

NEW DIGS

# FoCUS

Financing and Reimbursement  
of Cures in the US

WHITE PAPER



## Precision Financing Solutions for Durable / Potentially Curative Therapies

24 January 2019

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*This White Paper is the result of the thought leadership, writing, reviewing and editing from many participants of the FoCUS Project. We want to thank everyone involved for their time and the work put into creating this manuscript.*

# 1. Executive Summary

The NEWDIGS FoCUS consortium of multiple stakeholders (payers, providers, patient advocacy organizations, pharmaceutical developers, academics and others) has been working collaboratively since 2016 to address the need for new, innovative financing and reimbursement models for durable/potentially curative therapies in the US to ensure patient access and sustainability for all stakeholders. FoCUS does not address how to value these therapies or set their prices. Rather, FoCUS seeks to create precision financing solutions for durable/potentially curative therapies with large, upfront costs whose benefits accrue over time.

Durable therapies create three financial challenges:

- 1. Payment timing:** Therapies can involve substantial upfront payment for multiple years of therapeutic benefit.
- 2. Therapeutic performance risk:** Real world efficacy and durability are uncertain at the time of initial regulatory approval and market launch.
- 3. Actuarial risk:** The number of eligible patients in a payer’s population may be uncertain and could vary significantly from period to period.

The FoCUS methodology examines candidate product case examples to understand the challenges as well as identify and evaluate potential financial systems solutions. To date, products for blood disorders, ultra-orphan conditions and cancer have been examined by the collaboration.

## ONE SIZE DOES NOT FIT ALL; A PRECISION FINANCING TOOLKIT IS REQUIRED

A critical general principle emerging from this FoCUS work is that **while a few broad solutions have emerged, each must be tailored to the specific context**. Figure 1.1 illustrates the influences.

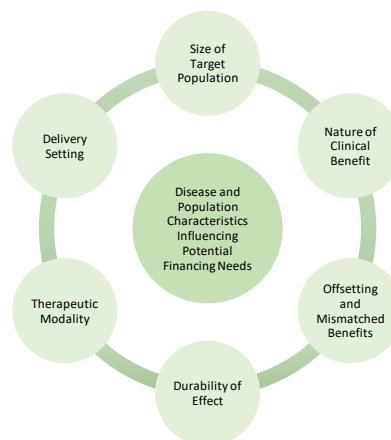


Figure 1.1: Therapy Characteristics Influencing Precision Financing

In addition, US payers divide into segments that vary by number of covered lives (size), types of lives covered (children, elderly, mixed), funding sources (self-funded, premiums, taxes), and regulatory context. The four main segments are self-insured employers, commercial insurers, and Medicaid and Medicare, with over 20 sub-types.

These variations subject each payer segment to different levels of payment timing, therapeutic performance, and actuarial risks. Similarly, the varying financial capacity, administrative capabilities and preferences of different developers may influence how, or even whether, potential financing solutions are offered and whether additional enablers or third-party intermediary assistance are needed. Addressing the variations in these influences requires a corresponding range of financial tools employed as payer needs and preferences will also shape financing model selection and use. [Get the Payer Segmentation Research Brief](#)

In FoCUS “Design Lab” workshops held every six months with working teams between, the multi-stakeholder FoCUS participants created a set of potential, customizable solutions. These precision financing **solutions were:**

- **Driven by the case studies** and the stakeholder challenges they presented;
- **Informed by research** conducted by joint stakeholder and MIT teams examining issues such as regulatory constraints, reinsurance, payer perspectives and the product pipeline; and
- **Aligned across stakeholders** in the Design Labs, again in the context of the hemophilia and beta thalassemia blood disorders, ultra-orphan and CAR-T cancer case studies.

The four highest potential precision financing solutions identified by the FoCUS participants vary by the financial issues they emphasize:

- ☑ **Addressing Immediate Uncertainty: A One-Year Milestone-based Contract** may alleviate the short-term performance risk with minimal implementation hurdles.
- ☑ **Spreading the Surge: A Five-Year Performance-based Annuity** addresses payment timing and product performance effectiveness and durability risk. By spreading payments over multiple years, it also partially mitigates the actuarial risk of both a surge from patient backlog and rare but high cost cases.
- ☑ **Smoothing the Risk: Risk Pooling** could potentially allow particular payers to mitigate their actuarial risk.
- ☑ **Creating Population and Operational Scale: Orphan Reinsurer and Benefit Manager (ORBM)** would combine the risk-bearing of reinsurers with the therapy contracting capabilities of pharmacy benefit

managers, the provider network-building and medical management capabilities of insurers, and perhaps a specialty pharmacy distribution capability. The ORBM provides: payers with predictable costs; providers with appropriate reimbursement; developers with market access; and patients with a single point of contact.

Table 1.1 summarizes the FoCUS participants’ perspectives regarding the general match of precision financing solutions with each product archetype and payer segment. These matches are meant as a guide, a particular product and payer segment may find that a different solution better fits its circumstances. Large population Quantum Leaps have not yet been deeply studied by FoCUS so the best solution, existing or new, is yet to be determined.

In this white paper, we emphasize insights regarding the milestone-based contract and performance-based annuity solutions. For a deeper description of the ORBM concept see the [ORBM Research Brief](#) and [article](#).

## INCLUDING PATIENTS AND PROVIDERS

To ensure appropriate adoption of these transformational therapies, the needs of the full system, including patients and providers must be understood and considered.

**FoCUS recommends eliminating patient deductibles and co-pays** via redesign of patient benefits for these durable/potentially curative therapies. For these therapies with clear eligibility criteria and extensive pre-authorization expected, deductibles and co-pays appear unnecessary and are a minor financial contributor. Even without deductibles and co-pays, durable therapies also can exacerbate patient financial challenges from lack of coverage, and limited provider networks – particularly cross-state – may create significant travel costs and lost wage costs.

**There is a need for education for patients and families mediated by physicians or patient organizations**, regarding the effectiveness and duration of effect uncertainties, along with the risk of treatment-associated adverse events.

**Providers need reliable, appropriate reimbursement mechanisms** for their medical services including adverse event management and the patient outcomes follow-up that durable/potentially curative therapies require. Failing to do so could place providers in conflict with patients’ best interests and so reduce appropriate uptake.

	Self-Insured Employers	Insurers/MCO	Medicaid	Medicare
Orphan Disrupters with cost offset (Hemophilia)	ORBM	Performance Based Annuities	Milestone Based Contract	
Orphan Disrupters without cost offset	ORBM	Performance Based Annuities	Risk Pooling	
Novel Breakthroughs		Performance Based Annuities	ORBM	Milestone Based Contract
Oncology				
Quantum Leaps	To be Determined			

**Table 1.1:** Primary Precision Financing Solution by Payer Segment and Product Archetype

**REGULATORY, OPERATIONAL, AND RISK MANAGEMENT ENABLERS REQUIRED**

**Precision financing must navigate regulatory issues** such as anti-kickback rules, Medicaid best price and average selling price (ASP) reporting, FDA manufacturing communications guidelines, and HIPAA privacy protections as shown in Table 1.2.

**Critical operational enablers are also needed,** particularly for outcomes data collection and provider reimbursement mechanisms. Secondary enablers include:

- **Skilled staff** in contracting, medical, and risk management functions will be needed to customize, implement and scale precision financing across multiple products and geographies.
- **Education and support** for (smaller) organizations developing such capabilities could be helpful.

**Precision medicine needs risk management innovation** especially for multi-period solutions.

- **Patient mobility among payers requires solutions to enable multi-period precision financing approaches.** Patients moving across plans is a risk for the first payer bearing the durable therapy expenditures while the next payers reap the lower future expenses potentially associated with cost offsets and lower installment payment arrangements over time. Patient mobility also inhibits the patient tracking that the first payer needs to adjudicate a performance-based annuity

while transferring the first payer’s contractual terms to subsequent payers is unlikely.

- **Centers of Excellence:** The outcomes of durable/potentially curative therapies depend on providers’ skill in patient selection and therapy administration. A centers of excellence approach may help ensure that all patients can access certified providers.
- **Reinsurance/Stop-loss:** Existing reinsurance and stop-loss products help payers and employers manage single year actuarial risk but may need to become multi-year to better match these therapies’ durable effects.

The unique characteristics of durable/potentially curative therapies as well as the diverse needs of payers, patients, providers and developers necessitate a range of precision financing tools that extend beyond traditional models. Transforming precision financing solutions from idea to implemented mechanisms will require policy changes and clarifications, new operational capabilities, robust risk management elements and collaborative efforts to ensure that each stakeholder’s needs are met so that patients benefit appropriately and rapidly.

Collaborative stakeholder action could enable more rapid development of these and similar financial solutions. The table above briefly summarizes the areas that would benefit from collaborative action to develop new capabilities, mechanisms and policies.

Enabling Change	Milestone-based Contract Solution	Performance-based Annuity Solution	ORBM Solution
<b>Regulatory</b>			
Revised Price Benchmarking	✓✓✓	✓✓✓	✓✓✓
Anti-Kickback Statute safe harbor inclusion	✓✓✓	✓✓✓	✓✓✓
FDA Manufacturer Communication Guidelines for early discussion & using outcome metrics not in label	✓	✓	✓
HIPAA revisions to ease patient outcomes collection & sharing	✓✓	✓✓	✓✓
<b>Operational</b>			
Outcomes data collection	✓	✓✓	✓✓✓
Provider reimbursement mechanisms	✓✓	✓✓	✓✓
<b>Risk Management</b>			
Center of Excellence	✓✓	✓✓	✓✓
Patient mobility mechanisms	✓	✓✓✓	✓✓
Reinsurance/Stop-Loss evolution	✓	✓✓	✓✓✓

✓ (low), ✓✓, ✓✓✓ (high) indicates relative level of importance and opportunity

**Table 1.2:** Areas for Collaborative Action for Enabling Change

FoCUS is continuing its work to design and pilot precision financing solutions for these transformative therapies. A performance-based annuity pilot for orphan gene therapies is in design for implementation in 2019 by a consortium of Massachusetts payers for their fully-insured populations. A second pilot is being designed for a Medicaid population that employs a milestone-based contract solution for an inpatient adoptive cellular therapy such as CAR-T for oncology. In addition, continued research and solution creation are underway regarding patient and payer perspectives, risk pools, and policy innovation.

## 2. Diversity Creates Need for Precision Financing

The NEWDIGS FoCUS consortium of multiple stakeholders (payers, providers, patient advocacy organizations, pharmaceutical developers, academics and others) has been working collaboratively since 2016 to address the need for new, innovative financing and reimbursement models for durable/potentially curative therapies in the US, to ensure patient access and sustainability for all stakeholders. **FoCUS does not address how to value these therapies or set their prices.** Rather, FoCUS seeks to create precision financing solutions for durable/potentially curative therapies with large, upfront costs whose benefits accrue over time.

### FINANCIAL CHALLENGES GENERATED BY DURABLE / POTENTIALLY CURATIVE THERAPIES

#### Durable therapies create three financial challenges:

1. **Payment timing:** Therapies can involve substantial upfront payment for multiple years of therapeutic benefit.
2. **Therapeutic performance risk:** Real world efficacy and durability are uncertain at the time of initial regulatory approval and market launch.
3. **Actuarial risk:** The number of eligible patients in a payer's population may be uncertain and could vary significantly from period to period.

The FoCUS methodology examines candidate product case examples to understand the challenges as well as identify and evaluate potential financial systems solutions. To date, products for blood disorders, ultra-orphan conditions and cancer have been examined by the collaboration.

### THERAPY AND PAYER DIVERSITY

The mix of challenges described above, as well as appropriate solutions, vary by therapy and payer. A critical general principle emerging from this FoCUS work is that **while a few broad solutions have emerged, each must be tailored to the specific context** of the target population, the nature of clinical benefit, offsetting and mismatched benefits, the durability of effect, the therapy modality, and the delivery setting in compliance with all applicable regulatory requirements.

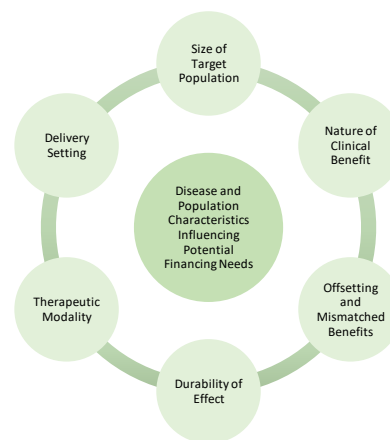


Figure 2.1: Therapy Characteristics Influencing Precision Financing

These therapy characteristics combine to create four durable/potentially curative therapy archetypes:

- **Orphan disrupters:** Treatments for orphan disease with a population of patients (<200,000 cases per year) that currently have an established treatment pathway<sup>1</sup>. Treatment is expected to disrupt the management of the disease as well as obsolete current standard-of-care treatments (e.g., hemophilia gene therapies) for a large portion of patients that meet eligibility criteria. The current SOC may or may not provide financial cost-offsets for durable/curative therapies.
- **Novel breakthroughs:** Therapies for conditions with <100 incident cases (ultra-orphans) with a high unmet need and preferably no alternative disease modifying treatments (e.g., Beta-thalassemia).
- **Oncology products:** Comparatively durable therapies (such as CAR-Ts) for oncology indications, usually with a high incidence-to-prevalence ratio.
- **Quantum leaps:** Indications with large incident and prevalent populations, representing a significant burden and potential surge effect for a new therapy in therapeutic areas, such as cardiology, metabolic disorders, neurology and rheumatology.

US payers divide into segments that vary by number of covered lives (size), types of lives covered (children, elderly, mixed), funding sources (self-funded, premiums, taxes), and regulatory context. The four main segments are self-insured employers, commercial insurers, Medicaid and Medicare with over 20 sub-types.

[Get the Payer Segmentation Research Brief](#) These variations subject each payer segment to different levels of payment timing, therapeutic performance, and actuarial risks. For

<sup>1</sup> In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people. This definition was created by Congress in the Orphan Drug Act of 1983.

example, Medicare and larger commercial insurers face less actuarial risk than self-insured employers, regional commercial plans, and some state Medicaid plans due to the significantly larger number of lives they cover. Financial solutions addressing actuarial risk may therefore be needed and appropriate for only smaller payers. Regulatory restrictions can also limit the appropriateness of financial solutions for different payers. For example, unlike commercial plans, State run Medicaid plans may have single-year contracting rules, limiting the use of contracts that amortize the costs of durable/potentially curative therapies over time.



