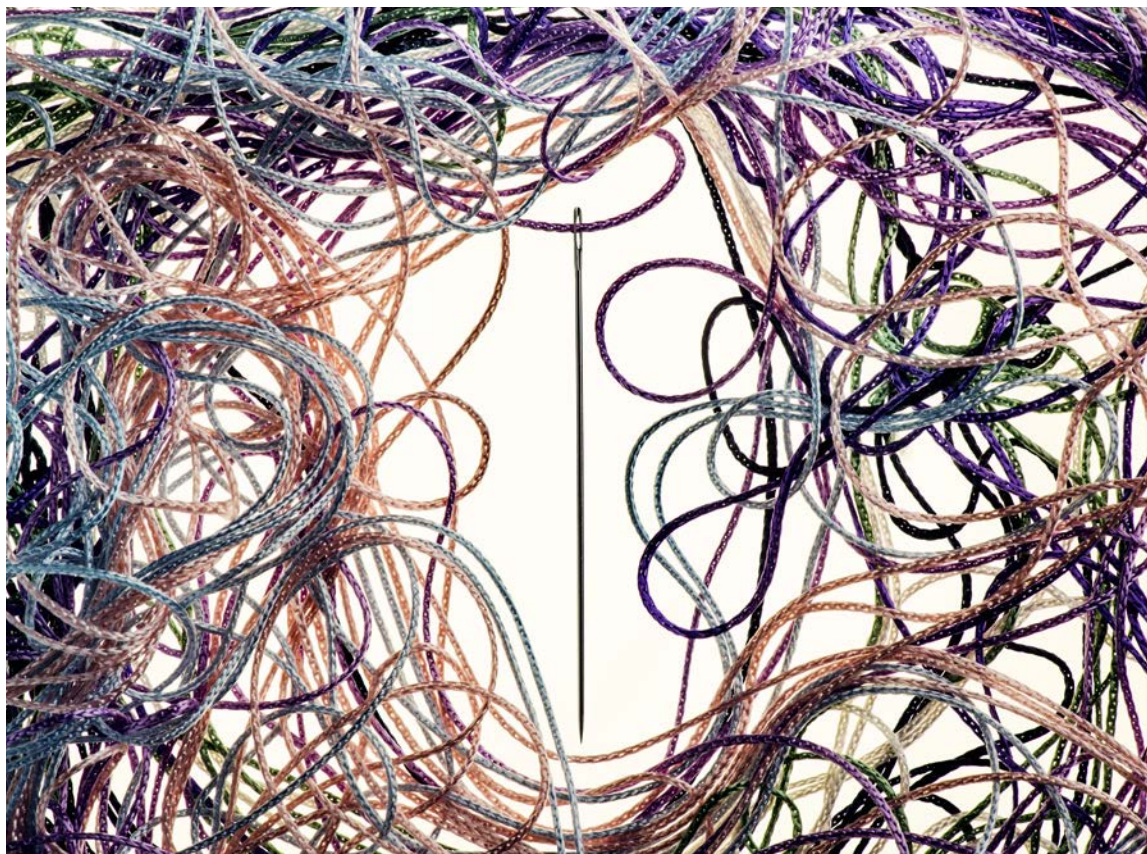


NEW DIGS

FoCUS

Financing and Reimbursement
of Cures in the US

WHITE PAPER



Warranty Model: A potential precision
financing solution for durable cell
and gene therapies

October 30, 2020

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EXECUTIVE SUMMARY

Durable cell and gene therapies are poised for rapid growth - potentially providing transformative benefits for patients and exacerbating financial challenges for payers, providers, patients, and therapeutic developers. With five durable cell and gene therapies currently approved by the FDA, the FoCUS Pipeline Analysis Model expects 50-75 approvals over the next decade¹. These durable therapies are administered in a 'one-time' treatment course yielding multi-year, possibly lifetime, benefits for patients and ensuring improvements for their families, communities and healthcare systems.

The one-time administration, large benefits and multi-year durability combine to create high patient value, which is condensed into a correspondingly high, one-time upfront payment. This can create a shock to the healthcare system which is accustomed to medicines requiring daily, monthly, or at most quarterly dosing. Durable cell and gene therapies disrupt the classic 'pay-as-you-go' drug payment paradigms.

Eighty percent of surveyed payers reported in 2019 that they are "highly" or "extremely" concerned about the existing and future costs of durable therapies². In 2017, the Massachusetts Institute of Technology, New Drug Development Paradigms Initiative (NEWDIGS) launched the Financing and Reimbursement of Cures in the US (FoCUS) Project, a multi-stakeholder consortium assembled to improve patient access to these therapies by addressing the financing challenges they pose. In October 2019, the FoCUS consortium published a white paper summarizing the results of the then most recent payer survey. Key takeaways included:

- Payers have a heightened concern regarding the financial risk and sustainability of high cost one-time durable treatments
- BOTH the high upfront cost of individual treatments and cumulative impact of multiple treatments are a concern
- Payers are interested in implementing new financing approaches—especially those that align payment with the actual patient benefit received
- Multiple barriers must be resolved—especially regulatory compliance and data collection³

FoCUS Precision Financing Solutions address one or more of the following financial challenges presented by durable cell and gene therapies:

- **Payment Timing:** mismatch between the initial first year cost and the multi-year stream of benefits
- **Performance Risk:** uncertainty regarding the degree of response and its durability for an individual patient or population of covered patients
- **Actuarial Risk:** likelihood of a payer encountering a case that is treatable with an available therapy in a given period

Though solutions such as milestone-based contracts and performance-based annuities address the core challenges, there are still implementation related hurdles. Two significant complexities are data aggregation/utilization and Medicaid Best Price (MBP) regulations. In April 2020, FoCUS investigated a new warranty solution that provides risk-sharing flexibility between payers and developers by addressing the performance risk challenge, reducing data aggregation complexity, and mitigates MBP exposure.

Benefits and how it works

The warranty repays healthcare claims resulting from inadequate therapy performance while adhering to Medicaid Best Price reporting requirements. It also reduces the administrative burden of complex data aggregation. The warranty limits Medicaid Best Price implications by containing reporting requirements to the amount of premium paid for the warranty coverage while also reducing data aggregation burden on all stakeholders. Warranty premiums are paid by the developer, not the payer, and are subject to Medicaid Best Price reporting requirements. Key benefits of the warranty include:

- MBP reporting compliant mechanism
- Standard claims process supports administrative simplicity leveraging existing claims channels
- Claims requirements and processes clearly defined at the time of warranty issuance
- Reduces burden on patients, providers, payers, and developers to track extraneous data outside of their current systems or protocols
- Insurance oversight guarantees solvency for claims payments via periodic audits and claims experience reviews

The durable therapy warranty allows for an agreement between the developers and payers to "warrant" the coverage of future ongoing healthcare costs if the therapy does not meet a specified efficacy standard. As an example, in the case of Hemophilia A, the developer may insure payers against the consequences if the durable effect diminished over time, resulting in the need for supplemental Factor VIII. In essence, the warranty would reimburse the payer for the costs of the supplemental Factor VIII if needed by a gene therapy-treated patient. The warranty would not provide any refund on the price of the gene therapy product itself. To further clarify, the therapy warranty concept is like that of a car manufacturer warranty. The car manufacturer 'promises' that the core components of the purchased vehicle will operate without failure for a period of time. In the event that one of those core components fails, such as the transmission, the manufacturer pays for the replacement transmission and perhaps other costs

¹ MIT NEWDIGS Research Brief 2020F207-v051 Pipeline Analysis available at <https://newdigs.mit.edu/sites/default/files/NEWDIGS-Research-Brief-2020F207v51-PipelineAnalysis.pdf>

² Barlow, J., Courtney, M., & Trusheim, M. (2020, April). "Payer Perspectives on Gene Therapy Reimbursement" Pharmaceutical Executive, pp 24-29. Available at: <https://www.pharmexec.com/view/payer-perspectives-gene-therapy-reimbursement>.

