Massachusetts Institute of Technology

MIT CBI Center for Biomedical Innovation

PROSPECTUS

FoCUS: Financing and Reimbursement of Cures in the US

This is an exciting time for patients. Novel, long awaited, potentially curative therapy options are becoming available. But, with over 600 premium-priced, curative treatments in clinical development, payers will be faced with a surge in claims for these products that is not affordable.

Biopharma companies can no longer assume the market will support premium pricing even for medicines that deliver meaningful and measurable improvements over the current standard of care. This combination points to the challenge of an urgent need for new financing and reimbursement models that ensure 1) patient access to needed treatments 2) affordability for public and private payers and 3) sustainability of innovation by manufacturers.

Addressing the challenge now

NEWDIGS, under the auspices of MIT Center for Biomedical Innovation (CBI) has launched a multi-stakeholder collaboration – the "Financing and Reimbursement of Cures in the US (FoCUS)" project - to address these issues. FoCUS provides a "think and do" platform for designing, evaluating, piloting and driving the timely implementation of nationally scalable solutions.

APPROACH

Who: Senior thought leaders and change agents from across the biomedical innovation value chain, including biopharma companies, patient advocates, investors, payers (public and private), physicians and policymakers, among others are FoCUS collaborators. A core group of initial project sponsors is in place. MIT CBI provides program leadership, serves as convener and neutral intermediary for all activities. NEWDIGS provides the systems approach and tools for engagement.

What: The FoCUS initiative will deliver

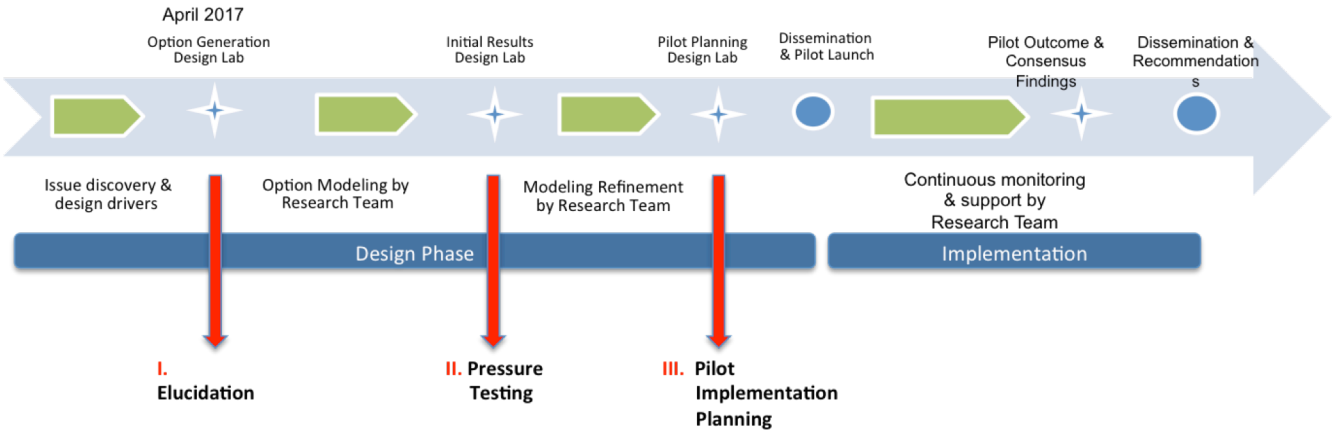

Figure 1: For each target area, NEWDIGS uses a staged approach to rapid cycle prototyping

practical, scalable policy and business solutions for providing affordable access to curative therapies. Those who participate in this process will have the opportunity to shape the immediate and long-term future of both medical innovation and financial solutions to chronic illness.

When: FoCUS is happening now. Formally begun on May 3, 2016 during an invitational Design Lab at MIT CBI, the following October saw the launch of two target area work groups in Gene Therapy and Durable Oncology with interest in a third, Antibiotics, expressed. The first case studies are underway and will be presented in Design Labs in April 2017.

MIT NEWDIGS:
Driving change in the development paradigm

The science: system gap cannot be addressed through our usual approach to innovation (i.e., siloed and reactive). The scale, complexity, and urgency of the required evolution represents a tipping point. We need to innovate how we innovate.

