

Policy actions for enabling Precision Financing Solutions

With the rise of durable cell and gene therapies, there is the potential to offer patients who suffer from severe and potentially fatal conditions long-lasting and transformative benefits with just a single course of treatment. These therapies pose reimbursement challenges to healthcare systems such as:

1. **Payment Timing:** Our current healthcare system is organized around a ‘pay as you go’ structure for treatments and therapies, not to cover one-time payments for therapies that offer multi-year benefits to patients.
2. **Performance Risk:** While the outcome for any particular patient is uncertain, therapeutic effects could extend for years or possibly even a lifetime. A system that would better distribute risk would adjust reimbursement for the actual patient value received, rather than provide a fixed amount for an expected patient benefit.
3. **Actuarial Risk:** The number of patients that will receive therapy is hard to predict which can result in financial volatility, especially for smaller Medicaid and commercial insurance plans, and self-insured employer health plans.

Value-based purchasing (VBP) arrangements, payment-over-time, subscription models, warranty programs and other innovations can all help address these challenges. FoCUS members have been advancing multiple precision financing solutions in the belief that downstream innovation in access and reimbursement, needs to match upstream innovation in R&D. A variety of solutions are needed given different needs, constraints, and disease and treatment characteristics. Policy support is required to remove existing inadvertent barriers to these arrangements and to proactively facilitate broader utilization of innovative payment models.

Specifically, our recommendations would be to:

- **Update the Medicaid Prescription Drug Rebate Program (Medicaid Best Price) regulations and law.** CMS Proposed Rule CMS-2482-P takes an important step forward in addressing calculation barriers to broader adoption of milestone-based contracts, but further clarification is needed, particularly for therapies treating small numbers of patients. Guidance or more permanent legislative change would significantly improve the ability to implement payment-over-time and subscription models.

Key points

Durable, potentially curative cell- and gene-based therapies may offer transformative benefits for some patients, but raise financial challenges due to their one-time payments, performance uncertainty and actuarial estimation risks.

A range of precision financing solutions will be necessary due to the distinctive differences between diseases, products, and types of payers.

Implementing these payment model innovations will require:

- updates to the Medicaid Drug Rebate Program (e.g., Medicaid Best Price reporting requirements),
- clear safe harbor provisions to address these unique payment models in the Anti-Kickback Statute and Stark Law,
- and investments in patient outcome tracking infrastructure, CMS administrative systems, and State Medicaid contracting capacity and data systems.

- **Explicitly include rebates and payments arising from value-based payment agreements in an Anti-Kickback Statute (AKS) and Stark Law safe harbors.** Regulations for both laws currently provide a safe harbor for traditional rebates, but do not explicitly include VBP arrangements that tie payments or refunds to outcomes and potentially pay for monitoring visits that relate to these.
- **Clarify FDA Guidelines for communication with payers** to ensure that alternative patient-reported or other real-world metrics can be used in precision financing solutions for label-indicated populations.
- **Reduce the administrative complexity and cost of precision financing.**
 - **Increase CMS grants to State Medicaid agencies** to develop the capabilities and capacity to design, negotiate, and implement these agreements.
 - **Simplify or eliminate CMS approvals for State Medicaid VBP arrangements.** For example, establish a pre-approved State Plan Amendment (SPA) template that a state could adopt for automatic approval.

- **Advance outcomes tracking platforms through HHS support** for distributed real-world data systems capable of national longitudinal patient monitoring. For instance, allow IT investments needed for performance tracking as part of State capability grants, facilitate cross-disease area and cross-sectoral collaborations to create scale in data collection, enable cross-state/cross-payer interoperability and linking given patient movement across payers. Existing surveillance programs should be leveraged wherever possible to avoid duplication of efforts.
- **Amend HIPAA regulations to ensure data access** for precision financing-contracted entities. Clarification is needed so that developers, prior payers and others have access to required outcomes and other data needed for payment adjudication.
- **Plan for needed provider capacity and the potential institutional impact of these therapies.**
 - **Support staff and facility capacity investments** especially for transplant center, hemophilia treatment center, infusion center, and orphan disease provider center of excellence capacity.
 - **Support fair and stable provider reimbursement**, evaluating unintended consequences of changing reimbursement calculations and VBP requirements, e.g., Average Sales Price (ASP) volatility, impact of the buy-and-bill model, certification and data tracking resource requirements.
- **Address patient out-of-pocket expense challenges.** Facilitate awareness and use of patient-centered solutions (e.g., pharmaceutical assistance programs, commercial-patient access support programs, provider discounts and charitable write-offs). Encourage consideration of benefit regulations that allow payers to make benefit design modifications beneficial to patients, such as reductions or waivers of deductibles, co-pays, and coinsurance payments with automatic regulatory approval upon filing with insurance regulators.

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 and potentially curable therapies that ensure patient access and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by these 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 in this endeavor.

About MIT NEWDIGS

MIT NEW Drug Development ParadIGmS (NEWDIGS) is an international “think and do tank” dedicated to delivering more value faster to patients, in ways that work for all stakeholders. NEWDIGS designs, evaluates, and initiates advancements that are too complex and cross-cutting to be addressed by a single organization or market sector. Its members include global leaders from patient advocacy, payer organizations, biopharmaceutical companies, regulatory agencies, clinical care, academic research, and investment firms. For more information, visit <http://newdigs.mit.edu>.

For further details on these concerns or to get involved, please go to payingforcures.org