³ <https://newdigs.mit.edu/sites/default/files/MIT%20FoCUS%20Payer%20Perspectives%202019F210v044.pdf>

such as a rental or loaner car, as opposed to providing the car purchaser with a refund tied to the car purchase price.

Each warranty program is unique to a therapy and can include a combination of special coverage considerations and/or riders to provide coverage for particular circumstances, such as life insurance, non-responders, portability, or quality of life metrics. The policy is issued to the payer for each patient and shelters the policy holder against future expenses incurred in the event that the covered therapy does not meet the developer's efficacy promise. The warranty model adheres to Medicaid Best Price regulations because the warranty payments from any single patient's claims represent covered damages against the warranty policy purchased by the developer as opposed to a rebate associated with the price of the therapy.

In the proposed operational warranty model, a third-party, or Outcomes-Based Contract Administrator (OBCA), administers the warranty for a developer (Figure 1). When the developer initiates a warranty program, a segregated financial 'cell' is established within the OBCA's regulated warranty insurer entity. This cell is developer-specific and holds the financial risk associated with the terms of the warranty and any associated claims payouts. Each developer is responsible for securing the required risk capital held by the cell. They also fund the expected warranty payments via a premium to the cell for each policy issued (per treated patient).

Under this model, a health plan would approve and pay for a patient's therapy as usual, likely as an upfront one-time payment. Should the warranty be triggered, the payer would submit a claim to the OBCA documenting the incurred and covered costs. The OBCA would then adjudicate the claim, requesting additional documentation as needed and reimburse the payer from the funds in that product's warranty 'cell'.

Challenges

Though the warranty addresses key issues such as administrative burden, complex data exchange, and Medicaid Best Price implications, other challenges remain. For example, the warranty model relies on clear, easily identifiable and measurable metrics for therapeutic failure in order to reduce warranty claims adjudication subjectivity. The warranty also requires clear definitions of covered claims for reimbursement. However, the warranty does provide the flexibility to use efficacy monitoring

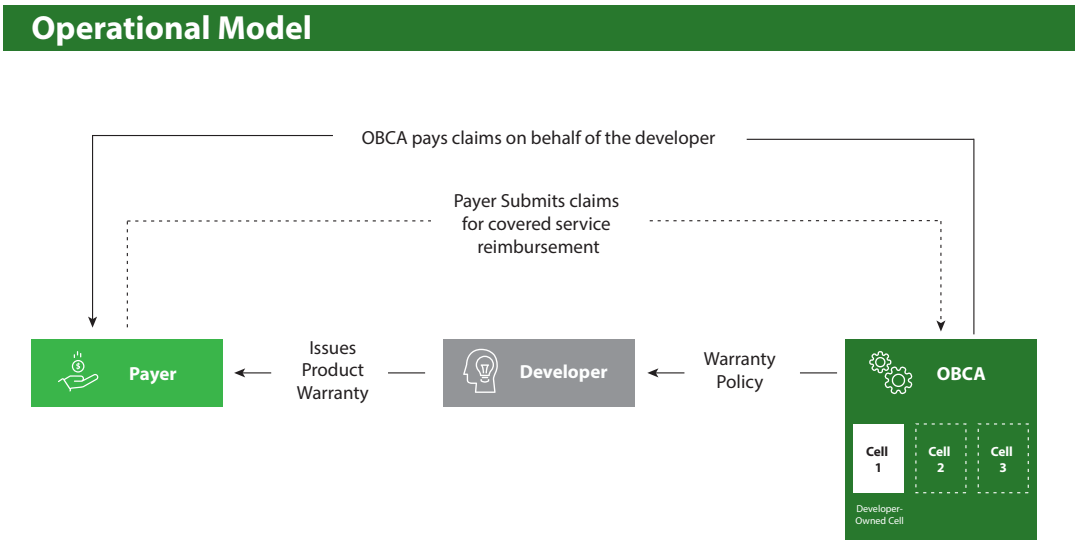


Figure 1. Proposed operational model for a warranty

mechanisms beyond those endpoints in the drug label as part of the pre-defined criteria. Additionally, the warranty model does not solve the patient mobility issues that are present with all multi-year solutions.

Possible extensions

The core of each warranty policy defines the therapy-specific developer promise, coverage terms, and triggers that can collectively result in claims payments. In some cases, it may be necessary to address additional outcomes-based scenarios that occur outside of the standard coverage. To create additional flexibility and extensibility of the warranty construct, policies may also include riders or addendums to cover specific scenarios. For example riders could cover scenarios such as:

- **Non-Responder Window**—When the parties wish to delay the warranty coverage period or otherwise define a distinct period. Examples might include, delaying a CAR-T therapy warranty until after the initial cell infusion has proven successful, or delaying an AAV-based therapy warranty period for six months to allow the transfected cells to reach stable clinical result. This rider also facilitates combining the warranty with other Precision Financing Solutions such as a short-term milestone-based contract.
- **Life Insurance**—With some therapies the result of therapy inefficacy may be mortality, perhaps with low resultant payer expenses. This rider allows the developer to warranty the potentially lifesaving therapies up to a pre-defined amount in the event of mortality, even if resultant claims do not reach that amount.

Even with these possible extensions, not all products may be suitable for a warranty approach due to lack of offsetting costs, the complexity of adding riders, the difficulties of data collection, the high performance of the therapy, or some combination of all these

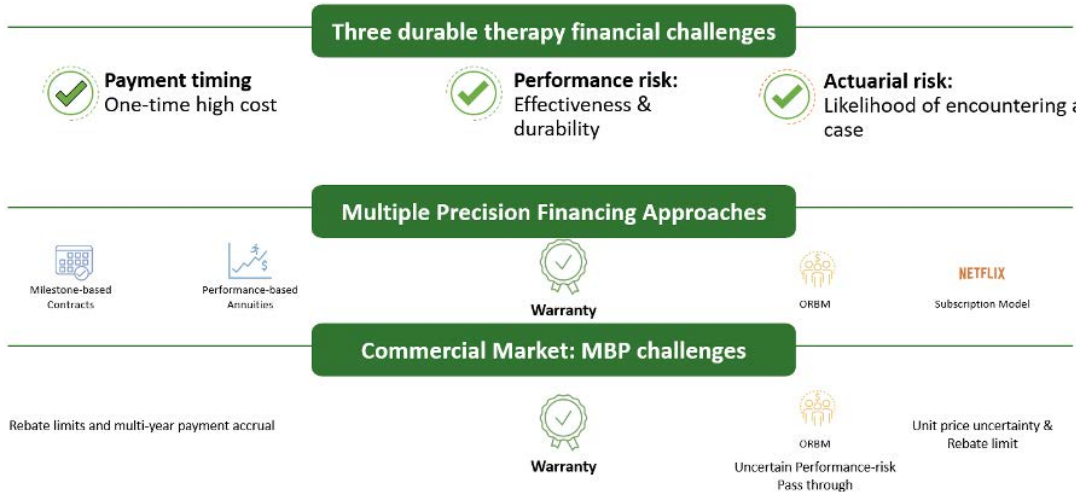


Figure 2. Warranty model strategic fit

considerations in addition to the preferences of the potential parties.

Combining with other Precision Financing Solutions

Prior Precision Financing Solutions that address performance risk have suffered from limited time coverage or regulatory impediments such as Medicaid Best Price reporting. At the April 2020 Design Lab FoCUS participants examined a new warranty model designed to address cell and gene therapy performance risk in a manner that complies with current Medicaid Best Price regulations. Moreover, the warranty model complements other precision financing strategies that address payment timing and actuarial risk challenges. Figure 2 below depicts how the warranty model performance risk management might complement the emphases of other Precision Financing Solutions.