"Adaptive Biomedical Innovation (ABI)" offers a strategic framework for the re-engineering of drug development, licensing, access, and use, and for NEWDIGS activities (*see Fig. 2*). It is a multi-stakeholder approach to product and process innovation aimed at accelerating the delivery of clinical value to patients and society. ABI offers the opportunity to transcend the fragmentation and linearity of decision-making in our current model and apply a common collaborative framework that optimizes the benefit and access of new medicines for patients while also creating a more sustainable innovation ecosystem.

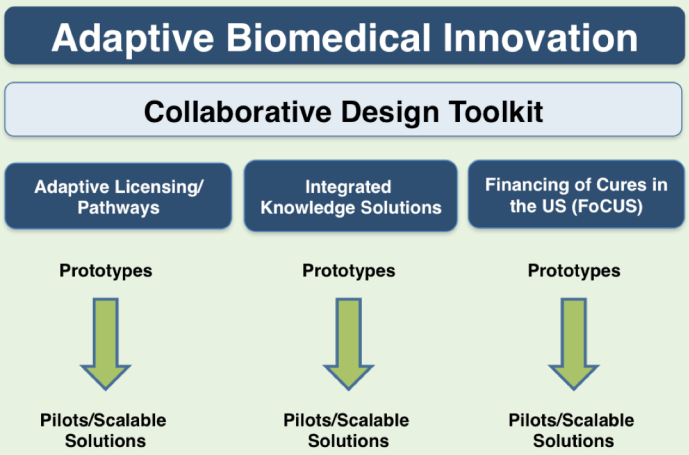

Figure 2: Overview of NEWDIGS activities, strategically integrated through the ABI framework. MIT CBI serves as convener and neutral intermediary for the pre-competitive "safe haven" activities of NEWDIGS

How: Collaborators work together within a 'safe haven'[1] environment, with efforts facilitated through the use of the proven NEWDIGS innovation processes and tools[2].

The *Design Phase* (see Figure 1) focuses on elucidation of key characteristics which have critical design implications and generating first round proposed financing solutions. 'Pressure Testing' of solutions follows by leading FoCUS partners through a staged process of structured brainstorming, concept generation and rapid cycle prototyping, with refinements informed by rigorous modeling and simulation. The last step is implementation planning for selected pilot designs.

The *Implementation phase* involves continuous monitoring, learning, and support of the efforts of FoCUS implementation partners, ending with a final assessment of outcome/impact, and dissemination of findings with recommendations for diffusion and scaling.

PROJECT STRUCTURE

The Collaboration Environment:
NEWDIGS is a unique, global, collaborative "think and do tank" focused on delivering new, better, affordable treatments to the right patients reliably and sustainably. NEWDIGS takes a holistic (i.e., technologies, processes, policies, and people) approach to designing, evaluating and catalyzing important system-wide evolutionary advancements. Success drivers in the approach include:

- Convening and engaging all key stakeholders for each activity
- Providing a neutral 'safe-haven' setting to foster candid dialogue
- Utilizing the NEWDIGS Design Lab methodologies and tools to drive rigorous quantitative evaluation of models

[1] Baird, Lynn G., and Gigi Hirsch. "Adaptive Licensing: Creating a Safe Haven for Discussions." *Scrip Regulatory Affairs*, (Sept 2013): 10–11.

[2] Trusheim, Mark R., Lynn G. Baird, Sarah Garner, Robyn Lim, Nitin Patel, and Gigi Hirsch. "The Janus Initiative: A Multi-Stakeholder Process and Tool Set for Facilitating and Quantifying Adaptive Licensing Discussions." *Health Policy and Technology* 3, no. 4 (December 2014): 241–47.

- Leveraging cross-disciplinary MIT expertise (e.g., systems and financial engineering)
- Driving meaningful, measurable, and sustainable transformational change.
- Disseminating insights to accelerate diffusion, implementation and scaling.

Team structure

The FoCUS team structure is designed to coordinate and channel the efforts of the distributed partner community to efficiently drive progress and impact. It is composed of a Steering Committee, ad-hoc project-wide teams and specific Design and Research teams as shown in Figure 3.

