Federal Policy Suggestions for Durable Therapies

Emerging classes of durable therapies with short (sometimes single dose) treatment regimens and lasting benefits create significant healthcare financial challenges. One key challenge is the heightened importance of uncertainty regarding benefit level at the time of treatment. FoCUS has developed potential solutions to durable therapy financial challenges, including milestone-based contracts, performance-based annuities and an Orphan Reinsurer and Benefit Manager (ORBM). These solutions require changes to many existing policies and practices for implementation.

Some of these changes can only be made by the Federal Government, while others might benefit from a global solution rather than workarounds. Some, though very important for durable therapies, may also be known issues that have broader impact beyond this area. FoCUS recommends consideration of the changes below to allow the US healthcare financing and reimbursement system to evolve in ways that will facilitate appropriate, timely patient access to these new therapies.

POLICY RECOMMENDATIONS AND SUGGESTIONS IN PRIORITY ORDER

Medicaid Drug Rebate Program Accommodations for Performance-Based Agreements

Issues

1. Unclear how to report performance-based rebates or payments that may occur years after treatment

2. For rare conditions, an insurer who has unusually poor outcomes in a small group of treated patients could set an unfairly low price for all Medicaid patients

Recommendations/Suggestions

1. Safe Harbor/waiver exempting pilots from incorporation into current Medicaid Drug Rebate calculations in order to test alternative approaches for calculating Medicaid rebates

2. Development of performance-based agreement reporting and rebate methods. Methods that avoid small sample size volatility could be based on one of the following:

   A. Average actual patient payments for patients with similar performance-based agreements (updated until agreement completion)

   B. National average actual patient performance applied to individual contract terms (updated until agreement completion)

   C. Expected average payment based on expected patient outcomes (at time of patient treatment, e.g. from clinical data) applied to individual contract terms

FDA Communication Guidelines to Enable Appropriate Performance Metrics

Issue

Payer agreements that use performance metrics not reported in the FDA-approved label may place the developer-manufacturer in violation of FDA Guidelines for communication with payers.

Recommendation/Suggestion

Clarify that patient real-world performance metrics are acceptable in performance-based agreements when therapies are administered to patients in accordance with the label indications and usage criteria. Metrics need not be limited to those included in the FDA label. FDA guidelines remain relevant for any communications regarding the metrics.

Anti-Kickback Statute to Define Explicit Safe Harbor

Issue

The AKS regulations currently provide a safe harbor for traditional rebates, but do not explicitly include value-based agreements that tie payments or refunds to outcomes and may pay for monitoring visits that relate to these.

Recommendation/Suggestion

Explicitly include rebates and payments arising from performance-based agreements in an AKS safe harbor.

HIPAA to Enable Patient Data Visibility to All Involved Parties

Issue

Sharing patient performance data among the involved parties in performance-based agreements (especially developers and subsequent payers) often requires additional hurdles due to HIPAA regulations that did not contemplate this sort of agreement.

Recommendation/Suggestion

Amend HIPAA regulations to ensure that those investing in patient health through long-term performance-based agreements can access needed, relevant data. Clarify any enabling requirements for patient consent forms.

Federal and State Insurance Regulation to Allow Deductible and Co-Pay Waivers

Issue

Current regulations usually require refiling and approval of insurance products if patient benefit designs are altered. This 12-18 month process can delay patient-favorable changes by payers.

Recommendation/Suggestion

Amend regulations to allow payers to make modifications beneficial to all participants, such as reductions or waivers of deductibles, co-pays, and coinsurance payments, increases in patient incentives, and other improvements in patient access for specific therapies with automatic regulatory approval upon filing with insurance regulators.

Fair Provider Payments

Issues

Some therapies provided in the inpatient setting are reimbursed through existing fixed-rate payments (e.g. DRGs) that are intended to cover a range of possible treatment options. The inclusion of new high-cost therapies in this set creates a substantial loss for providers if they choose to use such a therapy, even after potential new technology add-on payments.

Therapies that can be provided in the outpatient setting may qualify for 340B pricing or be automatically paid at the rate of "Average Selling Price"+6%, creating much higher reimbursement than in the inpatient setting. Provider reimbursement may increase with treatment cost.

Recommendation/Suggestion

Establish fair, separate and fixed reimbursement rates for these therapies outside of the payment mechanisms for the clinical care and medical management of the patient to make provider reimbursement appropriate in all settings.

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**