Conclusion

The warranty model provides a flexible solution for developers to address payer concerns regarding the efficacy and durability uncertainties of cell and gene therapies. Moreover, the model facilitates risk sharing between payers and developers in a manner generally accepted as compliant with Medicaid Best Price regulations. Despite the solution extensibility, challenges remain, and the warranty model may not work for every therapy, especially if clear performance metrics are not easily available. The warranty model provides a standard framework for designing warranties and a third-party administrative approach for efficiently implementing and administering warranties at scale.

INTRODUCTION

Why a warranty?

Cell and gene therapies represent innovation and the next frontier in the treatment of rare diseases, however they also pose an unparalleled risk to the healthcare ecosystem. These therapies, while potentially durable, often have small clinical trial populations resulting in efficacy uncertainty. Furthermore, therapies are commercialized with extreme price tags, which creates an imbalance in the standard healthcare payment paradigm. Combining performance risk with high costs results in coverage hesitation by payers which then leads to limited access to these life altering/saving therapies. The following white paper provides an in-depth analysis of an evolving risk sharing model referred to as a “warranty”. This new model focuses on driving patient access to innovative cell and gene therapies while providing a mechanism to facilitate efficacy risk sharing between therapy developers and payers. The analysis will focus on the durable therapy market overall, how the warranty works operationally, model challenges, extensibility, and applicability to the Cell and Gene Therapy market.

Durable therapies

To date, four durable cell and gene therapies have received FDA approval:

- Luxturna (Voretigene neparvovec: Spark Therapeutics) for Leber’s congenital amaurosis;
- Zolgensma (Onasemnogene abeparvovec: Avexis/Novartis) for spinal muscular atrophy;
- CAR-T cell therapies for leukemias
 1. Kymriah (Tisagenlecleucel: Novartis) for B-cell acute lymphoblastic leukemia (ALL) in those aged 25 and younger and for large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL) in adults
 2. Yescarta (axicabtagene ciloleucel: Kite Therapeutics/Gilead) for large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL) in adults

At least thirty to sixty durable therapies are expected to gain approval by 2030 benefiting an estimated 350,000 people with the most recent estimates suggesting that up to 80-100 therapies may be approved.^{4,5} These treatments are administered as a “one-time”, single course of treatment offering durable (at least 18 months), potentially curative benefits for patients. Depending on the indication, they may also make costs of disease progression and alternative therapies avoidable.

Durable therapy financing challenges

Cell and gene therapies reimbursements have reflected these multi-year benefits resulting in three financial challenges:

1. **Payment timing:** mismatch between the initial first year cost and the multi-year stream of benefits and avoided other costs
2. **Performance risk:** from the uncertainty regarding the degree

of response and its durability for an individual patient or population of covered patients

3. **Actuarial risk:** regarding the number of treated patients that will occur in a payer’s population in a particular period

Payers are recognizing these challenges. In October 2019, the FoCUS consortium published a white paper summarizing the results of a 77-payer survey representing 153 fully insured commercial, Medicaid, Medicare Advantage, and self-insured employer plans. The key takeaways from the survey included:

- Payers have a heightened concern regarding the financial risk and sustainability of high cost one-time durable treatments
- Payers are most closely aligned with the benefit of paying for what works
- Multiple barriers must be resolved: Regulatory issues must be addressed at the structural systems level⁶

Furthermore, the payers are asking for some level of assurance that the therapies will indeed result in the expected benefits touted by developers including clinical efficacy and savings on other aspects of care. Payers want developers to assure that their products provide the expected benefits through outcomes-based contracts using techniques such as milestone-based rebates or performance-based payments over time.

Payers, including CMS, have entered into value-based (or outcomes-based) agreements with developers. These agreements currently evaluate the clinical effectiveness and/or responsiveness of a particular product or therapy. If the clinical result is not met by a certain time, then usually a partial refund on the cost of the product is returned to the payer via a rebate. The warranty model, described in detail below, maintains the spirit of the traditional value-based agreement approach while also addressing the challenges facing Precision Financing Solutions, specifically reimbursement/payment timing restrictions created by Medicaid Best Price regulations. Moreover, the warranty model focuses on payer-developer risk sharing as a method to address concerns around efficacy and durability as opposed to a solution to address the typically high one-time therapy cost.

Challenges facing precision financing solutions

Since the introduction of the first CAR-T therapies and subsequent gene therapies the healthcare industry, and FoCUS, have dedicated significant resources to solving the issues surrounding durable therapy efficacy and cost. This has resulted in multiple precision financing models such as:

⁴Quinn, Casey, et al. “Estimating the clinical pipeline of cell and gene therapies and their potential economic impact on the US healthcare system.” *Value in Health* 22.6 (2019): 621-626. Available at [https://www.valueinhealthjournal.com/article/S1098-3015\(19\)30188-3/fulltext](https://www.valueinhealthjournal.com/article/S1098-3015(19)30188-3/fulltext)

⁵FoCUS Paying for Cures Toolkit. Expected availability to 2031. <https://www.payingforcures.org/toolkit-overview/pipeline/>. Accessed April 2020.

⁶<https://newdigs.mit.edu/sites/default/files/MIT%20FoCUS%20Payer%20Perspectives%202019F210v044.pdf>

- Milestone-based contracts: upfront payment for therapy and refunds tied to performance
- Performance-based annuities: payments spread over time; payments tied to performance
- Orphan Reimbursement Benefit Management: focused on holistic therapy management not just financing
- Subscription models: pay for access regardless of utilization, similar to the models used for Hepatitis C cures

Each of these models has unique independent challenges independently but also share a number of common issues as shown in Table 1 below.

Table 1. Challenges faced by all Precision Financing Models addressing performance risk

Challenge	Description	Model Impacted
Performance Data Tracking	<ul style="list-style-type: none"> • Multiple data sources • No central aggregator • Administratively complex • (payer and manufacturer) 	<ul style="list-style-type: none"> • All
Patient Mobility	<ul style="list-style-type: none"> • High cost remains the burden of the original payer • Patient churn impacts therapy investment decision • Currently no recourse for payer making investment 	<ul style="list-style-type: none"> • Milestone Contracts • Performance-based Annuities
Regulatory	<ul style="list-style-type: none"> • Medicaid Best Price • AKS & Stark • HIPAA 	<ul style="list-style-type: none"> • All
Administrative Burden and Cost	<ul style="list-style-type: none"> • Payer and Developer Contract Management • Small patient volumes, customized processes per therapy • No standard administrative process for traditional VBAs or precision financing models 	<ul style="list-style-type: none"> • All

AKS and Stark Law

The Anti-Kickback Statute (AKS) and Stark Law do not apply as restrictions to the proposed warranty model. Specific exceptions to AKS regarding warranties (see CFR 1001.952 (g)⁷) serve as precedent for the use of a manufacturer therapeutic warranty. In context, AKS refers more specifically to the use of warranties as a mode of remuneration to providers for services rendered to beneficiaries. Additionally, the exceptions state that “The manufacturer or supplier must not pay any remuneration to any individual (other than a beneficiary) or entity for any medical, surgical, or hospital expense incurred by a beneficiary other than for the cost of the item itself”. When applied to the proposed warranty model and to the payment of damages in the form of exact cost reimbursement for medical, surgical, or hospital expenses, the AKS exception serves as logical precedent for the use of a warranty for risk sharing between cell and gene therapy developers and payers (both commercial and government).