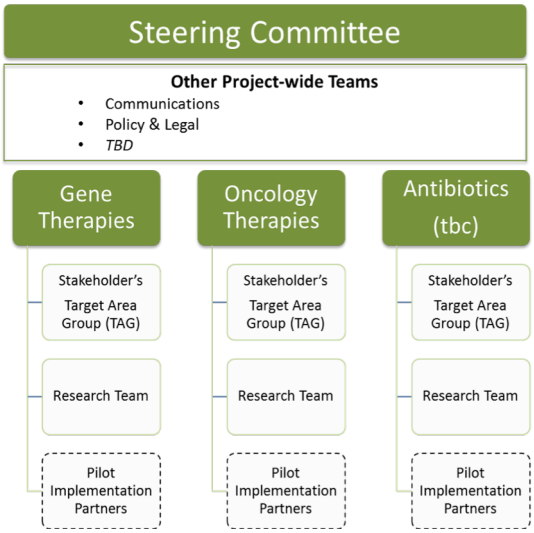

Figure 3: FoCUS team structure

The **Steering Committee** (SC) helps to shape strategy, identifies emerging opportunities, facilitates access to expertise, data, and other mission-critical resources as the project unfolds, increases awareness and visibility of FoCUS and its emerging outputs to accelerate and amplify impact. SC members have calls/meetings at least twice per year, with attendance at the Design Labs for each target area expected (3 days, 2 times per year).

Other teams that drive value across target areas will also be critical for success. Some of these (e.g., Communications) are standing teams staffed through MIT CBI, though active input from the partner community will be vital. Others (e.g., Policy and Legal) may be established on an ad hoc basis to address key issues that arise. These teams will be tailored for purpose, and may involve the engagement of experts within partner organizations, researchers from MIT or other academic institutions, and/or consulting advisors.

Each targeted disease area (e.g., Gene, Oncology, Antibiotics etc.) has a multi-stakeholder **Target Area Group** (TAG; see figure 2) and a **Research Team** who work together in a coordinated fashion through the process as described above. The TAG provides the case study content, input into concept generation, design considerations and refinement processes, with the Research team doing the modeling and simulation exercises to generate quantitative analytics and support for the refinement process.

TAG members come from patient advocacy, pharma, biotech, prescribers, investors, payers, and others who have a deep understanding of critical strategic and tactical issues that must be considered to increase the probability of success in the shaping of new financing/ reimbursement models, and the design and execution of associated pilots.

Members bring a constructive, collaborative style to the project. Not only do they work together within FoCUS, but serve as ambassadors within their own organizations on project progress, inputs and outcomes. Internal outreach efforts are key for coordination and information sharing; the FoCUS leadership team assists in these efforts as called upon. Typically stakeholder organizations deploy a representative to the TAG (and often two) in order to ensure that someone is always available to join regular FoCUS calls (~every 2-4 weeks) and Design Labs.

The NEWDIGS Health Economist/Research Lead heads the research efforts in collaboration with faculty and students from the MIT Sloan School of Management and others from the FoCUS partner community who choose to engage in these activities. Participation in this small technical team is welcome, and is arranged on a case by case basis.

BENEFITS TO ALL PARTICIPANTS

- Collaborate with senior leaders across the Biomedical innovation value chain in Design Labs, research, and special invitational events.
- Shape emerging financing & reimbursement models; translate insights into actionable strategies within your organization.
- Early awareness of emerging knowledge from Project's collaborative activities.
- Influence domestic and global policy via co-authorship with other FoCUS thought leaders of high impact, peer-reviewed publications.
- Close collaborations with researchers at MIT, an institution with an established longstanding history of transformational innovation involving industry, academia, and government

FoCUS is advancing now

Be at the table with your peers, competitors, and counterparts as we shape the next trend in financing and reimbursement models for curative therapies.

ADDITIONAL BENEFITS

Biopharma Companies:

- Opportunity to gain valuable insights on an R&D product through selection for evaluation in Design Lab exercises
- Potential opportunities to pilot novel financial solutions in collaboration with sponsors, driving corporate PR value while also advancing the greater good

Investors:

- Help shape incentive models to ensure the sustainability of innovation
- Gain early insights into emerging financing and reimbursement models for current and future portfolio companies

Patients:

- Contribute to the shaping of financial solutions which will affect patient co-pays and access

Payers:

- Shape financial solutions which focus not only on value, but also affordability across emerging portfolios of curative treatments

Physicians:

- Understand the potential financial constraints which may influence patient access options and decisions
- Provide information to enable informed decision making when considering therapy options for patients

CONTACT

Gigi Hirsch, M.D.
Executive Director
MIT Center for Biomedical Innovation
NEWDIGS/Initiative
617-253-9609
ghirsch@mit.edu