

NEW DIGS

FoCUS

Financing and Reimbursement
of Cures in the US

PROSPECTUS



FoCUS collaboratively addresses the need for new, innovative financing and reimbursement models for durable/curative therapies with acute upfront costs and accrued benefits to ensure patient access and sustainability for all stakeholders. Our forecast indicates about 40 such therapies will gain FDA approval in the next 5 years¹. Will current payment mechanisms buckle? Who will bear the financial, performance, and actuarial risk? Can we demonstrate solutions that fit everyone's needs?

FoCUS is developing a toolset that integrates durable therapy characteristics, patient populations, and payer segments to create 'fit for purpose' precision financing models. In the next phase we will pilot select solutions, create implementation 'playbooks', expand the toolset, quantify the impacts, and seek catalytic policy change.

**FoCUS Project
membership is open!**

See page 3 to learn
about the benefits of
joining.

Biopharma companies can no longer assume the market will support premium pricing even for durable and potentially curable therapies that deliver meaningful and measurable improvements over the current standard of care. Patients and providers want access to novel treatments that offer positive, potentially

life-altering outcomes, but payers are hesitant to commit until they understand how large the drug development pipeline is and the rate at which therapies will come into the market. FoCUS centers its work on finding suitable precision financing solutions; it does not work on setting value or pricing.

¹ MIT NEWDIGS Research Brief 2017F211.v011-Pipeline

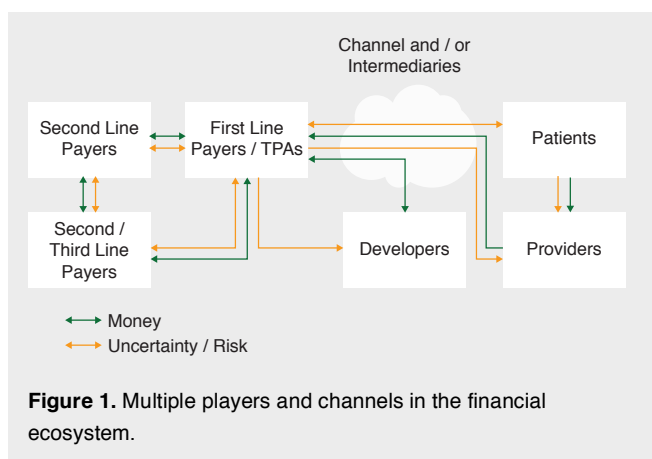
ADDRESSING THE CHALLENGE NOW

The Financing and Reimbursement of Cures in the U.S. (FoCUS) project was launched in 2016 by the Massachusetts Institute of Technology's New Drug Development ParadIGMs (NEWDIGS) Initiative. The objective of the FoCUS project is to deliver an understanding of the challenges and financial impact created by durable and potentially curable gene- and cell-based therapies and provide implementable financial solutions to manage the cost burden on the US healthcare system. The solutions aim to ensure as broad as possible access to patients who would benefit, while balancing the needs of other stakeholders appropriately.

APPROACH

Who: Senior thought leaders and change agents from across the biomedical innovation value chain, including payers (public and private), patient advocates, reinsurers, physicians, biopharma companies, and policymakers are FoCUS collaborators. MIT CBI provides program and thought leadership, serves as convener and neutral intermediary for all activities. NEWDIGS provides the systems approach and tools for engagement.

What: The FoCUS project has developed a process to establish and apply financing mechanisms that respond to systemic challenges identified in real-world case studies (see sidebar at right). This process has evolved into a toolkit with four components/frameworks that we have used to identify a full financial ecosystem, involving multiple stakeholders and channels as shown in Figure 1. Our tools support and facilitate greater understanding of the ecosystem, enabling effective, sustainable decision-making and opportunities to develop novel solutions and appropriate financing methods, which in turn are leading to actionable prototypes and pilots currently under development.



How: Collaborators in FoCUS drive the work, using a case-based approach to explore real-world exemplars of the issues, including regulatory and business practices, and to examine how proposed solutions would affect all stakeholder groups and what system-wide constraints might be in play.

Proven NEWDIGS tools² and practices—including the semiannual ‘safe haven’³ real-time interaction environment called Design Labs—enable members to work together throughout the year to ‘work the problem’—identifying primary and secondary concerns, proposing solutions, and generating a detailed understanding from each stakeholder perspective.

THE CASE STUDY PROCESS

Typically, each case study goes through the phases of **Elucidation** (who is affected, what their main challenges are), **Pressure Testing** (examines how viable the proposed solutions are), and **Implementation Planning** (examines what operational, logistical, regulatory, policy or other changes would be needed before implementation could occur).