Medicaid Best Price issue

By using a liability transfer mechanism paid for by therapy developers, the warranty allows for payers to benefit from reimbursement on warranty claims in excess of 23.1%. This differs from other models in that the developer pays a premium, which is subject to MBP reporting, for a warranty policy that covers subsequent damages that may exceed the 23.1% threshold for adult patients. Developers willing to provide assurances in the form of value-based (or outcomes-based) contracts have heretofore limited the amount of risk they will share by the perceived implications of complying with Medicaid Drug Rebate Program’s “best price” reporting methodology. These rebates rarely exceed the Medicaid Best Price threshold—23.1% of Average Manufacturer Price (AMP) for adult indications of brand name drugs. This consideration usually limits the amount of “risk” that developers agree to share with payers because exceeding the 23.1% threshold would result in an overall reduction in therapy AMP if the therapy fails to perform on an individual patient basis. Consequently, the “value” proposition of the contracts is relegated to the traditional rebate model. Specific issues include:

- **A deep discount for a single patient receiving little benefit** from a rarer condition therapy in a commercial, Medicaid MCO or Medicare Advantage plan may require ALL Medicaid units of that therapy receive a rebate based on that deep discount, regardless of the Medicaid patient recipients’ actual responses.
- **The first performance-based installment payment** could be considered the entire price for the therapy. This again could result in triggering a rebate for all Medicaid units of the therapy. For example, a first performance installment representing 20% of the total payments could trigger an 80% rebate for all Medicaid sales.

⁷ <https://www.law.cornell.edu/cfr/text/42/1001.952>

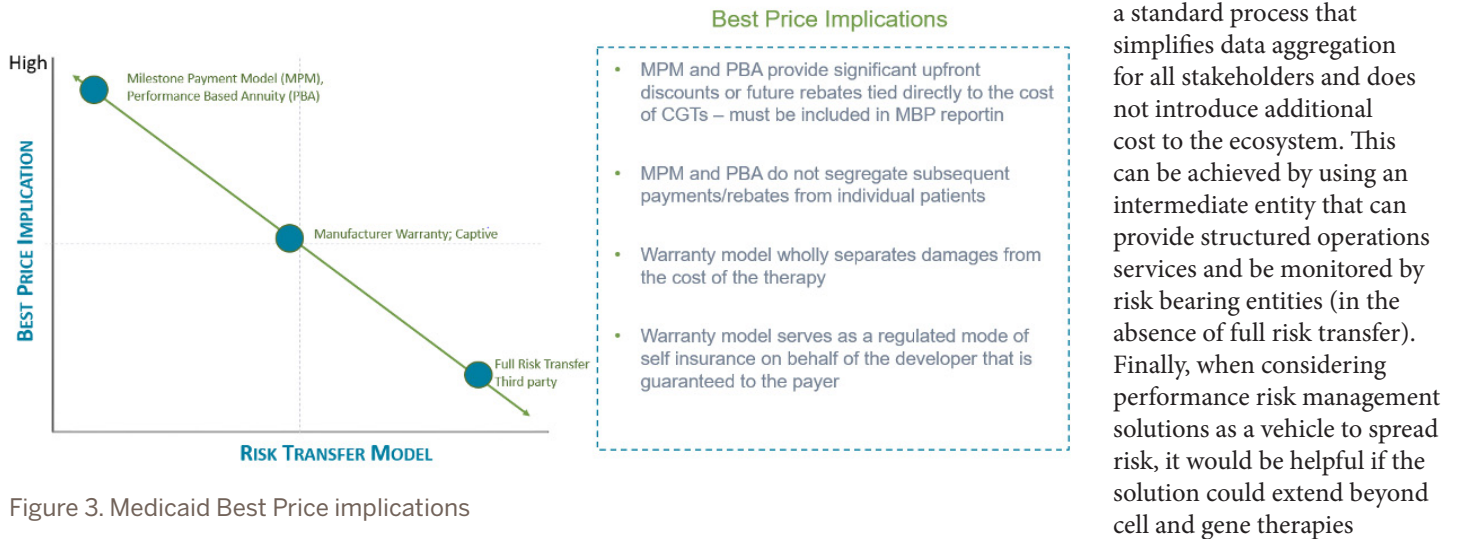


Figure 3. Medicaid Best Price implications

- **Outcomes-based rebates or installment payment plans are tied directly to the AMP of the therapy itself** as opposed to warrantied medical expenses associated with the performance of the therapy

The manufacturer warranty model allows therapy developers to leverage a self-insurance vehicle administered by the third-party OBCA and separate the financial risk associated with the outcomes-based contracts i.e., warranties. Moreover, the developers pay a premium to a developer-owned entity in exchange for a Contractual Liability Insurance Policy (CLIP). Though this insurance-based risk transfer mechanism provides developers with protection against future warranty damages payments, it is not fully insulated from MBP. The premium paid in exchange for the CLIP is still subject to MBP reporting. As part of its response to the CMS proposed rule 85 FR 37286⁸, the FoCUS consortium supported this interpretation.⁹

The warranty model was designed to meet the following criteria. Overall, when addressing therapy efficacy and durability uncertainty, the therapy cost must be separated from the outcomes-based contract for a rare condition. This is in order to avoid a large rebate for a single patient with poor results setting the national rebate for Medicaid under current interpretations of the Medicaid Best Price reporting system¹⁰. Furthermore, any future payments associated with the efficacy of a therapy must be made from the developer to the payer and must cover specific medical expenses versus an unattributed rebate (see “What Makes a Product a Good Warranty Candidate?”). In addition, when considering precision financing and performance risk management strategies, the solution must address the administrative burden associated with implementing and operating the contract vehicle. The solution should provide

⁸ <https://www.federalregister.gov/documents/2020/06/19/2020-12970/medicaid-program-establishing-minimum-standards-in-medicaid-state-drug-utilization-review-dur-and>

⁹ Letter dated July 20, 2020 in response to CMS-2482-P available at <https://newdigs.mit.edu/sites/default/files/images/FoCUS-Comments-CMS-Proposed-Rule-VBP-20200720.pdf>

¹⁰ http://newdigs.mit.edu/sites/default/files/FoCUS%20Research%20Brief_2019F201v032%20-%20Medicaid%20Best%20Price_0.pdf

UNDERSTANDING THE DURABLE THERAPY WARRANTY

The ‘warranty’ offering is a risk-sharing agreement that allows the developer to stand behind the promise of a durable therapy. The following sections will provide the fundamentals of the warranty construct and further explain how the warranty can be used across different disease states and therapeutic classes. This briefing will also focus on both the warranty construct as well as the concept of a new administrative entity referred to as an Outcomes-Based Contract Administrator (OBCA).

Durable therapies deliver variable clinical responses, even when many patients may not have any ongoing symptoms and potentially not need any further treatment. As an example, Hemophilia patients treated with durable therapies may have variability in their clinical response, yet no longer need any further Factor VIII/IX treatments. Moreover, the observed clinical responses at the time of therapy approval are often defined by limited clinical trial populations. This response variability is expected, and the standard deviation will likely increase as more patients are treated in real-world settings.

When a therapy does not meet a specified efficacy standard, the durable therapy warranty allows the developer to ‘warrant’ to the payer the reimbursement of resultant future ongoing healthcare costs. As an example, in the case of Hemophilia A, the developer may insure payers against the consequences if the durable effect diminished over time, resulting in the need for supplemental Factor VIII. In essence, the warranty would reimburse the payer for the costs of the supplemental Factor VIII if needed by a gene therapy-treated patient. The warranty would not provide any refund on the price of the gene therapy product itself. To further clarify, the therapy warranty concept is like that of a car manufacturer warranty. The car manufacturer “promises” that the core components of the purchased vehicle will operate without failure for a period of time. In the event that one of those core components fails, such as the transmission, the manufacturer pays

Durable Therapy Warranty Model



- Health plan approved of and pays for therapy



5 YEAR WARRANTY



- Developer provides warranty policy
- Health plan holds warranty in the event that the therapy fails to deliver on promise



- Warranty pays for supplemental factor needed to get the patient to proper circulation factor level
- Health plan submits claim to Outcom Based Contract Administrator (OBICA)
- Value delivered based on covered healthcare costs not a rebate

- MBP reporting compliant mechanism
- Standard claims process supports administrative simplicity leveraging existing claims channels
- Claims requirements and processes clearly defined at the time of warranty issuance
- Reduces burden on patients, providers, payers, and developers to track extraneous data outside of their current systems or protocols
- Insurance oversight guarantees solvency for claims payments via periodic audits and claims experience review

Figure 4. Warranty overview

for the replacement transmission and perhaps other costs such as a rental or loaner car, as opposed to providing the car purchaser with a refund tied to the car purchase price.

