

## NEW DIGS

# FoCUS

Financing and Reimbursement  
of Cures in the US

## PROSPECTUS

New cures and durable therapies for cancers and genetic disorders are here. They present unprecedented therapeutic promise—and equally unique financial challenges. Anticipating their arrival, the FoCUS Consortium set out to design thorough, real-world solutions to make sure these groundbreaking medical innovations are accessible by the patients who need them, and sustainable system-wide. Three years of work by hundreds of people representing the diversity of the healthcare ecosystem has yielded the FoCUS Precision Financing Strategies: a set of tools and techniques grounded in deep research and evolved in real-world cases. However, the most urgent work remains—launching FoCUS solutions into widespread adoption and implementation to transform the way the healthcare system pays for cures.

### FoCUS Project membership is open!

See page 3 to learn about the benefits of joining.

**With the potential** to eclipse the current standard of care, patients and providers want access to these novel treatments, but payers are hesitant to commit until they understand how durable the treatments are, how large the drug development pipeline is and the rate at which therapies will come into the market.

Developers ponder to how achieve effective returns for their investments in research and innovation. FoCUS (Financing and reimbursement of Cures in the US) collaboratively addresses these questions from all stakeholder's perspectives; exploring the need for new, innovative financing and reimbursement models, creating customizable solutions and leading pilot and prototyping initiatives.

### ADDRESSING THE CHALLENGE NOW

Launched in 2016, FoCUS has achieved its first objective to deliver an understanding of the challenges and financial impact created by new cell and gene therapies. However, to achieve the second objective, ensuring that emerging models are implementable, changes to today's regulatory environment and business operations are needed. These types of system-

wide changes need buy-in from all stakeholders and understanding by legislative and regulatory bodies, as well as those in positions of leadership within the business community. Baseline needs include the ability to track patient performance on a therapy over time for payer confidence and revisions to the Medicaid Best Price calculations to ally developer's fears of a single incidence creating a budgetary catastrophe. Members of the consortium are engaged in the development of pilots and prototypes, developing recommendations for catalytic policy change and engaged in the in-depth exploration of potential therapy impacts on patients and payers. FoCUS centers its work on finding and implementing suitable precision financing solutions; it does not work on setting value or pricing.

### 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's Center for Biomedical Innovation (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 studies. This process has evolved into a tool kit which helps 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.

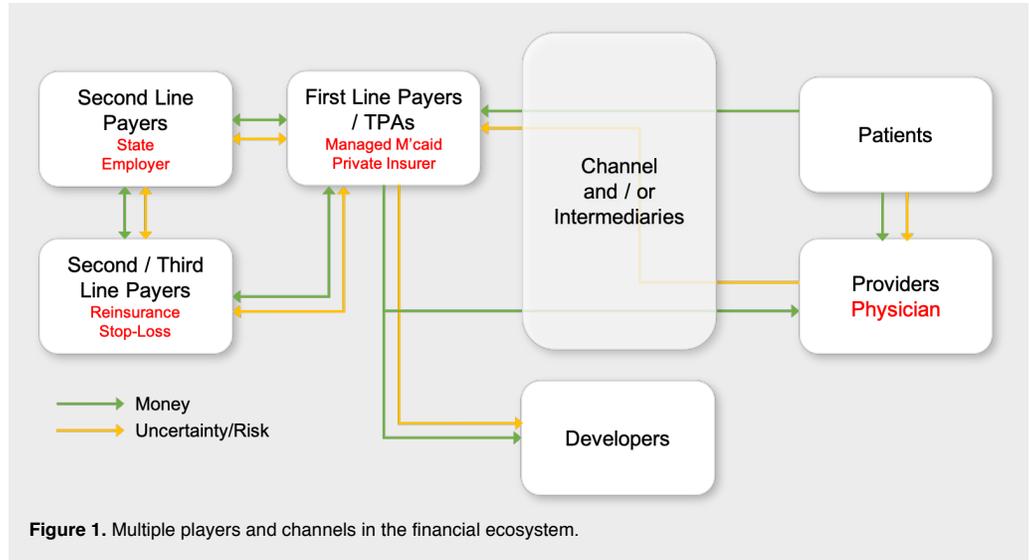
**How:** Collaborators in FoCUS drive the work, using a case-based approach to explore real-world exemplars of the issues, including regulatory and business practices, and to examine how proposed solutions would affect all stakeholder groups and what system-wide constraints might be in play.