### 3. A Portfolio of Solutions: Potential Tools

FoCUS participants identified over 20 individual financing tools ranging from **classic financial instruments** such as loans, receivables factoring and annuities; to **healthcare reimbursement techniques** such as the 340B program, buy & bill approaches, capitated reimbursement such as DRG (diagnosis related group), traditional rebates; and outcomes-based contracts, to **risk pooling** techniques including reinsurance and stop-loss policies, to **patient financing** including credit cards, support programs, asset financing/mortgages, to exotic instruments such as **securitized debt obligations**.

No single tool could meet all needs for every stakeholder. Rather, the participants moved to combining the tools into sets that formed Financial System Solutions (FSS), which in combination could address significant financial challenges for the stakeholders.

The multi-stakeholder FoCUS participants created these customizable Financial System Solutions in FoCUS “Design Lab” workshops held every six months with sub-teams working between Design Labs. These precision financing solutions were:

- **Driven by the case studies** and the stakeholder challenges they presented;
- **Informed by research** conducted by joint stakeholder and MIT teams examining issues such as regulatory constraints, reinsurance, payer perspectives and the product pipeline; and
- **Aligned by stakeholders** in the Design Labs, again in the context of the hemophilia and beta thalassemia blood disorders, ultra-orphan and CAR-T cancer case studies.

#### The four highest potential precision financing solutions identified by the FoCUS participants

We describe below the design and the implementation considerations for customizing these solutions for each durable/potentially curative therapy in the specific context of the target condition, product characteristics, payer segment, regulatory environment, reimbursement mechanics and operational capabilities of the stakeholders. Each precision financing solution varies in the financial issues it emphasizes:

- ☑ **Addressing Immediate Uncertainty: A One-Year Milestone-based Contract** approach, providing for a full or partial refund should the patient fail to achieve the agreed-upon minimum performance threshold at one year post treatment, emphasizes solving the short-term performance risk with minimal implementation hurdles.

- ☑ **Spreading the Surge: A Five-Year Performance-based Annuity**, in which payers immediately reimburse providers for treatment and follow-up tracking as it occurs, while paying developers over time in six equal payments (one upfront and five annual payments); with each annual payment dependent upon the patient’s condition meeting a pre-determined performance metric. Patients may be incentivised to participate in monitoring but will not incur future co-pays or deductibles related to these annual payments. This solution addresses payment timing and product performance effectiveness and durability risk. By spreading payments over multiple years it also partially mitigates the actuarial risk of both a surge from patient backlog and rare but high cost cases. This comes at the cost of more complex mechanics for data tracking and patient mobility as well as the need to navigate multiple regulatory challenges.
- ☑ **Smoothing the Risk: Risk Pooling** could potentially allow particular payers to mitigate their actuarial risk. FoCUS envisioned two approaches to this. In the first, state Medicaid agencies could form a risk pool with a carveout, which can then be used to pay for patients. Commercial insurers and self-insured employers pool through reinsurance and stop-loss policies respectively.
- ☑ **Creating Population and Operational Scale: Orphan Reinsurer and Benefit Manager (ORBM)** would combine the risk-bearing of reinsurers with the therapy contracting capabilities of pharmacy benefit managers, the provider network building and medical management capabilities of insurers, and perhaps a specialty pharmacy distribution capability. This ORBM provides the population and operational scale to offer smaller private-sector payers and self-insured employers a premium-like cost consistency, providers with appropriate reimbursement, developers with efficient market access coupled with performance risk sharing or value based agreements, and patients with a single point of contact for expedited benefit management and support. The ORBM faces many of the challenges of the prior two solutions plus the need to assemble the disparate capabilities into a single entity or operating alliance.

Table 3.1 illustrates the general mapping of solutions to financing before tailoring for therapy diversity, payer segment or provider characteristics. Milestone-based contract solutions emphasize mitigating short-term performance risk. Performance-based annuity solutions extend the performance-risk mitigation over years rather than months. In addition, they better match payments with patient benefits and in so doing, also provide some actuarial risk management—reinsurance ‘on demand’. Risk pooling addresses actuarial risk. When done by states it may also accomplish cost-shifting, increasing buying power or both.



Solution	Financial Challenge		
	Actuarial Risk	Performance Risk	Payment Timing
Milestone-based Contract		✓	
Performance-based Annuity	Some	✓	✓
Risk Pool	✓		
ORBM	✓	Possible	✓

✓ means the risk is addressed

**Table 3.1:** Financial Challenges Generally Addressed by the Financial Solutions

These added features are outside the FoCUS mission and will not be further discussed. The ORBM design potentially addresses all three financial challenges.

In this paper, we emphasize the Milestone-based contract and Performance-based annuity solutions. For broad implementation, each solution may require enablers, including legal and regulatory policy changes, new operational capabilities and robust risk management elements. We will highlight these considerations for each solution in this Section and then synthesize these new elements and enablers in the subsequent Section.

Below, we describe and discuss the Milestone-based contract and Performance-based annuity precision financing solutions in sections detailing:

- The goals of the solution
- The key design features
- The needed regulatory, operational and risk management enablers for the solution

This is followed, for completeness, by a brief description of the Risk pool and the ORBM solutions. The ORBM is discussed in more detail in additional FoCUS publications: [ORBM Research Brief](#) and [article](#).

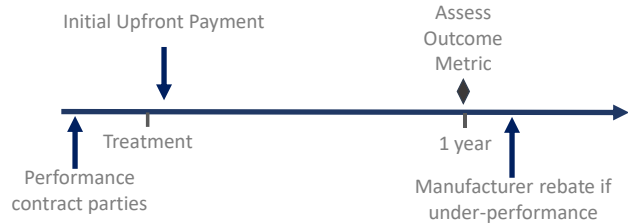
This white paper concludes with a description of the implications across stakeholders and the concluding discussion.

**SOLUTION 1 – ADDRESSING UNCERTAINTY: ONE-YEAR, MILESTONE-BASED SOLUTION**

**Description**

The one-year, milestone-based solution addresses the short-term performance risk uncertainty associated with a durable/potentially curative therapy. As the name implies, it

does not materially address the timing mismatch between multi-year benefit accrual and upfront payment. Neither does it address therapeutic durability risk past the first year, nor the actuarial risks of patient backlog surge or rare event cost smoothing. It is, however, the simplest performance-based approach to implement and does provide risk sharing for therapies such as CAR-T cellular therapy for cancer, which has significant immediate manufacturing and 30-day cellular infusion success risk, as well as substantial one-year morbidity risk.



**Figure 3.1:** Activity Flow – One-Year, Milestone-Based Performance Contract

This model begins with a performance contract in which an up-front payment of 100% of the agreed price of the product occurs between the relevant parties at time of patient treatment, an event the parties also need to clearly specify because most gene therapies have multiple provider visits which could provide the trigger. This could be a transaction between a provider (hospital or physician office) and the developer or between the payer and a developer, specialty pharmacy or wholesaler depending on the care setting and the medicine distribution model. The developer then offers outcomes-based performance rebates to the payer in the event of therapy under-performance. The developer and the payer pre-establish an agreeable patient outcome metric as well as the mechanics for measuring and adjudicating that outcome metric. In the design suggested in Figure 3.2, the outcome is assessed at the 12-month mark post-treatment. Failure to achieve the agreed upon outcome triggers a rebate from the developer to its counter-party of payer or provider. Achievement of the agreed upon outcomes triggers no rebate.

The list below summarizes the financial goals and the core elements of this solution:

**Financial goals**

- Shared performance risk between **payer and developer**
- Depending on the design, can remove financial risk from **providers** by eliminating buy-and-bill inventory risk and replacing the mark-up margin with negotiated fees for comprehensive patient care and data reporting
- Compatible with existing products from **reinsurance and stop-loss insurer** to manage actuarial risk
- Provides options for **patient** financial relief through one or more of the following: payer benefit design to reduce deductibles and co-pays and covering other out-of-pocket

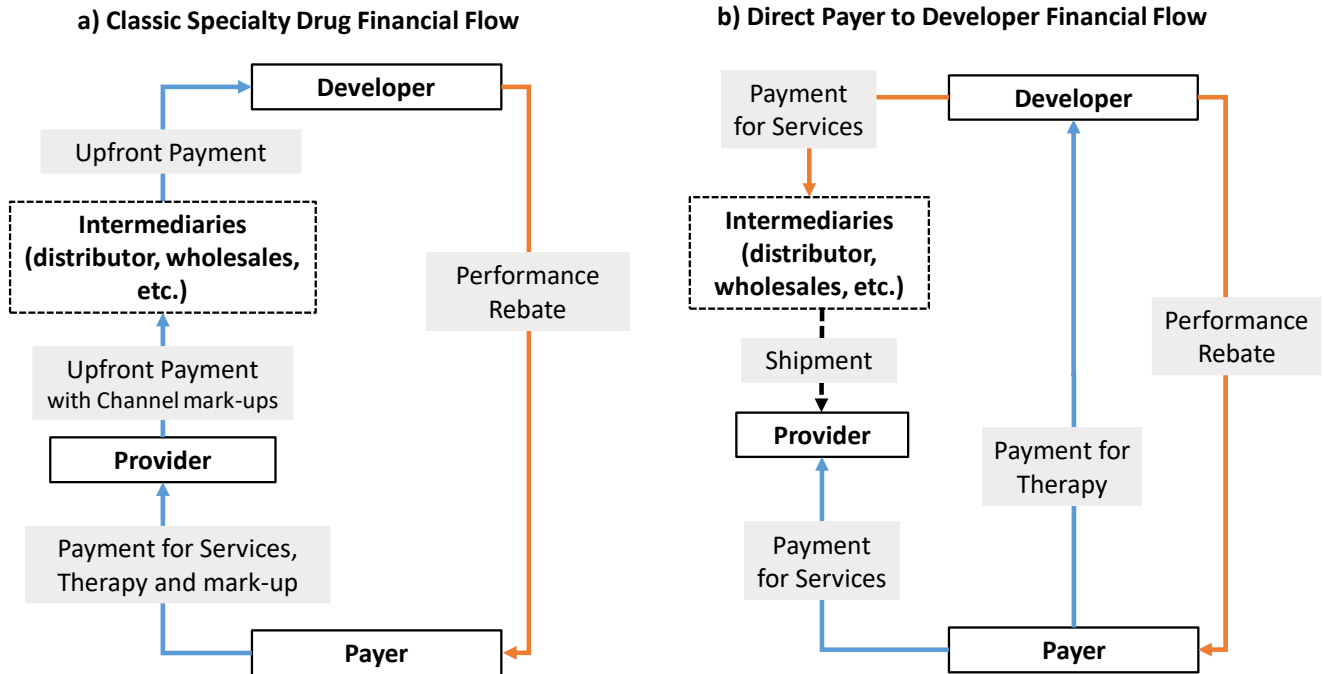


Figure 3.2: Alternative Payment Flows – One-Year, Milestone-Based Performance Contract



costs; developer patient assistance programs; provider waiving co-pay collection; and financial services products

Core elements of the One-Year, Milestone-Based Performance Contract solution:

- **One-year term core contract** between payer and developer
- Upfront payment for medicine by payer or provider; **refund by developer** on the core contract based on easily administered performance metric

Two potential cases of this exist, depending on whether the medicine is purchased from the developer by a provider or whether the payer chooses to avoid “buy and bill” by contracting directly with the developer for the medicine and paying the provider separately for medical services associated with its administration and follow-up care<sup>ii</sup>.

The boxes at right provide two case examples of the above models. The CAR-T therapy example in Box 3.1 describes a classic specialty drug financial flow for the KYMRIAH<sup>®</sup> oncology inpatient therapy. Box 3.2 contains the example of LUXTURNA<sup>™</sup>, a blindness outpatient treatment, that offers payers the possibility of direct contracting between payer and developer.

 <p><b>Box 3.1: In-patient Example</b></p> <p>The CAR-T therapies such as KYMRIAH<sup>®</sup> are developed through an individualized process for each patient and treatment is provided in the hospital. The hospital purchases the medicine from the developer. Payers negotiate contracts with the facilities that administer the treatment. Inpatient cost for the therapy is bundled into the total cost of inpatient stay and reimbursed to providers through DRGs (diagnosis related groups) and possibly supplemental provider payments under the “outlier” Prospective Payment System (PPS).</p>	 <p><b>Box 3.2: Outpatient Example</b></p> <p>Spark Therapeutics can sell LUXTURNA<sup>™</sup> directly to payers or payers’ specialty pharmacy, rather than to provider treatment centers. The approach avoids typical provider mark-ups on treatment acquisition costs and allows providers to avoid carrying costs on the therapy that they would normally bear in a buy-and-bill model.<sup>2</sup></p>
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<sup>ii</sup> “Buy and bill” refers to a situation where the provider purchases the medicine up front and then bills the payer for the medicines as well as the provider’s services in administering the therapy. The bill for

the medicine may include a mark-up by the provider for the handling costs.

**Enablers for the milestone-based contract solution**

FoCUS participants identified public policy, operational and risk management enablers required for the milestone-based contract solution. As the legal and regulatory issues are somewhat different if the product is administered outpatient (e.g., LUXTURNA™) vs. inpatient (e.g., CAR-T KYMRIA® and YESCARTA®), we have separated the two scenarios in Table 3.2. Outpatient and inpatient legal and regulatory issues also differ based on the buy-and-bill scenario.

**Needed Legal and regulatory enablers**

Policy enablers for milestone-based contract solution from the tables above are detailed briefly below.

