

FoCUS Project

Financing and Reimbursement of Cures in the US

RESEARCH BRIEF

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Designing Precision Financing for cures can be informed by a framework that tailors solutions to the three financing challenges of actuarial risk, performance risk and payment timing based on the characteristics of the cure archetypes and the payer segment in the context of the specific care delivery ecosystem.

By **MIT NEWDIGS FoCUS Project**
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Through a collaborative, Design Lab process the FoCUS Project has created a framework to guide precision financing design for durable medicines such as gene, cellular and anti-infective therapies.

An accelerating flow of durable therapies and cost is emerging from the global medicines development pipeline. In 2017 the first three gene therapies of the next generation were approved in the US. Nearly 1,000 therapies are in the development pipeline. [We estimate](#) that about 40 gene therapies will receive approval by 2022 with more thereafter in indications from cancer to cardiovascular to rare disease.

FoCUS does not address the proper value to set for these products. Rather, assuming they will be costly, FoCUS seeks to design financing solutions that help all stakeholders facilitate appropriate patient access.

The financial challenge posed by cures consists of three elements:

1. **Payment timing/affordability:** Upfront payment for multiple years of therapeutic benefit.
2. **Therapeutic performance risk:** Real world efficacy and durability are uncertain at FDA approval.
3. **Actuarial risk:** The number of eligible patients in a payer's population may be uncertain and could vary significantly from period to period.

KEY FINDINGS

1. **Cures create three financing challenges:** Payment timing, therapeutic performance risk and actuarial risk.
2. **Cure archetypes alter the financing challenge mix** via differences in indication size & unmet morbidity/mortality, patient backlog, therapeutic modality, therapeutic durability and degree of cost offsets.
3. **US payer segments emphasize different financial challenges** due to their size, funding sources and regulatory context.
4. **Precision financing extends beyond payers and developers.** Providers, patients, reinsurers and the distribution channel also need precision financing and play critical operational roles for financing.

Cure diversity creates distinct financing cases

Gene therapies, cellular therapies, anti-infectives and other durable effect medicines do not present the same mix of financial issues. Hepatitis C treatments have little performance risk but the large and uncertain number of patients (actuarial risk) combined with the upfront payment that considered the value of avoided

later costs created financial stress for many payers that in turn led to strict patient access criteria. Gene therapies for rare conditions may have less absolute cost challenge from the payment timing, but the unpredictable number of a few high cost cases (actuarial risk) may introduce worrisome financial volatility for smaller payers.

Based on differences in indication size & unmet morbidity/mortality, patient backlog, therapeutic modality, therapeutic durability and degree of cost offsets, FoCUS has identified the following archetypes:

Oncology products: Nearly half the expected therapies for indications with, unfortunately, small patient backlogs and often other high cost therapy choices. The performance risk is also important with patient response variability and relatively long but likely not decades of survival benefit.

Novel breakthroughs: Therapies for ultra-orphan (<100 incidence) non-oncology indications. Often but not necessarily fatal childhood genetic disorders.

Orphan disrupters: Nearly 1/3 the expected therapies are for conditions with <200,000 prevalent US patients. These often balance all three financial challenges.

Quantum leaps: Like hepatitis C, therapies with large prevalent, or incident, populations could be 1/8 of future approved durable therapeutics.

Payer segments emphasize different financial challenges due to their size, funding sources and regulatory context. [FoCUS has identified](#) four segments:

- Self-insured employers
- Insurers and managed care organizations
- Medicare
- Medicaid

National insurers and traditional Federal Medicare have innate scale that reduces actuarial risk concerns whereas self-insured employers, some state Medicaid plans and smaller regional insurers could face material concerns from a single unpredictable occurrence of a gene therapy case. Mitigating payment timing may also differ among payer types depending on the cure archetype.

Table: Cure Archetypes & Payer Types Impact Financial Engineering Emphasis

Payer Type	Self-Insured Employers	Insurers /MCO	Medicare	Medicaid
Cure Archetype				
Oncology	Per, A	Per	Per	Per, A
Novel Breakthrough	A, Per, Pmt	Per	Per	A, Per, Pmt
Orphan disrupters	A, Per, Pmt	Per	Per	A, Per, Pmt
Quantum Leaps	Pmt, Per, A	Pmt, Per	Pmt, Per	Pmt, Per, A

A: Actuarial Risk Per: Performance Risk Pmt: Payment Timing

The regulatory context of each payer segment also affects the financial tools available, the administrative burden or both. Medicaid plans may face state balanced budget constraints and single year contracting rules. Solutions that address commercial plan challenges must also consider how the financing tools they may use might also affect Medicaid best price mechanisms.

Precision financing extends beyond payers and developers to include providers, patients, reinsurers and the distribution channel. These stakeholders play critical information or intermediary roles for financing and may directly participate in precision financing solutions themselves. For example, gene therapy contracting and financing flows are not just a two-party transaction between the therapy developer and the primary payer but may also include provider reimbursement, perhaps provider buy-and-bill, patient co-pays and deductibles, channel product rebates and reinsurance/stop-loss claims.

Providers may participate directly and also need financing. Many gene or cellular therapies require concomitant in-patient or out-patient procedures which may be affected by buy-and-bill and 340B regulations.

Specialty pharmacies, pharmacy benefits managers or both may participate in the contracting and financial arrangements.

Reinsurers and stop-loss providers whose policies often trigger at amounts lower than incurred by a single gene therapy case may be involved and choose to actively participate in offering solutions for the actuarial financial challenge. Contracting and financing by other participants should consider these triggers and flows in designing their own solutions.

Patients through co-insurance, deductibles and co-pays also have significant episode-based financial involvement. Multi-period financial solutions may

need to consider whether those later periods invoke patient costs, or refunds.

State governments may act as second line payers to the Medicaid managed care organizations contracted to act as either administrators or risk-bearing, capitated insurers.

Precision Financing Archetypes

FoCUS has identified four precision financing approaches that provide customizable solutions:

- **Milestone-based contracts** are short term to address basic performance-based risk.
- **Performance-based annuities** extend over multiple years with either rebates or contingent payments to address performance risk and payment timing/affordability issues.
- **Pooling** addresses actuarial risk and with milestone-based contracts or performance-based annuities can also address the other financing challenges.
- **Business as Usual Creativity** exploits flexibility in the current system and roles of current players to mitigate some financial challenges.

Each cure type and payer segment niche in the specific care delivery ecosystem for a condition may emphasize a distinct blend of the financing challenges. Thus, a given therapy will be expected to employ multiple precision financing approaches. For example, self-insured employers may need a different precision financing solution than national insurers and both may differ from a Medicaid precision financing solution.

This variety arises in response to the complexity of the existing US healthcare system and the diversity of players within it. The FoCUS Project is building an armamentarium to describe, diagnosis and suggest solutions to the financing needs created by curative, or at least durable, therapies.

About FoCUS

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to collaboratively address the need for new, innovative financing and reimbursement models for durable therapies that ensure patient access and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by durable therapies leading to system-wide, implementable precision financing models. This multi-stakeholder effort gathers developers, providers, regulators, patient advocacy groups, payers from all segments of the US healthcare system, and academics working in healthcare policy, financing, and reimbursement.

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