The proposed warranty program is not without medical precedent. In 2009, Proctor & Gamble and Sanofi-Aventis notified CMS and subsequently launched a Fracture Protection Program. This early-stage value-based agreement between the manufacturers of the osteoporosis drug, Actonel, and Health Alliance Medical Plans provided a warranty up to \$30,000 to “cover the costs of average medical expenses for any non-spinal fractures”¹¹. Research has not identified any adverse CMS opinions or any actions taken against Proctor & Gamble or Sanofi-Aventis as a result of the Fracture Protection Program.

What the Fracture Protection Program tried to accomplish, and what the new warranty construct is attempting to do, is to reframe what constitutes “value” when considering the elimination of ongoing healthcare costs following therapy treatment. The approach replaces existing value-based agreements in which a manufacturer refunds an amount up to the “best price” limit if the durable therapy does not achieve the clinical efficacy. The warranty model, as described herein, is designed to cover the subsequent healthcare costs beyond the best price threshold. This construct allows payers and developers to manage performance risk in a CMS (Medicaid Best Price) compliant manner, while leveraging a simpler standardized claims driven method of administration. The warranty model design provides risk sharing coverage across all payer segments compared to other value constructs that primarily work for commercial payers only. The benefits of a warranty model are:

Figure 5 and Table 2 describe a warranty in action. Figure 5 illustrates the flow of activities from establishing a warranty through the administration of the therapy, the triggering of the warranty incurred expenses from inadequate outcomes and to the adjudication and reimbursement of those expenses from the warranty fund. Table 2 further describes the main actors and their roles in the warranty model flow of activities.

A WARRANTY IN ACTION

Table 2. Key warranty model actors and their roles

Actor	Description/Role
Developer	<ul style="list-style-type: none"> • Initiates the warranty creation effort and works with OBICA to develop terms and conditions • Contracts with OBICA to use regulated entity or forms separate entity to segregate financial risk and maintain risk reserves/capital • Issues warranty to payer at the time of therapy purchase/administration • Leverages OBICA to adjudicate and pay claims

¹¹ <https://www.managedhealthcareexecutive.com/drug-costs/pharma-backs-its-products-new-contracting-deals>

Warranty Model

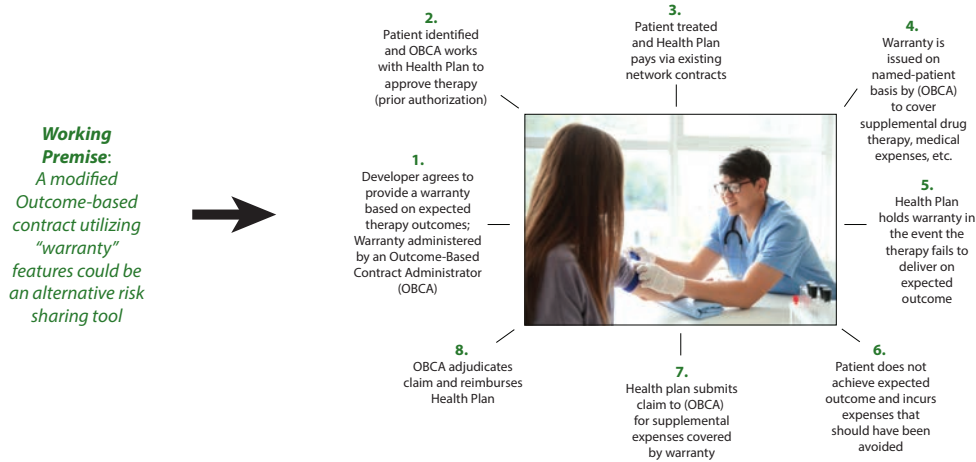


Figure 5. Warranty model flow

Actor	Description/Role
Patient	<ul style="list-style-type: none"> Receives therapy after payer approval
Payer	<ul style="list-style-type: none"> Approves therapy coverage and provides patient access Receives and holds warranty after therapy administration Monitors (or outsources monitoring of) patient outcomes against warranty terms Submits warranty claims to TPA (OBCA)
OBCA	<ul style="list-style-type: none"> Assists in developing warranty terms and conditions Manages risk capital and reserves Manages ongoing actuarial models Manages regulatory oversight Reviews, adjudicates, pays claims via coordination with payers and providers
Independent Review Organization	<ul style="list-style-type: none"> Leveraged to provide physician specialists as part of claims adjudication (as needed) Process appeals in the event initial claim disposition is appealed
Department of Insurance and Securities Board/ Department of Insurance	<ul style="list-style-type: none"> Approves Contractual Liability Insurance Policy Monitors Captive and Incorporated Cells including risk capital requirements Routinely audits operational performance of warranty, risk reserves, premium amounts, etc.

OPERATIONAL AND LEGAL CONSTRUCT

The warranty operates similarly to an insurance product and, as such, falls within the scope of insurance regulation. The following describes the associated structures and oversight as well as development considerations (depicted visually in Figure 6):

- Warranty policies are created based on the unique characteristics of each durable therapy/disease state. The terms of a warranty are best designed by a developer in consultation with payers. Together they should define the measurable ‘promise’ of the therapy as well as what potential consequences are covered in the event of failure.
- A third-party administrator, or Outcomes-Based Contract Administrator (OBCA), administers the warranties. This company can be formed in multiple ways, however the assumption is that the OBCA is an independent, regulated entity. The example entity used for this exercise is registered in the District of Columbia and is subject to the regulatory oversight of the D.C. Insurance and Securities Board (DISB). In this case, the regulated entity takes the form of a sponsored captive. The captive structure is recommended as a means to segregate financial risk associated with each warranty program.
- Manufacturer warranties are typically regulated on a state by state basis and are not related to health insurance, as they are guarantees to pay resultant damages caused by product regardless of the type of product. However, the warranty coverage is provided on a national level and pools the risk of all patients who have received a specific developer’s therapy. Thinking back to the manufacturer car warranty example, the cell and gene therapy model warranty leverages the same legal construct used by manufacturers across other industries. The construct is referred to as a Contractual Liability Insurance Policy (CLIP) and is used by manufacturers (therapy developers in this case) to purchase a warranty against their own product from a self-insured entity (the captive in this case).