- **Price Benchmarking:** The first issues relate to the method by which the US Government, usually CMS, creates benchmark prices to establish discounts, rebates and/or ceiling prices for a variety of Federal drug and therapy purchasing programs. These benchmarking systems were designed before outcomes-based reimbursement was envisioned. They were also designed assuming a relatively large number of prescriptions would be filled in any reporting period so that averages would

Scenario	Legal/Regulatory		Operational	Risk Management
	Outpatient	Inpatient		
<b>No buy-and-bill (payer purchases medicine)</b>	<p><i>Revised Price Benchmarking rules</i></p> <ul style="list-style-type: none"> <li>• Medicaid Best price regulations</li> <li>• 340B ceiling prices</li> <li>• Impact on 5i AMP</li> <li>• Impact on ASP</li> </ul> <p><i>Other Policy Enablers</i></p> <ul style="list-style-type: none"> <li>• Anti-Kickback Statute safe harbor inclusion</li> <li>• FDA Manufacturer Communication Guidelines encouraging value-based payment arrangements</li> <li>• Privacy Policy: HIPAA-compliant mechanisms for patient outcomes collection &amp; sharing among contracting parties</li> </ul>	<p><i>Revised Price Benchmarking rules</i></p> <ul style="list-style-type: none"> <li>• Medicaid Best price regulations</li> <li>• 340B ceiling prices</li> <li>• Impact on 5i AMP</li> <li>• Impact on ASP</li> </ul> <p><i>Other Policy Enablers</i></p> <ul style="list-style-type: none"> <li>• Anti-Kickback Statute safe harbor inclusion</li> <li>• FDA Manufacturer Communication Guidelines encouraging value-based payment arrangements</li> <li>• Privacy Policy: HIPAA-compliant mechanisms for patient outcomes collection &amp; sharing among contracting parties</li> </ul>	<p><i>Contract design features</i></p> <ul style="list-style-type: none"> <li>• Relevant outcomes measure</li> <li>• Outcome measure threshold</li> <li>• Definition of covered population</li> <li>• Patient mobility approach</li> <li>• Rebate basis: by patient or population</li> <li>• Rebate amount &amp; structure</li> <li>• Contract Term</li> </ul> <p><i>Execution enablers</i></p> <ul style="list-style-type: none"> <li>• Data capabilities, sources, roles and architecture</li> <li>• Provider reimbursement mechanisms</li> <li>• Organizational capacity</li> </ul>	<ul style="list-style-type: none"> <li>• <i>Center of Excellence</i></li> <li>• <i>Patient Mobility management</i></li> <li>• <i>Actuarial risk via Reinsurance / Stop Loss</i></li> </ul>
<b>Buy-and-Bill (provider purchases medicine and bills payer)</b>	<p><i>Above plus...</i></p> <p><i>Price Benchmarking-related needs</i></p> <ul style="list-style-type: none"> <li>• Impact on ASP (Average Sales Price) if included in Medicare Part B</li> </ul> <p><i>Non-pricing rules-related needs</i></p> <ul style="list-style-type: none"> <li>• Federal and State Anti-kickback statutes</li> </ul>	<p><i>Above plus...</i></p> <p><i>Price Benchmarking-related needs</i></p> <ul style="list-style-type: none"> <li>• ASP may not apply</li> </ul> <p><i>Non-pricing rules-related needs</i></p> <ul style="list-style-type: none"> <li>• Federal and State Anti-kickback statutes</li> </ul>	<p><i>Same as above</i></p>	<ul style="list-style-type: none"> <li>• <i>Same as above</i></li> </ul>

**Table 3.2:** Summary of Needed Enablers – One-Year, Milestone-Based Contract Solution

contain thousands or even millions of transactions. Durable/potentially curative therapies may have only a handful of transactions in a period. Milestone-based contracts also separate the initial treatment billing period from the rebate period by up to a year. The price benchmarking systems assume that the rebates occur within 36 months and that the large number of transactions would smooth any distortions. The example 12-month milestone falls within this window if structured as a rebate, but the small number of patients in any single period would likely introduce volatility. The result is the potential for radical price reductions, even to the extent of requiring manufacturers to provide their products for free to all Medicaid patients, rather than being a rebate to a prior transaction.

- Medicaid Best Price regulations:* Medicaid Best Price (MBP) rules were put into effect to ensure that the Medicaid program serving the poorest patients always receives the lowest price for a given medicine. As currently written, a performance-based contract negotiated with a commercial payer, Medicare or a managed Medicaid plan that results in a realized average performance rebate greater than the standard, mandatory Medicaid rebate of 23.1% would create a new floor that applies to all Medicaid sales for that quarter, regardless of whether Medicaid committed to a performance guarantee contract and regardless of how well the product performed for Medicaid patients. The price reporting mechanics were also established assuming a significant number of patients would obtain the medicine in each quarter in each reporting geography. Gene therapies for rare conditions may only have a single patient in a state in a quarter whose unique, unfortunate, full rebate-triggering experience could then set the price for all Medicaid patients. The great majority of those Medicaid patients will receive higher benefits and so would earn no or lesser rebates in the non-Medicaid milestone-based contract. In an extreme example, if a developer were to offer commercial payers a full rebate in the event of non-performance and a commercial patient actually triggered the rebate in the same period as the treatment and was the only patient in a reporting state, then the Medicaid price reporting system would show the \$0 net price as the new MBP. This could require the company to provide the therapy for free to all Medicaid plans, even for those patients for whom the medicine performed well. The current best price reporting rules are not flexible enough for the new payment model innovation that is being contemplated, especially for therapies treating rarer conditions. Three potential solutions to this challenge are: substituting MBP with a mechanism that Medicaid simply be offered the same milestone-based contract
- terms; calculating the MBP at a national level; or calculating the MBP for all patients at the end of the contract period.
- 340B ceiling prices:* Section 340B of the Public Health Service Act requires manufacturers to provide discounts on Part B, outpatient, drugs to thousands of covered entities (CEs) for ALL their purchases, as a condition for Medicaid coverage of the drug. The price to CEs, according to the statute, cannot exceed the “340B ceiling price”. Medicaid best prices become 340B ceiling prices two quarters later, thus carrying through the challenges described above. Inpatient-delivered products that are purchased by the CE and then bundled with services when billed to the insurance company also receive the 340B price. Thus, the price benchmarking challenges of MBP extend outwards to non-Medicaid outpatients and even non-Medicaid inpatients based on Medicaid outpatient drug price benchmarking.
- 5i AMP (Average Manufacturer Price):* AMP, the price developers charge wholesalers and pharmacies net of discounts, is used as part of the Medicaid Best Price calculation. The Medicaid Drug Rebate Program regulations separately designate drugs that are inhaled, infused, instilled, implanted or injected (“5i drugs”). Many, perhaps most, cell and gene therapies fit this category. Unlike regular AMP, 5i AMP requires developers to additionally include any alternative payment contracts, not just “gross-to-net sales” discounts, it might strike with their purchasers. As with MBP, any zero bill would pull down AMP immediately. In addition, for medicines with small numbers of patients, including the impact of alternative payment impacts can introduce significant pricing volatility. 5i AMP allows developers to smooth the threshold calculation over a rolling 12-month period to reduce volatility in AMP prices. For 12-month milestone-based contracts, however, the rolling 12 months still may not capture performance rebates properly, particularly in the first year and especially for medicines with small numbers of patients.
- ASP (Average Sales Price):* ASP is the average net sales price received by a developer from ALL purchasers, not just retail pharmacies and wholesalers as used in AMP. ASP is used by some payers to reimburse providers for medicines purchased and administered by the provider (buy and bill). As currently written ASP takes into account performance rebates that are paid to payers. If a provider purchases a medicine up front and then is reimbursed at a later date as a function of ASP, there can be a difference between the price the provider paid and its

reimbursement. The small patient numbers of many of these treatments will also increase the volatility of ASP and magnify the risk of payment volatility to providers.

- **Anti-Kickback Statute (AKS):** Current AKS rules can hinder milestone-based contracts that connect rebates to later outcomes. Current rules do not explicitly place milestone-based rebates in the safe harbor that includes traditional rebates. Milestone-based rebates might therefore be categorized as inappropriate payments resulting in significant penalties. The AKS intends to prevent exchanges of value between developers and other parties to inappropriately induce those parties to prescribe the developers' products. Federal and state anti-kickback laws, which carry significant financial and criminal penalties, prohibit persons from knowingly and willingly offering, paying, soliciting or receiving any remuneration in return for referring or recommending an item or service that is reimbursable, in whole or in part, under a federal health care program (e.g., Medicare, Medicaid). The definition of remuneration specifically includes rebates, coupons, and other discounts. There are a number of exceptions and safe harbors that protect certain arrangements from prosecution under the AKS, even if the arrangement otherwise might constitute a technical violation of the statute. However, there is no specific safe harbor for value-based performance guarantees and elements of existing safe harbors do not fully address the situational cases. Today, a performance guarantee to a provider that included products administered to a Medicare or a Medicaid patient could run the risk of being considered an inducement for use. In addition, the AKS limits developers' ability to compensate others to establish the programs needed to administer the milestone contract such as outcomes tracking. AKS interpretations may also prevent developers from participating in funding programs to support treatment success, such as education, and case management-programs. While typically supported by payers today, developers sharing outcomes risk could be ruled to have direct interest in such programs again resulting in significant penalties. More detailed examples of this barrier can be found in the paper by Duke Margolis on [Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products](#). Explicit safe harbors for milestone-based contract performance payments, administration infrastructure and provider/patient support programs are critical to facilitate their creation and use.

Contract negotiation and implementation can raise two additional legal and regulatory areas that precision financing approaches must navigate and for which some enabling policy changes are already occurring or may be needed:

- **FDA Regulation of Manufacturer Communications:** A manufacturer may only communicate information about its medicine that is consistent with FDA communication guidelines. For instance, communicating information to physicians not included in the drug label has historically been considered "off-label promotion" and subjects the offending company to financial penalties and promotional restrictions via misbranding actions by the FDA. Similarly, a developer is generally forbidden to communicate (promote) to physicians and the public about a medicine prior to its formal FDA approval. The FDA has recently announced<sup>3</sup> and released guidance<sup>4</sup> to enable value-based arrangements of which the FoCUS solutions may be considered a sub-type. The most recent guidance allows developers greater and earlier communication with payers prior to approval and for health care economic information (HCEI), such as alternative endpoints, that are not on the drug label, as long as such information is presented with "a conspicuous and prominent statement describing any material differences between the health care economic information and the labelling approved for the drug"<sup>4</sup>. These FDA regulatory interpretation clarifications mitigate but may not eliminate three community concerns. First, that contracting discussions for alternative payment models may begin prior to launch – when labels are not yet available. Second, that developers and payers may negotiate contracts that employ measures or patient sub-populations not specifically included in the clinical label. Third, that developers may share healthcare economic information with designated stakeholders such as a payer or formulary committee considering coverage and reimbursement for the medicine.
- **Privacy Policy and HIPAA Compliance:** The milestone-based contract solution requires tracking patient outcomes over time and sharing that information among payers, developers and any other parties engaged in the contracting, data acquisition and adjudication of that data for purposes of triggering a performance rebate or other value exchange. These data transfers must be designed and implemented in compliance with HIPAA<sup>iii, 5</sup> that aims to protect the privacy and security of patient health information. Several solutions may be possible. HIPAA anticipates that "business associates" may have access to patient level information for proscribed uses

<sup>iii</sup> Health Insurance Portability and Accountability Act of 1996 as interpreted by HHS in conjunction with the 2009 Health Information Technology for Economic and Clinical Health Act, and

the Genetic Information Nondiscrimination Act of 2008 and their amendments.



including reimbursement adjudication, but also assume responsibilities and potential liability for doing so. Patients may need to be informed and perhaps explicitly consent to such sharing and use. A third-party intermediary might facilitate the data collection.

### Operational Enablers

The FoCUS participants identified core payer-developer contract terms, data capabilities, provider reimbursement and organizational capacities such as adequate personnel with appropriate skills as critical operational enablers for a one-year, milestone-based contract solution. Box 3.3 describes current examples of durable therapy milestone-based contracts.

#### Box 3.3: Example Milestone-based Rebate Offers

Novartis proposed in 2018 a voluntary outcome-based agreement for KYMRIA<sup>®</sup>'s pediatric Acute Lymphocytic Leukemia (ped-ALL) indication, under which it does not bill participating treatment centers for the cost of KYMRIA<sup>®</sup> when a patient does not achieve a complete remission (CR) or CR with incomplete blood count recover (CRi) 28 to 35 days following infusion.

Spark Therapeutics for LUXTURNA<sup>™</sup> offered to pay rebates to commercial payers if a patient's outcomes fail to meet a short-term efficacy (30-90 days) or longer-term durability (30 months) measure based on full-field light sensitivity threshold (FST) testing scores, with a baseline to be established for each eligible patient before administration of LUXTURNA<sup>™</sup>.

**Core payer-developer contract terms** for a milestone-based contract solution are:

- **The relevant outcomes measure** that will trigger the milestone rebate, including how it will be specifically measured, at what milestone (clinical event or set time) and by whom. For cell and gene therapies, we expect any of many data sources could be employed: lab-measured values, clinical assessments, patient-reported outcomes and/or claims data. These are not always easy to align on or measure objectively. An example set of potential metrics considerations building on the oncology work at NEWDIGS/FoCUS may be found in Box 3.4.
- **The outcome measure threshold** that will trigger the rebate. It is possible to envision a variable range in which the rebate changes according to the value or by defined value partitions.
- **The definition of the covered population** must be clear regarding not only initial patient eligibility but also

what occurs if a patient initially receives out-of-network care, is covered by more than one health plan, or receives care through a sub-contracted carrier.

- **The patient mobility approach** must also have clear rebate mechanics. Even with a short milestone duration of one year, after initial treatment a patient may leave the initiating health plan product, die, or otherwise change status.
- **The rebate basis** may be for each patient or for the population. And if for the population, the method for both aggregating the individual patient values and triggering the population rebate such as average, median, quartile or other technique must be defined. A population approach must also clarify if every patient must be tracked or a sample of patients. The terms should also define what occurs if a patient's outcome data is missing – examples include forfeiting the rebate, extrapolating from the population or some other alternative method to use for calculating the rebate.
- **The rebate amount and how it will be structured.** The amount to be rebated must be specified as a fixed amount or a percentage of some verifiable amount. The rebate structure including the form (rebate, credit, or other), timing (net 30 days, etc.), triggering notification and receiving party (payer, provider, pharmacy, reinsurer) must all be detailed.
- **The contract term** will likely extend for multiple years even as the milestone for any single patient, in this example, is a year. The contract must specify whether the last patient to be considered for rebate must reach the final milestone payment date prior to the end date of the overall contract agreement or simply receive treatment prior to term end date with the appropriate clauses surviving until the process completes.

