NEWDIGS
FoCUS Financing and Reimbursement of Cures in the US

Massachusetts Institute of Technology

Financing models for durable and potentially curative therapies

Addressing payment and performance risk

Summary

- New financing approaches can help address access challenges for durable, potentially curative therapies.
- Payers and developers are discussing and beginning to implement a variety of solutions.
- Two key types of solutions are milestone-based contracts where payment is for results, and payment over time. These can be combined into a third solution: Performance-based annuities.
- To see broad adoption of these solutions, we need updates to US pricing rules, particularly Medicaid Best Price calculations, and infrastructure to track patient outcomes over time.

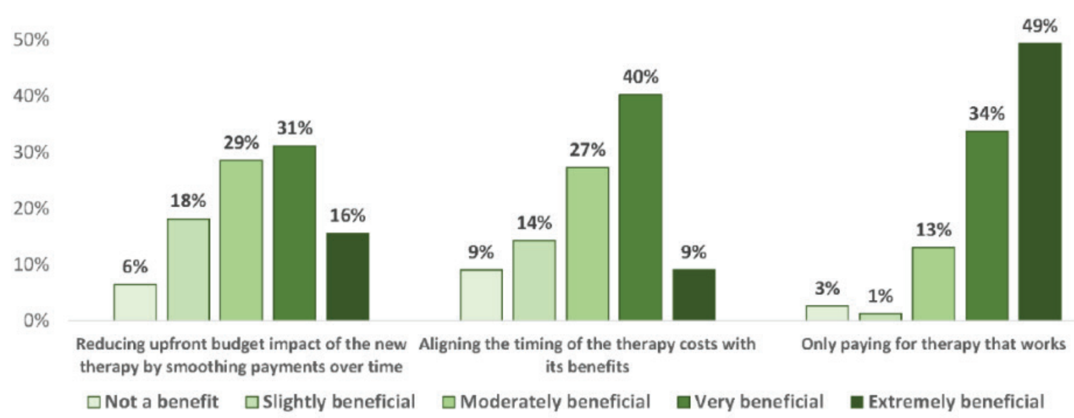
Payers and developers are discussing a variety of financing approaches to make gene and cell-based therapies available. The FoCUS Consortium has brought together over 70 developers, payers, providers and health systems, patient advocacy organizations and other stakeholders to identify and develop solutions.

These solutions help address some of the challenges payers face when they think about contracting for such durable, potentially curative therapies:

- Some payers may have challenges paying for a new treatment all at the same time, either because they are a smaller payer or because there are a large number of patients waiting for the treatment (payment risk)
- Some payers may not be able to easily predict how many patients in their plan may need or use a new treatment. This can create financial risk in managing their costs (actuarial risk)
- Some payers and developers may disagree on the expected performance of a particular therapy and want a guarantee of performance in case the treatment doesn't work well for a particular patient (performance risk)

Payers are particularly interested in financing solutions that manage performance risk and allow them to only pay for therapies that work, as the graphic below summarizing FoCUS research with payers, illustrates.

Benefits of alternative financing approaches (n=77)



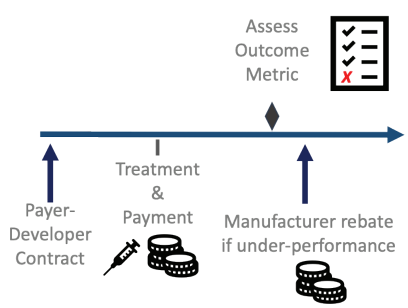
MIT CBI Center for Biomedical Innovation

The FoCUS consortium has identified multiple solutions for addressing payment, actuarial and performance risk. This document focuses on three solutions that address performance and payment risk: Milestone-based contracts, payment over time and performance-based annuities. Other solutions, including reinsurance, risk pooling, orphan reinsurer and benefit managers, are more focused on actuarial and executional risk, are further described on payingforcures.org/toolkit.

1. Milestone-based contracts/Performance Guarantees

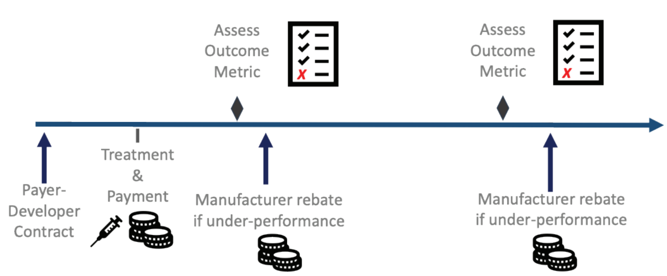
What is it? A milestone-based contract helps payers manage potential performance risk associated with a therapy. As the figure below illustrates, it assumes a payer and a developer agree on a price for a medicine. The payer pays that amount up front. The developer then guarantees the payer that it will provide a refund for non-performance if specific performance milestones or outcomes are not met.

Example Milestone-Based Contract



If the measurement of outcomes occurs over multiple years, there can be a multi-year milestone-based contract.