Operational Model

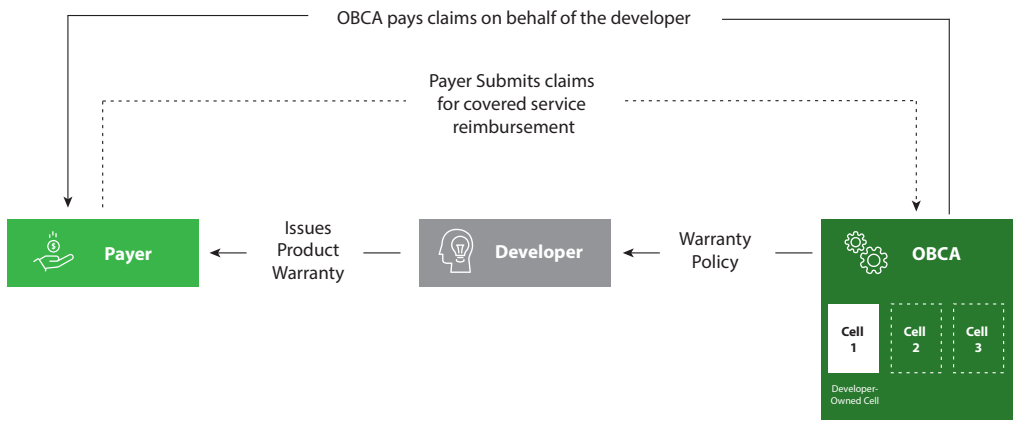


Figure 6. Warranty operational model

- The CLIP is transferrable to the end payer (payers in this case). The insurance regulators oversee the formation of the captive, require specific risk capital provisions, approve the CLIP, and routinely audit the captive entity and all business operations.
- When the OBCA operationalizes a warranty program designed in conjunction with a developer, a segregated “cell” is established within the OBCA entity and has the following characteristics:
 - Each cell is developer-specific and wholly owned by the developer (exemplified by the gray “Cell 1” in Figure 6 below).
 - The developer-owned cell houses the financial risk associated with the coverage terms of the warranty and any respective claims.
 - The developer-owned cell only contains risk associated with a single developer’s warranty policy or policies. This confines a developer’s financial risk to their cell and has no impact on other cells.
 - Based upon the actuarial risk assessed at the time of warranty creation, the developer pays a premium to the cell for each warranty policy issued (premium flow depicted in Figure 6 below with the blue arrow).
 - The OBCA aggregates the premiums from the developer, distributes them to the developer’s cell, and performs all financial administration on behalf of the developer. This includes regulatory reporting, audit oversight, and ongoing actuarial review.
 - Developers and payers enter into agreements that include the warranty policy. The policy defines the duration of coverage and for which specific services/treatments the warranty will pay claims. Additionally, the policy covers any “riders”, exclusion criteria, and transferability or termination clauses.
 - In the event of a treatment failure, the payer submits a claim

against the warranty policy to the OBCA for coverage and medical review.

- The OBCA processes the claim, determines payment, and remits payment from the specific cell related to the developer.
- If claims exceed expectations, the developer will be required to increase the cell capital reserves. Additionally, due to the DISB oversight, the capital reserves are under constant evaluation to guarantee that the cell is solvent and can pay claims.

Biomedical warranty examples

CareMetx created OutcomeRx in 2019 to develop innovative financial and insurance products for therapeutics, as well as the services to administer them as an OBCA. Beyond OutcomeRx, other organizations in the joint replacement and medical device spaces have implemented similar warranty concepts as outlined in Table 3.

Table 3. Warranty examples across healthcare space

Company	Warranty
OutcomeRx	Provides first warranty offerings for bi-pharmaceuticals including cell and gene therapies. Warranty coverage includes costs for alternative therapies, hospitalizations, and other medical expenses associated with therapy inefficacy.
Zimmer Biomet	Lifetime warranty on the Oxford Partial Knee covering revision surgery and comparable replacement product as defined in the warranty terms and conditions ¹²

¹² <https://www.zimmerbiomet.com/medical-professionals/bone-cement-warranty/terms-and-conditions.html>

Company	Warranty
Medacta and Geisinger Health Plan	Lifetime guarantee covering revision surgeries for failed joint replacements ¹³
Medtronic	Various limited warranties across product line covering replacement products or alternative products based on terms and conditions of warranty ¹⁴
OutcomeRx	Provides first warranty offerings for biopharmaceuticals including cell and gene therapies. Warranty coverage includes costs for alternative therapies, hospitalizations, and other medical expenses associated with therapy inefficacy.

WARRANTY KEY FEATURES & FLEXIBILITY

The warranty model provides a flexible and extensible construct to support a variety of business purposes on behalf of developers. The warranty policies offer coverage flexibility by allowing developers to provide highly customized risk protection for each therapy to payers in the form of covered services or damages if their therapy does not achieve the minimum benefits promised in the warranty. Additionally, the legal and insurance infrastructure described below allow developers to leverage a standardized administrative and operations structure regardless of the type of therapy under warranty. The infrastructure allows developers to separate warranty financial risk and coverage for damages in exchange for a premium amount. The following sections will provide detail on the key warranty features and the flexibility of the construct.

Table 4. Warranty key terms

Key Terms	Description/Definition
Outcome Based Contract Administrator (OBCA)	Third-party administrator contracted by developers to provide administrative, underwriting/insurance, and operational services to support the warranty model
Sponsored Captive	Regulated entity formed by the Outcomes-Based Contract Administrator (OBCA). This entity is regulated by the department of insurance and used to separate the financial risk (risk capital and premiums) associated with the warranty. For the purposes of the warranty model, the captive acts as an administrative entity that provides services such as warranty policy underwriting, claims adjudication, accounting, audit and actuarial analysis. Developers contract with the OBCA to rent these services as well as to manage the developer-owned incorporated cell (discussed below).
Developer Owned Cell	Referred to as an incorporated cell. The OBCA establishes a cell on behalf of a developer, and the entity is wholly owned by the developer. Each cell is independent and only contains risk associated with a single developer. Moreover, the financial risk of a single cell has no impact on the other cells or the sponsored captive. Similar to the sponsored captive, each cell submits a business plan to the department of insurance, receives approval, and is regularly audited by the department of insurance to ensure that ongoing operations are supported by the warranty premiums and risk capital reserve.
Risk Capital Reserve	Risk capital set aside in the incorporated cell to help fund warranty claims. The amount of risk capital is determined in part by actuarial modelling as well as monitored and approved by the department of insurance. This amount will increase as more warranties are written and claims experience is analyzed. Risk capital is coupled with ongoing premium payments to ensure the cell remains solvent in order to pay warranty claims over time.

¹³ <https://www.geisinger.org/about-geisinger/news-and-media/news-releases/2019/11/08/19/57/medacta-geisinger-extend-lifetime-guarantee-to-revision-knee-surgeries>

¹⁴ <https://www.medtronic.com/us-en/patients/treatments-therapies/deep-brain-stimulation-essential-tremor/getting/dbs-system.html>

Key Terms	Description/Definition
Premium	Standard, one-time, developer cost associated with each therapy warranty issued. The premium is based on the actuarial risk associated with therapy efficacy and not specific to any one patient’s underlying health factors. Warranties are issued on a named patient basis and subject to a fee (premium) that supplements risk reserve capital. Premiums are set based on actuarial modelling and are paid by the developer for each warranty issued. These premiums are paid through the captive and stored in the developer-owned cell, less administrative fees. Premiums and risk reserve capital are used to pay claims filed against warranty policies.
Contractual Liability Insurance Policy (CLIP)	The CLIP is a policy purchased for a premium by the developer from its incorporated cell. The OBCA performs all operational steps to purchase and issue the CLIP to the developer. The developer holds the CLIP as proof of coverage and issues an accompanying warranty to the payer at the time the therapy is purchased/administered. The CLIP allows the developer-owned incorporated cell to assume the financial liability for claims associated with the warranty. Though the financial responsibility for warranty claims still lies with the developer, the CLIP serves as an agreement between the developer and the OBCA-administered cell to pay damages in the event of a claim.
Qualifying Claim	A warranty claim is initiated by a payer as the beneficiary of a warranty policy. Claims are submitted to the OBCA by completing a claims form and required supporting documentation as detailed in the warranty policy.
Qualifying Event	A qualifying event triggers a claim and is specific to the therapy and warranty coverage. Qualifying event examples include spontaneous bleeds in Hemophilia patients, reversion to ventilator usage for spinal muscular atrophy (SMA patients), and hospitalizations for opioid overdose therapies.

Warranty policy structures

Whether to employ standardized or customized policy structures is a common question that arises when analyzing the warranty program design. Generally, it is anticipated that warranty policy terms will be standard for each therapy supported, regardless of payer segment. Also the coverage of each therapy-specific warranty policy would be non-negotiable with individual payers. It may be the case that individual developers engage in supplemental discount contracting with payers, however this is wholly separate from the warranty policies. This approach guarantees that both commercial and government payers receive the same coverage without the added burden of complex negotiations. It is recommended that prior to finalizing warranty policy coverage scope, the developers engage with multiple payers across impacted payer segments to seek input on the warranty scope. This will ensure alignment on the value proposition of the therapy with the warranty policy.