### Data capability enablers

The data required to administer a milestone-based contract solution are significant. The data sources and responsible parties for obtaining, sharing and adjudicating that data require delineation among the participants, either in the core contract above or in concurrent side agreements among the parties. Key data include:

- treated patient identification;
- eligibility confirmation;
- initial treatment date(s) that trigger payments to developer, provider and patients;



### Box 3.4: Example Metrics Considerations & Oncology Case Example

Choosing the right clinical performance measures is important and can be challenging. While the specific metrics will vary across disease states and products, a few general principles hold. Metrics should be:

- ☑ **Meaningful**
  - Matter to patients, or strongly correlate to outcomes that matter to patients
  - Strongly relate to treatment effectiveness
- ☑ **Measurable**
  - Part of routine care (avoid added cost, consistently available)
  - Offer clear and unambiguous results
- ☑ **Timely**
  - Outcomes likely to happen during a reasonable contract duration
- ☑ **Robust**
  - Insensitive to potential biases, such as patient selection, interpretation of test results, availability of test results, and other confounding variables
- ☑ **Accessible**
  - Results accessible to both parties at no or low cost
  - If EMR data is required, the metric should be in structured data rather than free text
- ☑ **Predictable**
  - Evidence supports an estimate of expected success rates and expected variation in success rates
  - All parties should be able to make informed decisions about risks and rewards

For example, in Diffuse Large B-Cell Lymphoma (DLBCL) and Acute Lymphocytic Leukemia (ALL) treated with CAR-T, the NEWDIGS/FoCUS Oncology Team proposed use of overall survival at 6 & 12 months as the simplest solution.

- Overall Survival (OS) at 6, 12 and 24 months would be unambiguous, meaningful, measurable, and could be based on predicted survival rates from clinical trials.
- Quality of life, on the other hand, though important, is not routinely measured.
- Bone marrow transplant after CAR-T, though costly, may represent treatment failure in some patients but a therapeutic strategy in others.

Even the “simpler” option of overall survival creates challenges. About a quarter of commercial plan members change health plans each year.<sup>6</sup> Thus, even with the 6- and 12-month timeline, many patients will no longer be members of their original health plan when the outcome can be measured. Data on those ex-enrollees may no longer be available to the contracting payer. The NEWDIGS Oncology Working Group therefore conceptualized a model that relied only on continuing members and was calibrated to have a neutral outcome if the product performed as expected. Better survival could lead to bonus payments to the developer, while lower-than-expected survival could lead to rebates to the payer. Neither party would be systematically disadvantaged by lost-to-follow-up patients, since their performance would be assumed to follow the predicted path.

- outcome assessment date and results that trigger rebate calculation, provider follow-up care payments and perhaps additional patient co-pays; and
- adjudication and data audits in case of disputes.

The proposed FDA long-term follow-up requirement guidelines issued July 11, 2018 are unlikely to include the needed data.<sup>7</sup> The proposed guidelines are risk-based and focused on clinical study designs to assess adverse events for a maximum of 15 years with only the first 5 years requiring annual examinations. The FDA proposed guidance does not require that these studies include all treated patients nor that the studies include efficacy or other outcome metrics. Thus,

the data that developers are required to collect could provide infrastructure that could be leveraged to capture the performance data needed for the milestone-based contract solution. That said, the infrastructure created to capture the long-term safety data, could offer some opportunity for also tracking performance data.

Centralized or distributed data solutions can be envisioned. Roles for developers, payers, patient advocacy groups, providers, distribution channel participants such as specialty pharmacies or pharmacy benefit managers are all possible as either data contributors or managers of all or part of the data system. National or global systems could be built or smaller,

focused data systems for regional or even individual contracts might emerge as practical, immediate stop-gaps. Data solutions for each product may prove best or perhaps databases that cover all products for an indication or disease area such as blood disorders might be constructed to create administrative efficiencies and enable greater learning. Whatever approaches milestone-based contract participants use, administrative systems must not only store and report data but also facilitate its collection. Beyond the standard technical and legal issues, these data systems must ensure that “healthy” patients continue to return for tracking post treatment. Adding complexity to this is that patients may switch plans and providers, requiring potentially complex multi-stakeholder agreements to support patient tracking over time.

### ***Provider reimbursement enablers***

Appropriate alternative mechanisms for adequate provider reimbursement for administering and follow-up care for durable therapies were also identified by FoCUS participants as critical for patient access. This was especially noted for inpatient delivered durable therapies that do not employ buy-and-bill provider mark-ups. See Box 3.5 for a description of the challenges for durable therapies requiring inpatient administration under DRG capitated payments.

#### **Box 3.5: Durable Therapies and DRGs**

Hospital inpatient procedures are reimbursed through diagnosis-related group (DRG) codes, a statistical approach to grouping inpatient stays for the purposes of reimbursement. Hospitals are paid a fixed rate for inpatient services consistent with the DRG group assigned to a given patient. This fixed rate typically includes the cost of any medicines or services provided as part of the stay. New technologies that are more expensive than existing technologies may raise a hospital’s costs relative to the average costs included in a DRG to which that treatment may be assigned. Hospitals will have concerns about using new technologies if reimbursement within the DRG system is not sufficient as they will bear the financial risk. Applications for supplemental New Technology Add-On Payments can be made once a year after product launch, but even if accepted these do not cover all products and will not cover the full cost of those therapies. There is also additional time delay following the granting of an application for add on payment. For additional details on the CAR-T situation the following [Bloomberg article](#) or an [overview](#) by the American Society of Bone Marrow Transplant may offer insight.

In the **outpatient setting**, providers may find existing infusion, office visit and other follow-up reimbursement codes sufficient for their services. And to the extent that the providers do not already utilize other products that provide a buy-and-bill, 340B or similar margin no disincentive will be created. If providers and payers have historically shifted some reimbursement for medical or ancillary services to such product margins (such as those for hemophilia factors supporting hemophilia center operating expenses) renegotiation of rates, codes or both may be required.

In the **inpatient setting**, in addition to the relevant pricing issues detailed above, inpatient use of cell and gene therapies raises questions of adequacy of diagnosis-related group (DRG) codes and new technology add-on payments, as well as the availability of coding to support provider adoption of the treatments. While important and perhaps exacerbated by durable therapies, these issues do not pertain specifically to performance guarantees and alternative payment mechanisms and therefore are not further discussed here beyond Box 3.5. Similarly, the challenges associated with the lack of reporting and billing codes for hospital services that are specific to new therapies have been noted but are not further detailed.

### ***Organizational capacity enablers***

From an operational perspective, performance-based contracting requires effort and expertise. Not all payers, providers and developers may have these capabilities in-house. This may inhibit the adoption of milestone-based contracting and other precision medicine solutions even as it creates opportunities for intermediaries to provide the needed skills as out-sourced or partnered services.

Designing and implementing successful performance guarantees requires:

- **Experienced staff:** Design and negotiation of these types of contracts is often complex. It requires strong data management capabilities, as well as statistical and actuarial analysis, to ground the negotiations in an understanding of the patient base. All stakeholders may not have the required in-house expertise. Education and support for organizations, especially smaller ones, developing such capabilities could be helpful. For example, CMS could provide grants to State Medicaid entities to support the establishment of such capabilities such as they have done to support establishing electronic health record systems and capitation arrangements.
- **Resources:** Negotiating such contracts takes time, as does gathering and analyzing the follow-up data. Stakeholders may not be interested in dedicating resources to performance-based contracting. Indeed, a targeted payer research study conducted by MIT

NEWDIGS in 2017 found that over half of payers indicated that they would use current, traditional tools to contract for durable/potentially curative therapies.<sup>8</sup>

It should be noted that a performance guarantee operationally could take several forms: payer-developer; provider-developer; payer-provider; or payer-provider-developer. The first three of these require adaptations of existing processes – payer-developer contracts applied in new settings (e.g., inpatient), or provider-developer / payer-provider contracts with new performance elements. The latter three-way contract was not developed because it is not a typical model today and the complex contracting appeared prohibitive compared to alternatives.

### Risk Management

Three issues have been identified by the working group in terms of risk management: administration risk, patient mobility, and actuarial risk. FoCUS did not consider counterparty credit risks such as bankruptcy.

**Administration Risk and Centers of Excellence:** The outcomes of some durable/potentially curative therapies could depend on providers' skill in patient selection and therapy administration. The FoCUS oncology Target Area Group in particular highlighted the importance of selection of appropriate centers of excellence, defined as "...a program within a healthcare institution which is assembled to supply an exceptionally high concentration of expertise and related resources centered on a particular area of medicine, delivering associated care in a comprehensive, interdisciplinary fashion to afford the best patient outcomes possible"<sup>9</sup>. The term centers of excellence also applies to payer centers of excellence and networks of certified centers created by developers for their clinical trials and product launch. For example, for CAR-T, payers have leveraged the developer-certified centers based on the clinical trial sites for initial patient access. Payers and developers have then collaborated on expanding the network of centers to increase patient access. Certified centers and Centers of Excellence could also help enable consistent data collection and outcomes measurement.

**Patient Mobility:** Patients moving across plans is a risk from the perspective of one payer potentially bearing the costs and another reaping the benefits over time. Patients who move across plans and providers also create a potential risk from the perspective of tracking patient outcomes over time. While both are technically possible within a one-year period, this issue is discussed more fully in the section on the next multi-year performance-based annuity model, which suffers greater risk of patient movement.

**Actuarial Risk:** Existing reinsurance and stop-loss products help payers and employers manage single-year actuarial risk. Insurance companies include a percentage cost factor in their premiums to account for unexpected costs in a premium year. Reinsurance purchased by an HMO or insurance company allows them to pass all or part of their risk to another insurance company. Self-funded employers purchase stop-loss insurance to protect against very large claims on any one person (Specific Stop Loss) or higher than expected claims overall (Aggregate Stop Loss). In a one-year period, such as posited in this Milestone-Based Performance Guarantee model, we expect these tools can work effectively to mitigate actuarial risk, provided that reinsurance companies do not exclude such transformational treatments or the patients that could benefit from them from their offerings. Additional discussion of actuarial risk considerations may be found in the performance-based annuity section below.

### Conclusions and Limitations

This milestone-based contract solution is most appropriate for products with upfront uncertainty as to initial treatment success and for products whose one-year performance is indicative of their longer-term performance. It also offers benefits in terms of guaranteeing product performance within the first year. Finally, it is the most practical with the fewest legal and regulatory hurdles to overcome, combined with relatively straightforward operational mechanics.

The one-year milestone-based contract solution only partially addresses therapeutic performance risk due to its short period. It does not address either of the other two financial risks: the payment timing mismatch between the upfront payment and the later year benefits and the actuarial risk from an uncertain number of patients, especially for rare conditions.

This milestone-based solution, therefore, is less appropriate for products whose main value depends upon multi-year durability of efficacy, which many gene therapies are expected to offer.

Also, a one-year, milestone-based performance contract does not address either payer actuarial risk or any payer challenges with payment timing/affordability within a one-year period. Therefore, we may see payers managing those risks by implementing measures to limit patient access [e.g.: restrictive authorizations, overly strict interpretation of the product label and clinical studies] or identify third-party service providers such as reinsurers as complementary financial strategies to enable patient access.

**SOLUTION 2 – SPREADING THE SURGE: PERFORMANCE-BASED ANNUITIES**

Durable/potentially curative therapies create three financial challenges:

1. **Payment timing:** Therapies can involve substantial upfront payment for multiple years of therapeutic benefit.
2. **Therapeutic performance risk:** Real world efficacy and durability are uncertain at the time of initial regulatory approval and market launch.
3. **Actuarial risk:** The number of eligible patients in a payer’s population may be uncertain and could vary significantly from period to period.

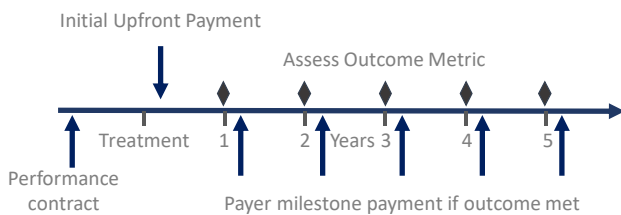
The mismatch between the upfront cost of a durable/potentially curative therapy and the multi-year benefits from that therapy underlies many of the financial challenges these therapies generate compared to traditional medicines for chronic conditions that are taken repeatedly over time.

As the name implies, the performance-based annuities solution directly addresses that mismatch by spreading the payments over time and addresses the uncertainty of a therapy’s efficacy across all patients and across many years.

Finally, the performance-based annuity also provides some actuarial risk mitigation by spreading the upfront cost over time, if the payments are structured as contingent payments rather than non-performance rebates.

**Description**

The performance-based annuity solution contains a core transaction between the payer and developer as illustrated in Figure 3.3 below. In this example of the model, an up-front payment of some portion of the product cost is made, as well as a commitment to further value exchanges with the developer every year for five years, triggered by outcomes measures. Those future value exchanges take the form of payments to the developer from the payer if the outcome threshold is achieved. For simplicity, we also assume that



**Figure 3.3:** Performance-based Annuity: Payer to Developer Structure

after the first outcomes failure, no further testing would be done, and future payments would also be forfeit.

This approach borrows and adapts techniques from the bond, mortgage and insurance markets by establishing a multi-year payment schedule with uncertain future payments based on product performance. When structured as deferred payments, it is not dissimilar to a standard mortgage or car loan with early redemption, although here the redemption is driven by product performance rather than early sale of the asset by the consumer or consumer default. In addition, the solution contains features designed to address provider and patient financial risks.

As with the milestone-based contract solution, this solution could be alternatively structured as a transaction between a Provider (hospital or physician office) with either the Payer or the Developer, Specialty Pharmacy or Wholesaler depending on the care setting and the medicine distribution model. For simplicity, we will not discuss these alternative structures.