Example Multi-Year Milestone-Based Contract



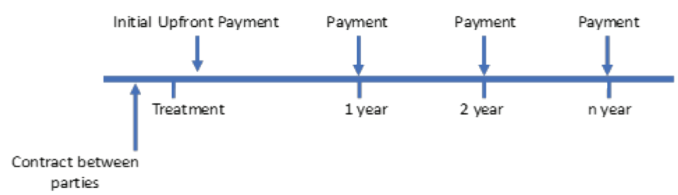
Do these exist today? Yes. An example variation on the above model is Spark Therapeutics' offer of an outcomes-based rebate arrangement for LUXTURNA® (voretigene neparvovec-rzyl), paying rebates if patient outcomes fail to meet specified thresholds in both the short term (30-90 days) and longer-term (30 months)[1].

Are these easy to do? They require effort to set up and run. Payers and developers need to agree on outcomes metrics. Running these models requires tracking patient outcomes over time, something which can be especially challenging if patients switch payers.

2. Payment over time/installment payments

What is it? Paying for a treatment over several years rather than in one upfront treatment. This can be helpful when the payer has a large number of existing patients waiting for a treatment, as it helps spread the cost of treating them over time. It can also help better line up paying for a therapy with receiving the benefits of that therapy. As the figure below illustrates, this approach might include an up-front payment of some portion of the product cost, as well as a commitment to further payments from the payer every year for a defined number of years.

Example Payment Over Time Contract



Do these exist today? Yes. Avexis proposed payment over time for ZOLGENSMA® in the US. Avexis arranged to have the option for payment over time be made available through a third party (Accredo specialty pharmacy of CIGNA's Express Scripts)[2].

Are these easy to do? in concept, these are possible. In practice, with current pricing regulations and accounting rules, they may be easier to do through a third party than directly.

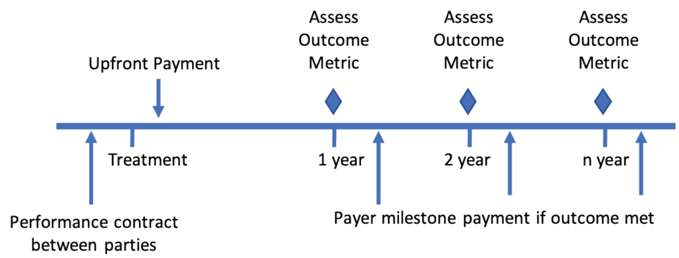
3. Performance-based Annuities

What is it? A combination of payment over time and a performance contract. Performance-based annuities are multi-year agreements between payers and developers that include an up-front payment by the payer of some portion of the medicine cost, as well as a commitment to additional payments from the payer every year for a agreed-upon number of years. Out-year payments are triggered when outcomes are achieved. This helps spread payments over time and also addresses any uncertainty about how a product will perform.

Do these exist today? Yes. Bluebirdbio has proposed a 5-year installment payment model in Europe for ZYNTEGLO® to treat beta-thalessemia, with payment only if the treatment works.[3]

Are these easy to do? This model combines performance guarantees and payment over time. Payers and developers need to agree on outcomes metrics and track patient outcomes over time. With current pricing regulations and accounting rules, they may be somewhat easier to do with Medicaid than with commercial payers, where a third-party intermediary may be helpful.

Example Performance-Based Annuity Contract



Broad adoption of these types of solutions will require updates to current pricing regulations – most importantly how Medicaid Best Price is calculated. Rebates (price discounts) to Medicaid plans were set up by law to give Medicaid the "best price" – a price for a medicine that is equal to or lower than the best prices received by most other payers. Best price is currently calculated on a unit basis so the lowest price for any one patient would become the Best Price for all Medicaid patients. Under the types of performance agreements described above where prices for treatments can vary depending on actual outcomes for patients, Medicaid Best Price calculations, as currently written, may result in inappropriately low prices for all Medicaid patients. For example, no manufacturer would agree to give a commercial payer a rebate of 100% (money back) if a product fails, because such an agreement could establish a "Best Price" of $0 for all Medicaid patients, even if 95% of all Medicaid patients had successful results.

Many of these financing models may be invisible to the patient in practice. Yet, the process of setting up these arrangements can lead to developer / payer conversations that may create opportunities for a holistic look at patient access considerations, including addressing co-pay issues, coverage for travel to treatment, etc. In financing models with performance guarantees, patients may have an opportunity to shape performance metrics and patients may need to help payers and developers track results over time.

[1] https://sparktx.com/press_releases/spark-therapeutics-announces-first-of-their-kind-programs-to-improve-patient-access-to-luxturna-voretigene-neparvovec-rzyl-a-one-time-gene-therapy-treatment/
[2] https://www.novartis.com/news/media-releases/avexis-announces-innovative-zolgensma- gene-therapy-access-programs-us-payers-and-familie
[3] http://investor.bluebirdbio.com/static-files/8c4eb7bb-37d6-4fba-941c-1a154c8bbfd6

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.