Warranty policy Qualifying Events (triggers)

Warranty policies require a specific definition of the Qualifying Events that trigger possible claims. These events may be actual events such as a hospitalization or the need for supplemental therapy due to a warranted therapy inefficacy. Due to the specificity required in defining a Qualifying Event, while not limited to these, warranties work best when applied to therapies and disease states that have the following characteristics:

- Clear and measurable clinical endpoints, preferably that allow for patient benchmarking, pre-therapy, and objective monitoring post-therapy
- Clearly defined success criteria i.e., achievement of certain clinical endpoints
- Clearly diagnosed population
- Attributable cause and effect relationship associated with an event i.e., hospitalization due to overdose

Qualifying claims

Warranty claims criteria and detailed submission details are included in each warranty policy. Each therapy and associated disease state have unique characteristics leveraged to develop the actuarial risk models and policy language. An OBCA works with developers to design their therapy warranty and efficacy promise based on factors such as the following:

- Warranty Duration (contract period)—typically 1-5 years based on the efficacy promise and clinical trial data or existing claims experience data at the time of warranty creation
- Warranty Measurement Timeframes—specific points in time when milestones should be reached based on therapy efficacy promise
- Warranty Measurement Criteria—clinical and/or non-clinical variables representing successful endpoints i.e., CHOP-Intend score, circulating factor level, dystrophin levels, etc.
 - Note: warranties often leverage a combination of variables and measurements to represent a complete claim

- Covered Services—physician, medical, alternative therapy (other Standard of Care), or other healthcare-related expenses explicitly named in the warranty
- Excluded Services—these typically represent specifically named services that could be confused with covered services i.e., if the warranty covers an alternative standard of care it may not also cover the cost of a hospitalization unless explicitly named in the covered services.

Each warranty has a detailed claim form that states the values required for a claim submission and the method for submission. The OBCA works with the payers to ensure claims forms are complete prior to adjudicating the claim. Moreover, some warranties may include clinical analyses of previously documented patient episodes that require physician review. This data is submitted as an attachment to the claims form and reviewed by one or more physicians specializing in the disease state as part of the adjudication process.

Warranty cap

One goal of the warranty model is to share risk between developers and payers in a manner that propels patient access to innovative therapies. Generally, warranties constrain the financial risk exposure for stakeholders to the amount of therapy investment. To maintain risk balance as well as accommodation for administrative expenses, warranties are typically capped at a percent of the payer's investment not to exceed 100%. Leveraging the warranty operational structure and coverage of specific damages (versus a blanket rebate) allows developers to share financial risk that exceeds the Medicaid Best Price reporting requirements, with payers. The warranty model is not providing rebates, it is a mode of self-insurance against future liability obligations (as detailed in the warranty policy) for which the developers set aside premium and risk capital to cover. Individual developers may choose to reduce the overall warranty benefit or extend it beyond the price of the therapy depending on the covered services. However, it is unlikely that developers will go at warranty risk beyond the therapy revenue incurred at the point of sale.

Riders extend the warranty flexibility and extensibility

Each warranty policy focuses on the therapy-specific developer promise, coverage terms, and triggers that can collectively result in claims payments. In some cases, it may be necessary to address additional outcomes-based scenarios that occur outside of the standard coverage. To create additional flexibility and extensibility of the warranty construct, policies may also include riders or addendums to cover specific scenarios. For example, riders can cover three extreme scenarios:

- Non-Responder Window—When the parties wish to delay the warranty coverage period or otherwise define a distinct period. Examples might include, delaying a CAR-T therapy warranty until after the initial cell infusion has proven successful, or delaying an AAV-based therapy warranty period for six

months to allow the transfected cells to reach stable clinical result. This rider also facilitates combining the warranty with other Precision Financing Solutions such as a short-term milestone-based contract.

- “Life Insurance—with some therapies the result of therapy inefficacy may be mortality. This severe outcome is possible with some existing therapies and may become more prevalent as the durable therapy space expands to therapeutic areas in which patients are treated at a late stage in disease progression. This rider allows for the developer to warranty the potentially lifesaving therapies up to a predefined amount in the event of mortality.
- Severe Morbidity Outcome—aside from mortality, there are other severe outcomes that may occur due to therapy inefficacy. Developers can also use the rider construct to provide coverage for costs associated with known severe outcomes. Depending on the therapy and therapeutic area this may also be included as part of the standard warranty language.

WARRANTY COMPLIANCE CONSIDERATIONS

Medicaid Best Price

- The use of a novel warranty approach which covers the subsequent cost of failure and does not rebate purchase price will be an acceptable means by which payers can realize acceptable remuneration for efficacy failures.
- Developers would report the cost of the warranty premium for best price calculations. Any future payments made from the warranty for covered services would not be subject to best price reporting/calculation.
- There is no legal requirement to provide a warranty to all payer segments. However, this is administratively simplest for the standard warranty. Similarly, there is no legal requirement for issuing standard warranties across segments and it is up to developer discretion. Operationally, customizing warranties per payer segment or for individual payers introduces additional underwriting procedures and negotiation between the payer and developer, which may introduce time-related barriers that impact patient access to therapies. Lastly, customizing warranties to offer different benefits to different payer segments or individual payers introduces some Medicaid Best Prices reporting considerations. However, the best price exposure would still remain restricted to the premium amounts which can be designed to remain below the 23.1% discount threshold for adult focused therapies. Additional considerations are as follows:
 - Offering a warranty only to commercial plans: Given that the Medicaid Best Price exposure is contained to the premium paid to purchase the warranty coverage, offering a warranty to commercial plans but excluding Medicaid/Medicare reduces the overall complexity of underwriting. This assumes that the therapy is efficacious enough to support a warranty premium amount below 23.1% of the AMP.
 - Medicaid and Medicare warranty recipient: Medicaid and Medicare introduce policy issuance hurdles in that

contracting must be done with the appropriate authority. However, issuing warranty policies to managed Medicaid/Medicare plans would align with the current practice of rebate and discount negotiations. In addition, the warranty, as opposed to rebates and discounts, would result in greater cost savings for Medicaid and Medicare on the warranty covered services. This is because there is less opportunity for MCOs to capture a spread, therefore passing the full savings back to the state and federal governments, which adheres to the spirit of the existing Medicaid Best Price regulation.

DEVELOPER CONSIDERATIONS

Developers will play an active role in the design, creation, and funding of the warranty policies. The OBCA will help to execute each of the steps associated with the design, as well as the implementation, operationalization, and long-term administration. However, it is important that developers understand some of the key components of the warranty construct design and how they may impact their planning processes.

- **Financial capital needs to establish ‘cell’**

A function of all insurance models is a degree of risk capital set aside to cover instances of larger than expected or catastrophic loss. Risk capital is determined via complex actuarial modeling exercises that contemplate all variables associated with the insured risk. The warranty model operates similarly to self-insurance in other markets in that risk capital must be set aside by the insuring organization to supplement premiums paid for each warranty policy. Risk capital requirements are defined initially via the actuarial modelling performed at the time of warranty creation and takes into account clinical trial data and/or market experience data, disease state characteristics, population data, etc. Furthermore, this model data is presented to the insurance board as part of the cell incorporation process and used to define the final risk capital requirements. Risk capital can then be provided in a combination of cash, line of credit, other collateral as approved by the insurance board. This provides the flexibility for both small and large developers to take advantage of the warranty construct.