The list below summarizes the financial goals and the core elements of this solution:

**Financial goals**

- Mitigate upfront payment and patient backlog surge effect on **payer** income statement and cash flow by spreading payments over time
- Share performance risk between **payer** and **developer**
- Alleviate some **payer** actuarial risk by payment spreading. In effect an ‘on demand’ stop-loss/reinsurance premium
- Remove financial risk from **providers** by eliminating buy-and-bill inventory risk and replacing the mark-up margin with negotiated fees for comprehensive patient care and data reporting
- Reduce **patient** out-of-pocket cost barriers

**Core elements of the Performance-based Annuity solution**

- **Five-year payment structure** between payer and developer for each treated patient
- **Annual installment after initial payment** on the core contract based on a feasible performance metric.
- **Patient co-insurance/co-pay:** limited to first year, with a design option that these be waived because appropriate access will be assured through strict eligibility screening by providers and payers and even high deductible caps provide a *de minimus* fraction of the cost, while being a barrier to access.

Multi-year performance-based annuities while solving some financial challenges of performance risk, payment timing and even some actuarial risk, introduce other operational and

regulatory challenges, which are further discussed below. They include:

- **Patient mobility** among plans introduces either challenges of sharing data across plans or an early termination feature to the annuity.
- **Medicaid best price** regulations and reporting mechanisms, as well as other pricing regulations, were not designed to accommodate multi-year payment approaches and may need modification, clarification of reporting approaches or both.
- **Stop-loss or reinsurance disruption** may occur for first-line payers if annuity payments fall below deductible levels or patients are lapsed out of policies in later years.
- **Accounting cost and revenue recognition** rules may limit the financial benefit by requiring the payer to accrue the full amount of the contract in the first year—thus eliminating the income statement benefits of spreading of the financial costs over time to better match benefit accruals. The developer may face similar restrictions on revenue recognition.
- **State multi-year contract prohibitions may prevent Medicaid participation**, although state cash accounting rules avoid the accounting recognition challenge above. Medicaid MCOs may however participate.

This solution balances matching benefits with payments taking real-world issues such as feasibility and diminishing returns into consideration. For instance, the term in this example has been limited to five years rather than the full life of the product's potential effect, which indeed might be a lifetime. Practical issues of measuring therapy performance easily and objectively and the diminishing benefit to payers of long-lived obligations given patient mobility contributed to this choice. In practice, parties will modify the terms and conditions of this general approach to best meet their circumstances.

### Enablers for the performance-based annuity solution

FoCUS participants identified public policy, operational and risk management enablers required for the performance-based annuity solution, just like for the milestone-based contract solution. Many of the issues overlap. Table 3.3 summarizes the enablers with the new elements or those repeated elements that are significantly more complex or important highlighted in bold. In our amplified description below, we do not repeat the elements already detailed in the previous section but concentrate on differences from the previous example or new elements that must be addressed.

The enablers of a performance-based annuity are summarized below. As with milestone-based contracts, the

elements differ slightly between the payer-purchase and provider-buy-and-bill situations.

### Needed Legal and regulatory enablers

Government price benchmarking and anti-kickback regulations do not clearly specify how to treat multi-year, performance-based annuities. The key incremental legal/regulatory issues (beyond those previously described in the one-year performance guarantee model description above) are:

- **Price Benchmarking:** the effects of current Medicaid Best Price regulations, 340B ceiling price, 5i AMP and ASP policies and mechanics have been described above in the one-year milestone-based solution section. The multi-year performance-based annuity model raises these additional issues:
  - **Initial payment could be considered the total payment.** Current reporting mechanics track invoice transactions and do not disclose the full contract price or terms. Thus, the first payment could be construed as the full price and so establish a new (total) best price.
  - **CPI penalty risk.** The first payment could also set an artificially low baseline AMP, which cannot be restated for later data. With AMP then increasing over time, the product could be subject to a significant artificial inflation penalty for price increases that outpace inflation as part of the Medicaid Best Price calculation.
  - **Historic AMP adjustment for future performance failure.** If the total potential payments in a multi-year agreement are reported, first quarter base AMP could include patients for whom the therapy is ineffective at some point in the future. Current guidance does not clearly address whether to adjust the historical base AMP entry for performance-based rebates or contingent performance payments, especially for those beyond the usual 36-month restatement period.
  - **The time value of money** is not considered in current guidance regarding how to adjust future payments in determining the initial period effective price.
  - **Three-year price reconciliation.** Current regulations allow manufacturers to restate best price and AMP for three years after the initial filing deadline (30 days after the end of the quarter). This term was set to balance the need for accuracy, the effort required to undertake longer-term reconciliation and the need for states to have more certainty in their rebate numbers for budgeting purposes. Price reporting for performance annuities with terms greater than three years are unclear and so effectively discouraged.



Scenario	Legal/Regulatory		Operational	Risk Management
	Outpatient	Inpatient		
<b>No buy-and-bill (payer purchases medicine)</b>	<p><i>Revised Price Benchmarking rules</i></p> <ul style="list-style-type: none"> <li>Medicaid Best price regulations</li> <li><b>MBP base AMP</b></li> <li><b>MBP CPI</b></li> <li><b>MBP 3 year reconciliation period</b></li> <li>340B ceiling prices</li> <li>Impact on 5i AMP</li> <li>Impact on ASP</li> </ul> <p><i>Other Policy Enablers</i></p> <ul style="list-style-type: none"> <li>Anti-Kickback Statute safe harbor inclusion</li> <li>FDA Manufacturer Communication Guidelines encouraging value-based payment arrangements</li> <li>Privacy Policy: HIPAA-compliant mechanisms for patient outcomes collection &amp; sharing among contracting parties</li> </ul> <ul style="list-style-type: none"> <li><b>Accounting recognition rules</b></li> </ul>	<p><i>Revised Price Benchmarking rules</i></p> <ul style="list-style-type: none"> <li>Medicaid Best price regulations</li> <li>340B ceiling prices</li> <li>Impact on 5i AMP</li> <li>Impact on ASP</li> </ul> <p><i>Other Policy Enablers</i></p> <ul style="list-style-type: none"> <li>Anti-Kickback Statute safe harbor inclusion</li> <li>FDA Manufacturer Communication Guidelines encouraging value-based payment arrangements</li> <li>Privacy Policy: HIPAA-compliant mechanisms for patient outcomes collection &amp; sharing among contracting parties</li> </ul> <ul style="list-style-type: none"> <li><b>Accounting recognition rules</b></li> </ul>	<p><i>Contract design features</i></p> <ul style="list-style-type: none"> <li>Relevant outcomes measure</li> <li>Outcome measure threshold</li> <li>Definition of covered population</li> <li>Patient mobility approach</li> <li>Annuity basis: by patient or population</li> <li>Annuity amount &amp; structure</li> <li>Contract Term</li> </ul> <p><i>Execution enablers</i></p> <ul style="list-style-type: none"> <li><b>Data capabilities, sources, roles and architecture</b></li> <li>Provider reimbursement mechanisms</li> <li>Organizational capacity</li> </ul>	<ul style="list-style-type: none"> <li><i>Center of Excellence</i></li> <li><b>Patient Mobility management</b></li> <li><b>Actuarial risk via Reinsurance / Stop Loss</b></li> </ul>
<b>Buy-and-Bill (provider purchases medicine and bills payer)</b>	<p><i>Above plus...</i></p> <p><i>Price Benchmarking-related needs</i></p> <ul style="list-style-type: none"> <li>Impact on ASP (Average Sales Price) if included in Medicare Part B</li> </ul> <p><i>Non-pricing rules-related needs</i></p> <p>Federal and State Anti-kickback statutes</p>	<p><i>Above plus...</i></p> <p><i>Price Benchmarking-related needs</i></p> <ul style="list-style-type: none"> <li>ASP may not apply</li> </ul> <p><i>Non-pricing rules-related needs</i></p> <p>Federal and State Anti-kickback statutes</p>	<p><i>Same as above</i></p>	<ul style="list-style-type: none"> <li><i>Same as above</i></li> </ul>

**Bold** = new elements or complexity versus one-year milestone-based performance guarantee

**Table 3.3:** Summary of Needed Enablers – Five-Year, Performance-Based Annuity Solution

In response, to date, developers have limited proposed contract approaches to payers with initial full payments and performance rebates within three years where the reporting rules and safe harbors from the Anti-Kickback Statute are clearer. For added compliance safety, developers have proposed limiting the total future rebates

to the standard upfront Medicaid discount. This approach can shift risk to the payer for ‘clawing back’ the performance discount and have the payer bear the time value of money (discounted value) which reduces the effective potential discount. These compliance issues limit the range of financial structures and their effectiveness-



especially by limiting the structure of performance triggered payments to developers.

- **Accounting recognition rules** may prevent payers from benefiting from payments spread over time. Current medical loss accrual accounting rules may require insurers to recognize all but the ‘uncertain amount’ of performance-based annuity in the initial treatment or payment period. A strict interpretation could consider even contingent future payments ‘certain’ if the therapy performance is considered highly likely or permit only a minimal medical expense deferral. Thus, a performance-based annuity may improve cash flow, but minimally reduce the initial income statement impact for the payer due to accrual accounting recognition rules, complicate insurance reserve requirement calculations and perhaps require establishing additional balance sheet accounts.

In contrast, State Medicaid programs operate under cash accounting rules which recognize costs at the time of cash outflow, and thus could benefit to a greater extent from the spreading of payments. The challenge for States is that many also operate under prohibitions to defer costs over multiple years. State Balanced Budget laws may exacerbate the challenge. Solutions may lie in creative collaboration among a State, its Managed Medicaid vendor(s), reinsurance firms and the developers to leverage their differing accounting situations to allow each to meet their recognition goals.

The developer will face similar but complementary challenges regarding to what extent to recognize revenue immediately or to defer into future periods. Some firms may prefer smoothing the revenue over multiple years while others may attempt to maximize the amount reported in the initial period.

## Operational Enablers

The FoCUS participants identified core payer-developer contract terms, data capabilities, provider reimbursement and organizational capacity as critical operational enablers for the performance-based annuity solution just as for a one-year, milestone-based contract solution. The extended time period exacerbates the challenges and suggests multi-stakeholder, more scalable solutions may be required.

**Core payer-developer contract terms** for performance-based annuity solutions are similar to those for milestone-based contract solution. The relevant outcome measure, performance threshold, covered population definition, installment structure and term must all be pre-defined.

- **Relevant outcomes** measures could emphasize functional, quality of life metrics as well as short term

biological, clinical metrics. Surrogate metrics easily observed in claims data such as reversion to prior therapies, or advancement to new therapies may be applicable.

- **The outcome measure threshold** that will trigger the installment or contingent payment may need to anticipate changing effectiveness over time with more variable rather than binary all-or-nothing financial designs.
- **Contract terms** will be longer to cover the annuity period. This increases operational complexity immensely over time if the metrics, payment schedules and included populations change frequently.
- **The patient mobility approach** must anticipate higher numbers of patients leaving their initial, treating plan. The options are described in more detail in the Risk Management section below.

### **Data capability enablers**

As described above for the milestone-based contract solution, data required to administer these longer-term arrangements are significant and unlikely to be mandated by FDA or other federal agencies.

Tracking patients over the longer annuity time presents added challenges – particularly for patients with therapy-transformed health who may become less connected to a specialist. Patients may not prioritize the ongoing testing and tracking required for performance guarantees. Incentives of waiving co-pays, or even perhaps refunding a portion of a patient’s initial deductible if they undergo evaluation may be required to obtain the needed performance data.

The longer period may also lead stakeholders to recognize the collaboration opportunities to reduce data tracking costs by developing mechanisms that include multiple products in an indication, and perhaps coverage for broader disease areas served by the same providers. For example, blood disorders from hemophilia to sickle cell anemia and beta thalassemia among others might benefit from a multi-payer, multi-developer, multi-provider system for tracking patient outcomes. The CIBMTR® (Center for International Blood and Marrow Transplant Research®) and its outcomes database of every allogenic transplantation and many autologous transplantations may be a model for other areas.

Policies supporting precision financing will therefore need to provide infrastructure to facilitate the capture, sharing, and quality control of patient data as well as provide clear guidance on the type of data that can be captured and shared.

### **Provider reimbursement enablers**

It would appear unlikely that providers would willingly assume a multi-year performance risk on the therapy, their medical services or both. It would require them to either execute separate contracts with payer and developer or enter

into a three-way agreement with the provider facing risk from both parties beyond their control. In addition, patient mobility risk would also likely shift to the provider who has the least resources to track the patient when they do.

Negotiating appropriate fees for the associated patient identification, qualification, therapy administration, adverse event management, follow-up care and data collection activities seems in the best interests of the provider as well as the other stakeholders. Implementing the approach may require increased agility for issuing new billing codes, adjusting DRG rates for the therapy used and explicit recognition by all of the provider services previously funded by buy-and-bill, 340B and similar margins.

### Risk Management

From a risk management perspective, a multi-year performance-based annuity raises additional challenges, and even opportunities, in the areas of patient mobility and actuarial risk.

#### **Patient Mobility**

As noted in the one-year milestone-based performance guarantee section, patients moving across plans result in the first payer bearing the upfront therapy costs while a later payer reaps the downstream benefits. A multi-year performance-based annuity heightens these risks. Many patients do not maintain continuous insurance coverage with the same payer for extended periods<sup>iv, 6</sup>, and receipt of a transformative, durable therapy may actually increase patient switching by decreasing connections to their provider specialists and payer care coordinators, increased employment mobility of the patient or a caregiver due to improved health, or other factors. Receipt of a transformative, durable therapy may increase switching: for example, a child's improved quality of life may enable a family to leave Medicaid as a full-time caregiving parent may obtain paid employment. A multi-year term increases the likelihood of patient mobility across plans, which further increases the data tracking challenges of locating the patient, incentivizing new plans and providers to share the data and doing so in a regulatory compliant manner.