Proven NEWDIGS tools<sup>1</sup> and practices—including the semiannual ‘safe haven’<sup>2</sup> real-time interaction environment called Design Labs (see sidebar) —enable members engage throughout the year to ‘work the problem’—identifying primary and secondary concerns, proposing solutions, and generating a detailed understanding from each stakeholder perspective.

### PROGRESS TO DATE

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

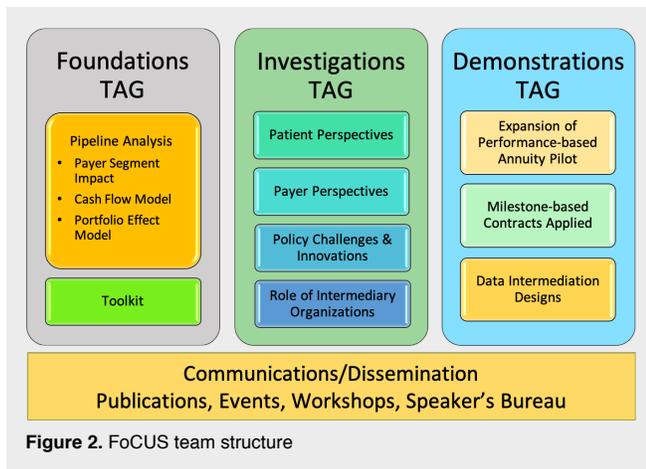
- Pioneering fit-for-purpose “Precision Financing” solution concepts with appropriate tools for use
- Proposed the new entity, “Orphan Reinsurance Benefit Manager” (ORBM) See [Research Brief](#) for full details
- Designing pilots and prototypes *Multi-period*, *Performance-based Annuities* and *Short-term 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/TCR treatments



- Inspired and maintained productive collaborations of >170 participants from >60 organizations across the biomedical innovation stakeholder community
- Disseminated findings and recommendations through Research Briefs (<http://newdigs.mit.edu/papers-publications>), articles, White Papers, peer-reviewed journals, public speaking engagements (ISPOR, OECD, and the Milken Institute Global Conference) and facilitated the *Paying for Cures: Ensuring patient access and system sustainability* Conference on 12 February 2019 in Washington D.C. to bring the message to the regulators, policy makers and others who can bring about change.
- Performed detailed research into the Drug Development Pipeline to size the potential rollout of cell and gene therapies along with the associated timeframe and expected costs to public and private payers.

### TEAM STRUCTURE

The FoCUS team structure is built around specific Target Area Groups (TAGs), which have morphed over time as issues were explored and conclusions reached and are now composed of Foundations, Investigations and Demonstrations (figure 2). The **Foundations TAG** provides the underpinnings of FoCUS with work on the drug development pipeline, including sizing the potential monetary impact by payer segment and cost modeling and toolkit work. The Toolkit team is synthesizing the work to date into a useable framework for public consumption. The **Investigations TAG** teams are performing a deep dive into specific subject areas, providing stakeholder perspective, recommendations and solutions. And, **Demonstration TAG** team members are working the precision financing solutions formulated by earlier work to an implementable state. **Communications/Dissemination** are responsible for ensuring the messages developed are disbursed around



the ecosystem and the creation of education channels for public and private parties. Teams 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.

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.

### 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 other key stakeholders, 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

### 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:

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

- Understand the potential financial constraints which may influence patient access options and decisions

Now is the time to implement precision financing solutions to ensure patient access and system sustainability.

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<sup>1</sup>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.

<sup>2</sup> Baird, Lynn G., and Gigi Hirsch. "Adaptive Licensing: Creating a Safe Haven for Discussions." *Scip Regulatory Affairs*, (Sept 2013): 10–11.

#### 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

Other NEWDIGS Projects include Adaptive Licensing, LEAPS (Learning Ecosystems Accelerator for Patient-centered Sustainable innovation) and the Dynamic Dossier in the Cloud.