pre-competitive "think and do tank" which drives impact and value by channeling the multi-stakeholder thought leadership of its sponsors and collaborators, and providing the following to its community of participants:

Access Unique safe haven "think and do tank" allowing interaction (formally and informally) with senior leaders, decision-makers, and change agents across stakeholder groups globally.

Influence Provides a platform to help shape and catalyze the future of pharma innovation.

Credibility MIT CBI serves as the neutral intermediary and collection point for world class academic researcher engagement with all key stakeholder groups in pre-competitive activities, lending gravitas and integrity to all proceedings

Innovation Opportunities External innovation strategies are diversified and augmented by traditional academia research partnerships with access to pre-competitive, multi-stakeholder collaborations

Actionable Strategic Insights

- *Ecosystem:* Evaluation of multiple case studies provide rich lessons to inform recommendations related to policy innovation, research priorities, and pilot opportunities designed to drive coordinated change.
- *Organization:* NEWDIGS forums provide emerging, future-oriented insights which participating organizations can use internally to set strategic priorities, redesign teams, and develop high impact process innovations.
- *Products:* Asset-specific insights can be gained from our global multi-stakeholder community in case-based confidential Design Labs, augmented by rigorous quantitative modeling and simulation.

Become a NEWDIGS Partner

The diverse multi-stakeholder community of global collaborators and sponsors in NEWDIGS provides an unusually rich opportunity for learning, building relationships, and creating new partnerships both within and outside of MIT CBI hosted activities.

Most importantly, the group shares a commitment to driving timely, sustainable, patient-centered change. Partners include leaders from biotechnology and pharmaceuticals companies, regulatory agencies, patient advocacy groups, public and private payers, health technology assessment officials, providers, and academic researchers, among others.

NEWDIGS partners contribute the resources and expertise required to deliver on the critical and timely mission of the WISDOM Project. Benefits to NEWDIGS sponsors include the following:

- **Opportunity to shape the future of data** access and use in biomedical innovation through high impact, actionable, collaborative activities with other project participants
- **Participating in all WISDOM Project undertakings and events**
- **Visibility** through co-authorship on subject matter publications and other communication vehicles about Project findings
- **Eligibility to nominate assets** from your R&D pipeline for analysis in NEWDIGS Design Labs
- **Openings for high performers within your organization** to engage in a broad range of career growth options in association with MIT CBI/NEWDIGS, including eligibility for a limited number of special appointments/secondments tailored to address your needs and interests.

Sign-up today!

Contact

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