FoCUS participants have identified four approaches for dealing with patient mobility:

1. The initial payer retains responsibility for the terms of the contract
2. The contract is terminated, with an appropriate payment being made to recognize the expected value of future payments

3. The new payer assumes responsibility for the terms of the contract as negotiated by the initial payer
4. The new payer assumes responsibility in a world where contracts are consistent in structure between a developer and all payers, but individual payers independently negotiate different prices and levels of discounts with manufacturers.

The first and second approaches are most consistent with how today's initial performance contracts function in which the initial payer retains responsibility. The second option of negotiating a termination payment when the patient leaves the plan is the simplest but forgoes the option for future performance adjustments. Continuing the contract retains the performance option but at the added cost and complexity to the parties, especially the payer, to obtain patient performance data from the subsequent payer(s) and perhaps providers. This may require receiving patient permission at time of intake to obtain future medical information from them and their future insurers and providers.

A variation of the first option, when payer milestone payments are used, would have the initial payer continue the payments but without checking patient outcomes. This would retain the payment spreading feature and so avoid unplanned larger termination payments but eliminate the option for future performance-based payment reductions.

The third approach seems unlikely because payers are reluctant to adopt other payers' agreements for reasons of disadvantageous terms, administrative complexity and incentivizing patient shifting.

The fourth model offers better alignment of therapy costs with benefits, retains the option for performance-based adjustments, preserves payer contract confidentiality, and works in a voluntary market-based setting. Figure 3.4, right panel, illustrates the core concept of the receiving payer assuming the remaining payments and opportunities for payment reductions according to the **receiving** payer's contract with the developer. This structure incentivizes the receiving payer to collect the needed data while reducing the patient confidentiality concerns. The construct requires, however, that the contracts' length and payment distribution conform across plans to ensure fairness. Some actuarial risk for payers remains as patient mobility may fluctuate and not be completely equal in each period. With a larger indication, or as a portfolio of indications is included, this risk diminishes.

If patients move to a non-participating plan, the initiating plan retains the original two options, depicted in the right

<sup>iv</sup> In a study of commercially insured patients in Massachusetts, 26% switched insurers during a two-year period.

panel of Figure 3.4, to have a pre-negotiated termination payment or to continue the payments with or without patient outcomes information. If a patient transfers to a participating plan from a non-participating plan, the receiving participating plan does not make contingent payments. These mechanisms allow a voluntary sub-set of payers to more fully benefit from performance-based annuities even with significant patient mobility over the multiple year term.

FoCUS is catalyzing a pilot to test the feasibility of this fourth model in fully-insured commercial populations. At the time of writing, insurers with the majority of the fully-insured Massachusetts market and a developer anticipating a near-term regulatory decision for a gene therapy product are designing the structure, contracts and implementation mechanics anticipating a 2019 launch. Over time, FoCUS and the participants hope to expand the number of products, payers, and beneficiary segments included.

In practice, the optimal solution will vary depending on the circumstances. Amount of cost-offsets, level of uncertainty regarding treatment outcomes, and similarity of perspectives regarding coverage across payers may all be important in determining which specific solution would be preferred in a particular situation.

**Actuarial Risk**

The durable/curative therapy pipeline is dominated by products for conditions with relatively small, often orphan level prevalence. This makes it difficult to predict the number of cases any single payer, especially small payers such as self-insured employers, may encounter in a quarter or a year. This case variability combined with the upfront cost concentration resulting in value-based pricing expected to approach \$1M or more per case create significant, perhaps catastrophic, financial risk for small and even medium-sized payers. As noted above, reinsurance and stop-loss products help payers and employers respectively to manage actuarial risk today. Performance-based annuities appear to conflict with existing reinsurance and stop-loss products but simultaneously

provide partial ‘on-demand’ stop-loss insurance while also addressing performance risk, which stop-loss and reinsurance do not.

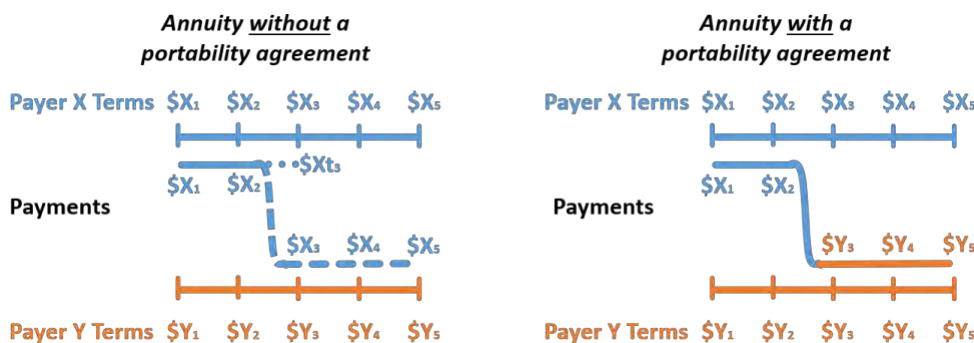
**Potential conflict with reinsurance and stop-loss policies.** Multi-year performance-based annuities conflict with these actuarial risk-management products purchased by payers that employ one-year contracts with perhaps a one-year extension option. Specifically:

- **Performance milestones may not be reached** due to the multi-year outcomes in the performance-based annuity and the single-year term of stop-loss and reinsurance contracts.
- **Deductibles may not be exceeded** because the individual year annuity payments do not reach the deductible triggers and the deductible may be applied separately to each year’s annuity payment. This could lower the quoted premium, but reinsurers may not recognize which clients employ performance-based annuities and so quote conservative, higher rates.
- **Later payments may be lasered out** of subsequent year policies. Stop-loss and reinsurance providers routinely exclude the known high costs of patients to adhere to the principle that these policies are to manage unknown financial risk, not to finance known costs. If performance-based annuity payments are included, patient exclusion (lasering) could occur or those now known payments will be included into the premium with a profit mark-up through experience rating.

Together, these effects could defeat the utility of the policies, especially for smaller health insurers and self-insured employers.

FoCUS participants identified these potential solutions:

- **New multi-year reinsurance and stop-loss products** to better match the performance-based annuities. This would have the benefit of preserving the



**Figure 3.4:** Patient Mobility Options for a Performance-based Annuity (Payer milestone payment if outcome met in each year)

typical structure for secondary insurance, namely that costs above a certain total level for a patient are borne by the secondary payer. Costs that occur in subsequent years that relate to specified treatments that were provided during the covered year would be included with other costs from the year of treatment to determine cost-sharing between the primary and secondary payers. The primary payer could maintain a share of the costs (proportional reinsurance) in order to create an incentive for appropriate decisions regarding downstream costs.

- **Carving out durable/curative therapies'** relevant payments into separate reinsurance coverage, either with or without ancillary medical costs. It could also be customized to each primary payer regarding included therapies and potentially even specific contracts.
- **Excluding performance-based annuities** because they innately provide 'on-demand' coverage (see below).
- **Reinsurers provide full coverage** by assuming the performance-based annuity contracting for the durable/curative therapies and charging an appropriate premium for assuming the capitated risk. See ORBM section below.

In all cases, it is critical to specify exactly what treatments and treatment costs are covered in the extended or new insurance coverage.

From the perspective of the reinsurers and stop-loss providers, any new products must have:

- **Sufficient monetary and operational scale** to make it worth developing, marketing, executing, and adjudicating. Only the largest and most expensive therapies that generate significant costs over time might be appropriate for carve-out. Over time a larger portfolio of products might also provide the required scale.
- **Accurately projectable costs** to enable appropriate pricing and risk management. This is not only related to the scale above, but also the historical experience required to make projections. It is not clear that clinical trial results will offer sufficient data for pricing targeted stop-loss for durable/curative therapies.
- **Minimal adverse selection risk** that primary payers could exploit, for instance by only buying the product when they knowingly expect higher patient numbers based on information undiscoverable by the issuer.

The interactions of stop-loss and reinsurance with performance-based annuities are complex. Practically, FoCUS participants suspect that the payer segments that use reinsurance and stop-loss products most heavily will be the slower adopters of performance-based annuities.

### ***Performance-based Annuities as 'On Demand' stop-loss/reinsurance***

Performance-based annuities in which payers make multiple, contingent payments innately provide partial reinsurance and stop-loss protection by spreading the cost over multiple years. Reinsurance finances the cost of a therapy across multiple clients charging each a premium, often setting that premium based at least partially on the previous experience of the primary payer. Performance-based annuities avoid paying a durable therapy stop-loss/reinsurance premium if an event does not occur, and then spread the cost of each event over multiple years, five years in this hypothetical case. It is in some sense experience rating conducted 'on demand'. From the primary payer perspective, actuarial risk is smoothed over multiple years without the costs of reinsurer administrative and profit margins. However, the pooling effect of spreading the event risk and resulting cost across the stop-loss/reinsurance issuer's larger population is eliminated and only partially mitigated by the payment spreading of each case.

A further advantage of the performance-based annuity compared to traditional reinsurance and stop-loss product is the opportunity for reduced payments if the therapy does not achieve the contracted outcome metric. If the therapy performs poorly compared to expectations, the payer savings could be substantial and more than off-set the partial mitigation of the actuarial risk. If the product performs so well that no performance-based reductions occur, the payment spreading does partially mitigate the actuarial risk, particularly since the payments are spread based on each patient event occurrence, not on an estimated population rate.

### **Conclusions and Limitations**

This performance-based annuity solution is most appropriate for products with long expected efficacy but significant uncertainty regarding the durability and consistency among patients of that efficacy performance. The solution also allows substantial spreading of the payments over time to better match costs with benefits, finance a potential surge of initial patients and mitigate the actuarial risk of rare events as partial stop-loss or reinsurance. Compared to the milestone-based contract it provides longer-lasting performance risk sharing between developer and payer. The performance-based annuity also addresses, at least partially, the payment timing and actuarial risks that the milestone-based contract solution does not.

The limitations of the performance-based annuity include added legal and regulatory issues, particularly regarding federal price benchmarking requirements, as well as added operational complexity particularly from patient mobility and long-term patient data tracking.

Self-insured employers may find performance-based annuities generally unappealing due to existing stop-loss insurance coverage options that effectively spread the upfront cost of rare but valuable therapies across many employers with modest premium increases. When durable/curative therapies become less rare the calculus balancing stop-loss premiums, stop-loss payouts and the opportunity for performance-based payment reductions may change.

Medicaid programs also face hurdles in implementing multi-year performance-based annuities ranging from restrictions on spreading costs from current medical events into future years to complexities of dividing responsibilities and payments between a State and its Managed Medicaid Organizations (MCOs).

To advance, performance-based annuities will require encouraging and clear policy regarding federal price benchmarking and anti-kickback statute safe harbor status. HIPAA policy to encourage patient data tracking would be helpful but perhaps not required. Facilitating efficient patient mobility mechanisms, including data tracking as well as common annuity structures, may benefit from third-party intermediaries playing a facilitating and organizing role. Third parties may also provide financial tools to address the accounting recognition challenges presented by performance-based annuities. Creating mechanisms for appropriate provider reimbursement—whether new reimbursement codes, adjusted DRGs, targeted buy-and-bill margins or some combination—will be critical to provide patients appropriate access without either overly encouraging or discouraging providers financially from offering these therapies.

Performance-based annuities are an important precision financing solution for durable/curative therapies, but they are not a solution for all product archetypes for all payer segments and provider situations. As Table 1.1 in the Executive Summary illustrates, the best match appears to be for Orphan Disrupters and Novel Breakthrough product archetypes for the commercial insurer payer segment. Further analysis may also suggest it is appropriate for Medicare/Managed Medicare for those product archetypes and perhaps for large surge, large population Quantum Leap therapies in the future.

### SMOOTHING THE RISK: ORBM AND RISK POOLING

Commercial health insurance, Medicaid and Medicare all pool healthcare risk and distribute it among their funding sources. For example, commercial health insurance pools employees of many employers and distributes the resulting healthcare costs (plus the implicit insurer administrative fees and profits) to employers and employees via premiums and to patients via co-pays, deductibles and co-insurance payments.

In the context of performance-based annuities, we discussed a secondary level of risk pooling performed by reinsurers and stop-loss providers.

FoCUS participants considered additional secondary risk pooling approaches to aid the financing of durable/curative therapies including state high risk pools and a new concept, the Orphan Reinsurer and Benefit Manager (ORBM).

### State high risk pools

Prior to the Affordable Care Act, some states established high risk pools to aid patients with high cost pre-existing conditions who were either priced out of insurance markets, refused coverage, denied employment due to insurance cost concerns, or some combination of these and other factors. The experience of these risk pools was generally poor due to inadequate funding for the costs of the patients included. FoCUS participants found this model a correspondingly poor fit for durable/curative therapies due to the emphasis of cost shifting to reduce insurance costs for the remaining pool, rather than an effective solution to provide funding and share unexpected risks for the affected patient populations.

### Bulk and pooled purchasing

FoCUS considered buying consortia such as hospital buying groups and state bulk purchasing such as proposed by the National Governors' Association.<sup>10</sup> FoCUS participants found these approaches emphasize lowering prices for therapies through increased purchasing power rather than addressing the FoCUS issues of how to create sustainable financing models for patient access after the therapy value has been negotiated.

### Orphan Reinsurer and Benefit Manager (ORBM)

Any individual orphan durable therapy may be managed within the existing healthcare financing system but collectively they may prove challenging particularly for smaller payers with limited ability to manage large upfront payments, therapeutic performance risk and actuarial risk. The FoCUS Project envisions Orphan Reinsurer and Benefit Managers as a precision financing solution to address the financial challenges created by durable therapies for non-oncology orphan conditions.

ORBMs would integrate three functions:

- **Managing actuarial risk** by carving-out and pooling patients across many payers
- **Contracting and reimbursement at scale**, especially for ultra-orphan therapies
- **Medical management and care coordination** for these specialized conditions and provider networks



Figure 3.5 illustrates the financial flows and contractual relationships an ORBM might construct to facilitate and manage the financial challenges of these therapies. As the name implies, the ORBM combines features of reinsurers, pharmacy benefit managers, and insurer medical and provider network management. In addition, they could provide financing to convert payment streams for individual participants from streams to lump sums or vice versa. The premium based relationship with the payer likely resolves the upfront accounting recognition issue for performance-based annuities.