PROGRESS TO DATE

Guided by a sense of urgency and pragmatism, the FoCUS team is committed to achieving real results and developing powerful tools. To date, project progress has included:

- Pioneering fit-for-purpose “Precision Financing” solution concepts with appropriate tools for use
- Proposed the new entity, “Orphan Reinsurance Benefit Manager”
- Currently designing two pilot projects *Multi-period, Performance-based Annuities* and *Short-term Milestone-based Contracts* with multiple payer entities and developers
- Completed detailed case studies on financing options for orphan diseases with cost-offsets, ultra-orphan therapies, and CAR-T treatments
- Inspired and maintained productive collaborations of >150 people from >50 organizations across the biomedical innovation stakeholder community
- Disseminated findings and recommendations through Research Briefs (<http://newdigs.mit.edu/papers-publications>), peer-reviewed journals (pending), and

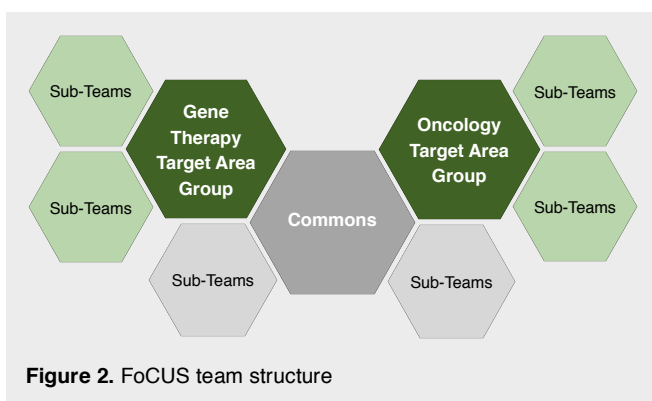
² Trusheim, Mark R., Lynn G. Baird, Sarah Garner, Robyn Lim, Nitin Patel, and Gigi Hirsch. “The Janus Initiative: A Multi-Stakeholder Process and Tool Set for Facilitating and Quantifying Adaptive Licensing Discussions.” *Health Policy and Technology* 3, no. 4 (December 2014): 241–47.

³ Baird, Lynn G., and Gigi Hirsch. “Adaptive Licensing: Creating a Safe Haven for Discussions.” *Scrip Regulatory Affairs*, (Sept 2013): 10–11.

international meetings such as ISPOR, OECD, and the Milken Institute Global Conference

TEAM STRUCTURE

The FoCUS team structure is built around specific Target Area Groups (TAGs), as well as Commons Teams as shown in Figure 2. These are led by MIT NEWDIGS staff, who coordinate and channel the efforts of the distributed collaborator community, with the consortium participants actively engaged in creating the deliverables.



A project leadership team shapes strategy, identifies emerging opportunities, and facilitates access to expertise, data, and other mission-critical resources. This ensures coherence across the teams and helps maintain awareness and visibility of FoCUS and its emerging outputs to accelerate and amplify impact.

Commons Teams drive progress on issues that cut across the TAGs. These teams emerge on an as-needed basis, and once their tasks are completed, dissolve. Commons Teams include:

- Communications (primarily engaged in dissemination activities)
- Portfolio Analysis and Modeling
- Payer Perspectives
- Patient Perspectives
- Tool Kit Development
- Policy/Simulation which includes Medicaid & Medicare related issues and financial system modeling

TAG membership draws from all FoCUS stakeholders as well as technical experts on the topics. Typically, stakeholder organizations deploy a representative to a 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.

ABOUT NEWDIGS AT MIT

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 approach (i.e., integrating technologies, processes, policies, and people) 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
- 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

WHY JOIN THE FOCUS PROJECT?

Those who join FoCUS have the opportunity to shape the immediate and long-term future of both medical innovation and financial solutions to appropriate patient access for durable/curative therapies.

Members bring a constructive, collaborative style to their work in the project. They also 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.

Benefits to all participants

- Collaborate in a pre-competitive environment 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 FoCUS’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

Additional stakeholder-specific 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 broader community

Financial Intermediaries (i.e. reinsurers, PBMs, etc.):

- 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 out-of-pocket costs and effective access

Payers:

- Shape solutions that address the financial challenges across the emerging portfolios of durable therapies
- Identify capabilities needed to implement the financial solutions within your organization and across your networks and members

Providers:

- Design alternative payment approaches to fund potentially uncompensated care activities
- Understand the potential financial constraints which may influence patient access options and decisions

Join FoCUS!

Successfully meeting the emerging challenges of financing durable/potentially curative therapies will take the hands-on engagement of all stakeholders in the healthcare innovation ecosystem. FoCUS provides an opportunity for innovation leaders to help shape practical, mutually-beneficial responses to this critical need.

To learn more about how to participate, please contact us.

More information on NEWDIGS projects can be found at: <https://newdigs.mit.edu/>

CONTACT**Gigi Hirsch, M.D.**

Executive Director
MIT Center for Biomedical Innovation
NEWDIGS Initiative
617-253-9609
ghirsch@mit.edu