ORBMs would likely customize their offered services to the needs of individual payers and therapies. Some payers may wish to use their own pre-authorization, utilization management, care coordination functions. Other payers may wish to use their local provider networks but access the specialized medical management expertise of the ORBM. When performance-based annuity or milestone-based contracts are in place, some developers may choose to factor the multiple, risky payment stream into a single, lower upfront payment with no future risk while other developers may choose to wait, retaining the therapy performance risk, anticipating higher eventual total revenue. Similar customization is already common in the PBM and even reinsurance marketplaces, but the range of services would be uniquely combined in the ORBM.

perhaps with the additional function of providing federal funding for durable/curative therapies was thought possible but unlikely. Should an ORBM market emerge, the firms will likely be diverse in their offerings and relative strengths, reflecting their origins and the desires of the other stakeholders. Recent announcements by Express Scripts demonstrate that at least one existing firm is beginning to offer aspects of the ORBM including facilitating direct payer purchasing under an outcomes agreement, outcomes data management, and patient care coordination systems.<sup>2, 11, 12</sup>

Additional information about the ORBM solution can be found in a [FoCUS Research Brief](#) and an [article in Pharmaceutical Executive](#).

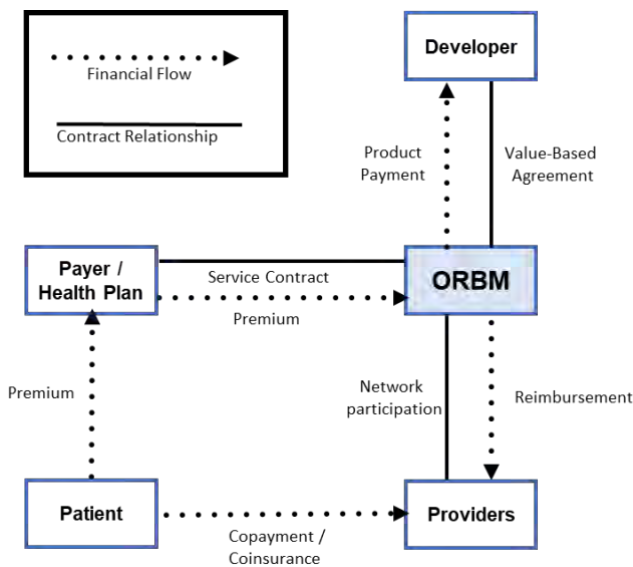
**Box 3.6: Express Scripts Actions Corroborate ORBM Solution**

On January 3, 2018 Spark Therapeutics announced a collaboration with Express Scripts affiliates, CuraScript Specialty Distribution and Accredo Specialty Pharmacy through which payers would purchase LUXTURNA™ rather than the treatment center under an outcomes rebate agreement. Spark Therapeutics assumes all drug in-transit, storage and handling risks even as Express Scripts executes the therapy distribution logistics, transaction tracking and billing.<sup>2</sup>

On May 29, 2018 Express Scripts further described the services that its Accredo affiliate offers for these therapies to include patient care systems including protocols and expertise offered through its Therapeutic Resource Center pharmacists, nurses and other clinicians to support patients, physicians and payers.<sup>11</sup>

On August 15, 2018 Express Scripts Holding Company confirmed it was in talks with BioMarin Pharmaceutical Inc. and bluebird bio Inc. to have its specialty pharmacy business exclusively distribute their new gene therapies when they become available in 2019 and 2020.<sup>12</sup>

These Express Scripts capabilities are similar to some of the key features of the ORBM described above.



**Figure 3.5:** Orphan Reinsurer and Benefit Manager (ORBM) interactions

FoCUS participants expected multiple ORBMs to emerge from existing insurers, reinsurers or pharmacy benefit managers individually or in collaboration. It was thought less likely for an ORBM to be formed *de novo* due to the broad skills required. Government action to create an ORBM,



## 4. Stakeholder Considerations

For simplicity the previous descriptions of the financing models emphasized the developer and the payer perspectives. In this section, we describe considerations raised by FoCUS participants for sub-groups of these stakeholders as well as the perspectives of patient, provider and reinsurer stakeholders.

### PAYERS

Payers face different challenges depending on their size, financial strength and existing regulations that govern their operations.

- **Smaller payers face larger impacts from actuarial risk:** The innate member scale of national insurers and traditional Federal Medicare reduces the impact of actuarial risk concerns from the orphan durable/curative therapies.
  - Individual high cost events could be a significant fraction of income, and for small self-insured employers perhaps catastrophically so. The smaller populations of regional insurers and MCOs for Medicare and Medicaid as well as smaller state Medicaid plans all face material financial income statement exposure from the variable occurrence of individual gene therapy cases in any given quarter or year even if such cases are a small portion of their balance sheet reserves and overall medical loss.
  - The temporal benefit mismatch of one-time therapy costs with the long-term benefits are exacerbated during the next few years when few patients are treated due to the small number of approved products. Payers or employers with small populations, high member turnover or both may be more concerned about their perceived over-absorption of the costs upon encountering a rare high cost case than payers with stable, long-term member beneficiaries. As annual treated patient numbers increase and coverage policies converge over time, this risk lessens.
  - Conditions with strong genetic inheritance exacerbate actuarial risk for their therapies. Some payers and providers may have regional familial patient clusters,

potentially increasing underwriting uncertainty and the absolute cost of an encounter.

- **Desire to mitigate payment timing** may differ among payer segments (commercial, self-insured, Medicaid, Medicare), their size and the epidemiology of the condition. For durable therapies for conditions with large patient backlogs, all payers may seek a financial solution that spreads the cost surge over time. For conditions with smaller populations, larger private health plans and Medicare with greater internal financial capacity may value this option less than smaller regional payers or state Medicaid programs.
- **Distinct legal/regulatory constraints:** Each payer segment has different legal and regulatory environments that affect their ability to adopt various precision financing tools. For example, solutions that address private sector financing challenges must consider how the tools used may also affect Medicaid best price and other federal pricing mechanisms. In contrast, performance contracts with State Medicaid programs may not trigger price reporting and the implications. Yet, unlike commercial plans, Medicaid plans may have single-year contracting rules and short-term balanced budget requirements, which necessitate workarounds to allow the use of contracts that amortize the costs of durable/curative therapies over longer periods of time.

These elements, along with differences in the four therapy archetypes (see Section 2 above) can result in different financial challenges for payer segments. Table 4.1<sup>v</sup> suggests how different combinations affect precision financing needs for the financial engineering solutions to address.

[FoCUS research](#) suggests that there is limited understanding and awareness today among payers of cell and gene therapies. Greater understanding by payers and other

Payer Type \ Cure Archetype	Self-Insured Employers	National Insurers	Medicare	Medicaid
Oncology	P, A	P	P	P, A
Novel Breakthrough	A, P, T	P	P	A, P, T
Orphan Disrupters	A, P, T	P	P	A, P, T
Quantum Leaps	A, P, T	P, T	P, T	A, P, T
A: Actuarial Risk    P: Performance Risk    T: Payment Timing				

**Table 4.1:** Cure Archetypes & Payer Types Impact Financial Engineering Needs and Solution Emphasis

**Oncology products:** Comparatively durable therapies (such as CAR-Ts) for oncology indications.

**Quantum leap:** Indications with large incident and prevalent populations such as cardiology, metabolic disorders, neurology and rheumatology.

<sup>v</sup> **Orphan disrupters:** Treatments for non-oncology orphan disease (<200,000 patients).

**Novel breakthroughs:** Therapies for conditions with <100 incident cases (ultra-orphans) with a high unmet need and preferably no alternative disease modifying treatments (e.g., Beta-thalassemia).

stakeholders of these treatments will be needed as we look towards broader use of precision financing tools.

## DEVELOPERS

In addition to therapy and condition characteristics, the varying financial capacity, administrative capabilities and business preferences of developers may influence which financing solutions they choose to offer to purchasers, if any.

Smaller firms may wish to convert complex performance-driven, multi-year annuity arrangements with payers into upfront single payments to satisfy financial growth targets or provide immediate cash to fund ongoing operations. These developers may not have sufficient internal infrastructure to adjudicate payer- or patient-specific clinical performance guarantees. Conversely, smaller developers may need outside assistance from financial institutions to guarantee future payer rebates. Larger firms may perceive these operational and cash flow considerations as internal issues, and might even directly or indirectly offer financing for other stakeholders. Mitigation of payment timing issues might be possible through financial services such as Royalty Pharma offers for biopharmaceutical royalty payments, which factor the expected payment stream to an upfront amount, but with some loss due to fees and discounting.<sup>13</sup>

Developers may also employ third parties for areas they do not deem critical internal competencies or where third parties may offer solutions to technical legal and regulatory challenges created by historical reimbursement approaches such as patient monitoring and data tracking.

## PATIENTS

The NEWDIGS FOCUS group discussions have identified a number of additional potential challenges patients may have in accessing durable/curative therapies. These issues include:

- **Lack of coverage or restrictive formularies:** Patient access to therapies depends on their insurance status, and when insured the coverage and formulary decisions of payers. If payers either exclude cell and/or gene therapies or institute restrictive formularies, patients will be limited in which therapies they can benefit from.
- **Accessible provider networks:** To control costs, some plans may restrict their provider networks, establishing “narrow networks.” Moreover, for durable therapies, there may be limited providers (centers of excellence) who are authorized by either a developer or a payer to deliver a particular therapy to ensure quality administration. Patients may need to seek out new providers or travel – perhaps even across state borders – in order to access treatment. This can have cost implications for patients, in addition to time lost from work, and may require payers

to establish specific processes for patients to access providers who are not typically “in network.” This is particularly relevant for Medicaid payers who may traditionally work only with in-State providers.

- **Patient financial burdens:** Patients affected by conditions targeted by durable therapies often already face high financial burdens. Patient direct healthcare out-of-pocket costs include co-pays, coinsurance payments, deductibles, and high annual cost sharing limits. Some patients may have separate medical and pharmacy deductibles. If a cell or gene therapy is covered under the medical benefit, Medicare patients will be subject to either an un-capped 20% out of pocket cost or the share of cost dictated by their Medicare Advantage plan or Medigap plan. Should the treatment be covered under the pharmacy benefit, Medicare Part D patients will face an un-capped 5% coinsurance payment after meeting their plans’ initial deductibles and coinsurance payments. Just as for payers, the concentrated upfront payments for years of subsequent benefit present barriers.

Additionally, patients have non-medical out-of-pocket costs including travel and possible loss of income due to treatment.

With respect to the financing models discussed earlier, a few elements deserve further consideration:

- **Co-pay and coinsurance limits:** Current levels of patient-payer cost sharing may deter access and actually increase costs over time for conditions with alternative, costly care. These patient costs primarily serve to encourage patients to make more appropriate healthcare choices when effective, lower cost options are available. For durable therapies this design objective is often moot given the alternatives. Plus, the cost sharing is a small portion of the total cost. FoCUS participants, including several payers, suggested that co-pays, deductibles and coinsurance be waived for these products provided it could be done without inducing adverse selection.
- **Sharing performance-related rebates and payments with patients may be merited.** Offsetting the benefits of transparency, fairness and incentive alignment, the costs of administering such a system and the likelihood that other medical costs would simply substitute to still reach the deductible cap may make this concept ineffective in reducing patient costs. This issue resolves in the patient’s favor if co-pays, deductibles and coinsurance are waived as described above.
- **Ongoing co-pays from performance-based annuities are not levied, as described above.** However, risks remain that computer systems and administrative errors in implementation could result in patient

financial stress. Future payers may also not share the FoCUS perspective on this point and design their patient benefits to require co-pays in each annuity period. FoCUS participants strongly recommend that safeguards for patients be established to prevent ongoing patient payments.

**Current manufacturer-administered assistance programs** are a partial solution that may also apply to these therapies. Manufacturers of chronic, rare disease medications (e.g., intravenous immunoglobulin (IVIG) or enzyme replacement therapies) often offer co-pay support programs for patients with commercial insurance as well as donations to 501(c)(3) organizations that offer financial support for patients with public insurance. In the gene therapy space, Spark Therapeutics has established an [access support program](#) and a fund that is available for IRD patients to support access for public insurance patients at [The Assistance Fund \(TAF\)](#). These types of programs are helpful, but likely inadequate. Further limiting the patient benefit of assistance programs, some Pharmacy Benefit Managers (PBMs) have instituted Accumulator Adjustment Programs (AAPs) that can have a negative financial impact on patients by no longer allowing co-pay assistance amounts to count towards a member's accumulator, which is the dollar amount applied to the patient's deductible or out-of-pocket costs. If AAPs are applied to curative therapies, then any positive effects of a manufacturer's assistance program would be negated.

**The financial services industry** already provides patients with second mortgages, consumer loans and credit card debt, among other services to aid in financing healthcare costs. Additional financial instruments, such as personal healthcare loans<sup>14</sup>, have been suggested. Continued innovation in financial services, particularly for patients with poor current credit scores but improved prospects after therapy, is needed.

**Providers** may also provide discounts and charitable write-offs, particularly non-profit providers with both mission and legal rationale to do so.<sup>15</sup> Such capacity however is limited.

- **Future costs from performance uncertainty** regarding therapy effectiveness, durability of effect, and adverse events, may occur. Appropriate patient education, facilitated by providers and patient organizations, to ensure that patients and their families understand these therapies will be important.

## PROVIDERS

Durable/curative therapies pose potential issues for providers such as new accreditation requirements for administering the therapies and financial risks from buying product (inventory risks), inadequate reimbursement for ancillary medical services, and service mix shifts.

- **Accreditation risk:** The cost and the complexity of administering some durable therapies is leading payers and also manufacturers to certify which providers may offer the products. Both Gilead/Kite and Novartis have limited access to their CAR-T therapies to company-certified treatment centers. Beyond core medical competence, some stakeholders suggest providers should meet additional quality procedure standards and/or reporting requirements. Insurers may want assurances that providers have sufficient expertise and that the laboratory and other sources needed by patients on these treatments are readily available. Therapy-specific accreditation programs may be an avenue to define standards and criteria for providers who wish to provide these more complex treatments and services.

More broadly, as noted earlier, we may see more movement towards centers of excellence. A center of excellence is “a program within a healthcare institution which is assembled to supply an exceptionally high concentration of expertise and related resources centered on a particular area of medicine, delivering associated care in a comprehensive, interdisciplinary fashion to afford the best patient outcomes possible.”<sup>9</sup> Establishing centers of excellence as part of a specialty network typically includes defining:

- Selection criteria
- Volume/outcome reporting requirements
- Quality improvement plans
- Designation/de-designation processes
- Clarity in how members can identify designated centers
- Care coordination
- Benefit language
- (Maybe) contracting strategies

Ideally, COE networks will help to ensure a consistent quality of patient care and encourage better clinical outcomes, while creating incentives for cost-effective care with disincentives for waste. Challenges will include implementation of consistent but minimally burdensome reporting and quality standards, and broad access to patients regardless of geography, clinical need and socioeconomic background. See the [FoCUS Research Brief](#) for additional discussion of this topic.

- **Provider financial risks:** Historically provider financial risks have been related to overall reimbursement levels and payment risks under a buy-and-bill model. Cell and gene therapies may also change service patterns, which will raise additional financial considerations.
  - **Inadequate DRG rates:** The milestone-based performance model section raised this issue in the context of existing inpatient DRGs not including the

**Box 4.1: Financial Sustainability—Hemophilia Treatment Centers**

In 1973, the National Hemophilia Foundation (NHF) launched a campaign to establish a nationwide network of hemophilia diagnostic and treatment centers (HTCs). The goal was to provide a range of comprehensive services for patients and families within one treatment facility. The federal government recognized the work of the HTCs and established a grant program to provide funding for important services typically not covered by insurance. The 146 such funded Hemophilia Treatment Centers (HTCs)<sup>16</sup>, manage approximately 90% of hemophilia patients.<sup>17</sup>

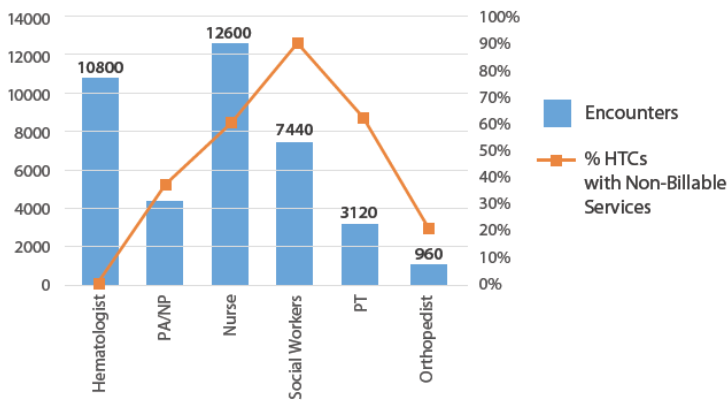
Approximately 85% of HTCs provide clotting factor and injection supplies to patients as necessary. Most HTCs are located in or adjacent to academic medical centers and children’s hospitals, and participate in the 340B Drug Pricing Program under their federal designation as a comprehensive hemophilia treatment center. The 340B Drug Pricing Program is a US federal government program created in 1992 that requires drug manufacturers to provide outpatient drugs to eligible health care organizations and covered entities at significantly reduced prices. Providers then bill payers at higher prices creating a financial margin.

Income from providing clotting factor and injection supplies to patients represents a significant percentage and, in many cases, the majority of an HTC’s income. This income is utilized to support the multidisciplinary integrated care provided to the hemophilia population.

In 2014, a survey was conducted by the National Hemophilia Program Coordinating Center (NHPCC) of HTCs that have 340B pharmacy programs.<sup>18</sup> The survey showed that most HTCs with 340B programs fund >50% of the salary costs of non-physicians with 340B program income. 90% of HTCs utilize 340B program income to support social workers. Home and school visits by the HTC team are almost entirely supported by 340B program income.

In addition (see table below), the majority of centers also utilize 340B program income to provide over 90% of the funding for phone triage, care coordination, case management, and patient education. Most of these services are not typically reimbursed by insurance making the 340B program income vital to the HTCs.

**Outpatient and Follow-Up Visits**



Hemophilia Treatment Center Services	# of Encounters	% of HTC's Reporting ≥ 90% 340B Funded
<b>Telephone Triage Urgent/Emergent</b>		
Annual Mean Encounters per HTC	1,968	76%
Annual Total Encounters 29 HTC	57,072	76%
<b>Medical Care Coordination</b>		
Annual Mean Encounters per HTC	2,088	80%
Annual Total Encounters 30 HTC	62,640	80%
<b>Care Management/Psychosocial/Vocational</b>		
Annual Mean Encounters per HTC	960	70%
Annual Total Encounters 30 HTC	28,800	70%
<b>Patient Education</b>		
Annual Mean Encounters per HTC	516	75%
Annual Total Encounters 30 HTC	15,480	75%

costs of durable/curative therapies. This may be partially addressed by Medicare New Technology Add-on Payments, but these often: significantly lag the therapy launch, are not approved, and only provide partial coverage.

- **Unavailable or inadequate outpatient reimbursement:** A therapy administered in an outpatient setting may need to receive a distinct and separate code, e.g. a “J code,” to enable reimbursement filing. Code issuance is often delayed, with commensurate patient access delays and

significant financial risk to providers during the interim. Once issued, adequate federal and private reimbursement levels are not assured.

- **Product inventory risk:** Under a buy-and-bill model, providers acquire the product, administer it and then receive payer reimbursement later with a mark-up. During that time the provider bears stocking and inventory carrying costs, as well as risk of wastage, contamination and expiration. These costs and risks increase with the therapy’s cost and any payment timing delays.

- Potential financial risk from shifting service mix:** Gene therapy may replace existing treatments. If the administration of gene therapy is performed by a different provider or if the provider was dependent on income from administering existing treatments, the advent of gene therapies may require re-evaluation of the services a provider offers, alternative approaches to achieve greater efficiency in service delivery, and/or re-negotiation of payer reimbursement levels for remaining services. This type of financial risk is particularly a concern where providers may be informally cross-subsidizing one service with another's income. An example may be found in Box 4.1 below, which provides a concrete example of how gene therapy might affect Hemophilia Treatment Center income and services.
- Buy-and-bill elimination:** As described in Box 3.2, an early gene therapy (LUXTURNA™) is being distributed in a manner that eliminates buy-and-bill provider mark-up. Provider reimbursement disruption may occur for those therapies that either directly would have been treated as 340B or buy-and-bill products or that indirectly displace standard-of-care therapies under such arrangements.

described above avoids this scale issue by carving-out the existing therapies and patients.

Financial solutions for durable/curative therapies that include actuarial risk management thus pose both disruptive challenges and opportunities for reinsurers and stop-loss providers. For further discussion of reinsurance and stop-loss see the [July 2018 FoCUS Research Brief](#).

## REINSURERS AND STOP-LOSS CARRIERS

Robust actuarial risk management solutions will be important to support successful uptake of durable/curative therapies. Reinsurance purchased by an HMO or insurance company allows them to pass all or part of their risk to another insurance company. Self-funded employers purchase stop-loss insurance to protect against large claims on any one person (Specific Stop Loss) or higher than expected claims overall (Aggregate Stop Loss).

In a one-year period, such as posited in the milestone-based contract model, FoCUS participants expect current tools can work effectively to mitigate actuarial risk, provided that reinsurance companies do not exclude such therapies or the patients that could benefit from their offerings.

In contrast, multi-year performance-based annuities could disrupt reinsurance and stop-loss markets, which are currently concentrated in one-year contracts with perhaps a one-year extension option. The FoCUS working group has identified two potential solutions: extension of annual secondary insurance contracts to include specified downstream costs beyond the contract year, or carving out of durable therapies into separate coverage. As with all such reinsurance and stop-loss policies, exact descriptions of covered therapies and costs would be required. In the near-term, insufficient volume of such therapies may prevent the profitable offering of either solution. The ORBM solution



## 5. Summary, Collaborative Change and Future Directions

Table 5.1 reprises the precision financing solution generally preferred by FoCUS participants for each payer segment and product archetype combination to address the three financial challenges of durable/curative therapies:

- 1. Payment timing:** Therapies can involve substantial upfront payment for multiple years of therapeutic benefit.
- 2. Therapeutic performance risk:** Real world efficacy and durability are uncertain at the time of initial regulatory approval and market launch.
- 3. Actuarial risk:** The number of eligible patients in a payer’s population may be uncertain and could vary significantly from period to period.

For Orphan Disrupter gene therapy archetypes<sup>vi</sup>, FoCUS suggests an ORBM approach for self-insured employers and performance-based annuity solutions for insurers/MCOs. The better Medicaid match for these products changes depending on the existence of cost offsets. When cost offsets

exist the milestone-based contract solution appears superior. When no cost offsets exist a risk pooling solution is suggested. All precision financing solutions might apply to Novel Breakthroughs depending on the product technology and natural history of the condition as well as on payer size and capabilities. For oncology products, the milestone-based contract solution is currently best across all payer segments due to the shorter durability of these therapies such as CAR-T and the incidence driven population characteristics of oncology that limits the backlog surge effect. Existing stop-loss insurance for self-insured employers will continue as the near-term financial risk management tool across the first three product archetypes. Work is continuing on designing Medicare and Quantum Leaps preferred solutions.

### AREAS FOR ENABLING CHANGES

Implementing these solutions requires multiple relationship changes among stakeholders as well as some targeted policy refinements and perhaps new roles for intermediaries.

The financial solutions are not merely two-party performance-based agreements between developer and payer. Rather the solutions address needs among many

	Self-Insured Employers	Insurers/MCO	Medicaid	Medicare
Orphan Disrupters with cost offset (Hemophilia)	ORBM	Performance Based Annuities	Milestone Based Contract	
Orphan Disrupters without cost offset	ORBM	Performance Based Annuities	Risk Pooling	
Novel Breakthroughs		Performance Based Annuities	ORBM	Milestone Based Contract
Oncology			Milestone Based Contract	
Quantum Leaps			To be Determined	

**Table 5.1:** Primary Precision Financing Solution by Payer Segment and Product Archetype

<sup>vi</sup> **Orphan disrupters:** Treatments for non-oncology orphan disease (<200,000 patients).

**Novel breakthroughs:** Therapies for conditions with <100 incident cases (ultra-orphans) with a high unmet need and preferably no alternative disease modifying treatments (e.g., Beta-thalassemia).

**Oncology products:** Comparatively durable therapies (such as CAR-Ts) for oncology indications.

**Quantum leap:** Indications with large incident and prevalent populations such as cardiology, metabolic disorders, neurology and rheumatology.



Enabling Change	Milestone-based Contract Solution	Performance-based Annuity Solution	ORBM Solution
<b>Regulatory</b>			
Revised Price Benchmarking	✓✓✓	✓✓✓	✓✓✓
Anti-Kickback Statute safe harbor inclusion	✓✓✓	✓✓✓	✓✓✓
FDA Manufacturer Communication Guidelines for early discussion & using outcome metrics not in label	✓	✓	✓
HIPAA revisions to ease patient outcomes collection & sharing	✓✓	✓✓	✓✓
<b>Operational</b>			
Outcomes data collection	✓	✓✓	✓✓✓
Provider reimbursement mechanisms	✓✓	✓✓	✓✓
<b>Risk Management</b>			
Center of Excellence	✓✓	✓✓	✓✓
Patient mobility mechanisms	✓	✓✓✓	✓✓
Reinsurance/Stop-Loss evolution	✓	✓✓	✓✓✓

✓ (low), ✓✓, ✓✓✓ (high) indicates relative level of importance and opportunity

**Table 5.2:** Areas for Collaborative Action for Enabling Change

primary stakeholders: patients, providers, payers and developers. Roles of second-line payers such as reinsurers and stop-loss providers, delegated payers such as managed Medicaid and Medicare Advantage plans, contracting intermediaries such as Third-Party Administrators on behalf of self-insured employers, specialty pharmacies, and pharmaceutical benefit managers will also be affected. Most of these will evolve as specific products launch and the scale from a portfolio of such therapies grows.

Collaborative stakeholder action could enable more rapid development of these and similar financial solutions. The table above briefly summarizes the areas that would benefit from collaborative action to develop new capabilities, mechanisms and policies.

**FUTURE DIRECTIONS**

[The FoCUS webpage](#) contains additional discussions and resources. Some are already available including the FoCUS White Paper on “Designing financial solutions to ensure affordable access to cures” and twelve FoCUS Research Briefs on topics such as payer segmentation, actuarial risk, patient mobility, the ORBM, and reinsurance.

Additional topical Research Briefs and White Papers will be released over time. In addition, FoCUS intends to release resources and tools to aid organizations as they consider applying these financial solutions in collaboration with other stakeholders. These will include issue maps, templates for examining the issues and perhaps some quantitative tools. Resources describing the policy issues and possible approaches to aid in the discussion and revision of rules and regulations to facilitate these solutions are also planned.

Two pilots are being designed for launch in 2019 or 2020. The first is a performance-based annuity solution pilot in Massachusetts fully-insured commercial plans for one or more orphan indications, viral vector delivered gene therapies, likely delivered in an outpatient setting. The second is a Medicaid population pilot employing a milestone-based contract solution for an inpatient adoptive cellular therapy such as CAR-T for oncology.

**CONCLUSION**

Durable, potentially curative, therapies promise transformative benefits for patients while simultaneously promising unique and substantial financial challenges for

nearly all healthcare stakeholders: patients, providers, payers, policy makers and developers among others. Financial innovation in addition to scientific, clinical and regulatory innovation is needed to provide appropriate patient access.

FoCUS participants found that a single innovative solution cannot address all stakeholder financial needs in all situations.

Precision financing is needed which customizes solutions for the clinical context, therapeutic characteristics, and payer segments while simultaneously deploying financial tools that address adequate provider reimbursement for all their needed activities and which reduce, if not eliminate, financial toxicity for patients.

Three precision financing solutions for durable, potentially curative, therapies described here have been designed by the FoCUS group over two years of multi-stakeholder, collaborative effort. The FoCUS group hopes these templates inspire further financial innovation and real-world pilots to facilitate patient access to these important therapies in a sustainable manner for all healthcare stakeholders.

## 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.

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