- **Premium Adjustment**

Warranty premiums may require adjustment over time, based on claims experience and the increase in data availability as patients are treated. The developer-owned cell will undergo regularly scheduled audits to guarantee adequate risk capital and premium funding. These audits include a review of the actuarial modeling to incorporate claims experience data over time. If the warranty program experiences lower claims than projected, premium amounts may be reduced over time (reduction in MBP exposure). Alternatively, if the claims experience exceeds the claims projections, the premium amounts paid for each warranty may need to increase (increase in MBP exposure). Depending on the volume and payment of

claims, the captive manager and insurance board may require an off-cycle audit to adjust premiums or risk capital. Furthermore, premium adjustments do not directly impact the other stakeholders, as they are a function of the warranty’s self-insurance vehicle supported by the developers.

- **Warranty Claim Adjudication**

The warranty construct as described leverages an OBCA to issue warranty policies on behalf of developers, as well as to adjudicate and pay claims. Depending on the therapy and the damages the warranty covers, claims adjudication may be complex and require physician review. This detailed review can be performed by the OBCA or an independent entity as required. As described in the operational model, claims are submitted directly to the OBCA by payers along with required supporting material. Instructions for claims submissions, claims forms, and submission options are detailed in the warranty policy documentation and delivered to the payer at the time of issuance. To reduce administrative complexity, the claims submission process is standard across warranty policies for all therapies and payers. This standardized submission process also alleviates much of the burden traditionally associated with data aggregation for other outcomes-based contract arrangements. Moreover, this reduces the regulatory costs of complying with department of insurance oversight of the claims process.

- **Role for ‘re-insurance’ of the cell?**

The department of insurance may advise the OBCA/captive sponsor and developer to obtain reinsurance on the warranty risk assumed under the incorporated cell. This is not an initial requirement but may be a desirable approach to protect against greater than expected damages. For this reason, an OBCA that is also able to issue reinsurance is best suited to administer the warranty.

PAYER ELIGIBILITY FOR WARRANTY PARTICIPATION

Warranty impact on payers by segment

The warranty model serves as a means to protect the individual plans, the patients they cover, and the government payers against efficacy and durability (performance) risk—and allow them to contain future cost exposure in the event that the durable therapies do not adhere to the developer’s promise. Many assume that outcomes-based contracts are only viable in the commercial payer market. However, the warranty and associated administration model may create significant value for the Medicaid and Medicare payers/administrators. Medicaid and Medicare plans are administered by independent entities that have similar risk exposure to their commercial counterparts and these plans must also consider both current and future cost containment mechanisms. Moreover, by embracing outcomes-based models such as the warranty, the healthcare system can address the costly populations that impact the entire ecosystem. As an output of the Design Lab, FoCUS hopes to extend the thought leadership on this topic at a macro level.

Medicaid plan eligibility

Medicaid plans could participate in the warranty either through a supplemental rebate agreement or through their MCOs. State Medicaid programs would receive their normal rebates in addition to the warranty protection.

States with prohibitions due to legislation or state constitution that prohibit multi-year Medicaid cost agreements would likely also be prohibited from accepting a warranty, but this will require assessment in the context of each state's specific language.

Medicare plan fit

The warranty is designed to benefit all payer segments by offsetting future covered medical expenses introduced when cell and gene therapies meet the trigger criteria. However, some government programs, as currently structured, may not benefit from the warranty construct. For example, the classic Medicare structures (Part A, B, and B+Medi-gap) are not well suited to participation in the warranty, based on the Medicare coverage divisions, if the expected warranty reimbursed expenses do not occur in the same division as the one-time therapy costs. For example, CAR-T costs will fall within Medi-gap coverage (administration in Part A) which would likely not include costs of therapy failure borne by the Part A or B coverage, such as an adverse event hospitalization. Therefore, there is a misalignment of risk and benefit from the warranty. It could be argued that Medicare as a whole would benefit from a warranty structure if the full benefit of the warranty was passed back to the Medicare program and patients by the Medi-gap carriers, however this is not currently required as part of the Medi-gap standard coverage rules. Moreover, this misaligns the goals of the warranty from a Medi-gap carrier perspective.

Medicare Part D presents similar complexities in regards to expense responsibility and accountability. At the extreme, Part D only plans would cover the costs of Part D covered therapies, but could only claim future Part D covered expenses when many costs might occur in Part A or Part B.

Alternatively, Medicare Advantage (MA) is well suited to support the warranty construct given the robust coverage provided under MA plans. Moreover, MA plans leverage cost-containment mechanisms that help protect patients against catastrophic financial exposure. If a warranty that covers the cost of the cell and gene therapies was issued to a MA carrier, they hold the ongoing durability risk exposure for as long as the patient remains on the plan—similar to commercial insurance arrangements. It is also conceivable that Medicare Advantage plans could leverage warranties issued as part of their annual pricing submission and rebate arrangements with Medicare, which would positively benefit the MA plans themselves, as well as the patients, and Medicare as a whole.

Also like commercial plans, patient mobility affects warranties for MA plans. Unlike commercial plans, Medicare warranty portability may be possible between MA plans within individual counties. Legislative or regulatory changes that establish patient mobility tracking and either data exchange or warranty assignment mechanisms, could address this issue in ways not easily available to commercial and self-insured plans.

340B implications

The warranty is designed to provide durability protection based on the manufacturer determined coverage limit. Though the 340B program offers price concession for Centers of Excellence (COEs) and academic medical centers, it does not dictate the final cost of the therapies recognized by health plans. Moreover, the benefit of the warranty is provided to the health plan which would potentially incur future costs if the therapies are less than efficacious, not the COEs. Therefore, no direct correlation between the 340B facilities and the warranty exist in the current warranty construct.

Self-insured employers

Self-insured employers often rely on external administrative entities such as TPAs to perform their benefit administration. Though the warranty policies are issued to the self-insured entities themselves, they can work with their TPAs and/or PBMs to perform the warranty policy tracking. This may include services such as patient claims monitoring, periodic medical reviews, or patient follow-up. The OBCA can be contracted to also help the payers follow up with the patients covered by warranties. This may include patient and physician outreach as well as compliance and adherence services.

Re-insurance and stop-loss insurance

Re-insurance and stop-loss carriers typically offer one-year contracts and do not participate in drug price and rebate negotiations. However, they do occasionally gain access to the net therapy prices and may reduce the claim reimbursement amount accordingly.

While the claims-based reimbursement model is highly similar to the re-insurance and stop-loss mechanics, the use of additional performance information to trigger a claim presents an additional operational burden, beyond the usual total event cost attachment point mechanism.

In addition, a multi-year warranty, like all multi-year precision financing approaches, will likely prove difficult to implement as part of the one-year re-insurance or stop-loss contracts. Patient mobility issues will be amplified by client (insurer or employer) turnover for the re-insurance or stop-loss carrier. Carriers seeking to 'see through' to warranty payments might increase their client turnover by potentially creating an incentive for clients to change carriers to avoid disclosing those future warranty reimbursements.

PATIENT PARTICIPATION IN WARRANTY

It is hoped that warranties will aid payers in providing patient access to durable cell and gene therapies. The warranty construct is designed to have minimal financial impact, if any, on the patient. Warranty coverage is written to work within the bounds of the therapy protocol and provide financial risk protection to the payers. For this reason, the primary triggers for warranty claims are future costs that can be monitored by the payers (or their administrative entity), as well as the outcome of testing performed as part of the normal course of the therapy care plan. Moreover, payers will be responsible for encouraging patients to adhere to treatment protocols and follow-up testing. As mentioned previously, this can be accomplished via ongoing patient engagement services administered by the payers or an independent entity such as the OBCA. There is a risk that patients may disengage from the ongoing monitoring however this puts their own health at risk. Payers may also choose to leverage benefit design to further encourage patients to adhere to ongoing monitoring protocols that support warranty damages reimbursement. Overall, continued engagement with patients and their families is viewed as an important factor to cell and gene therapy success (with or without a warranty in place).

Patient financial sharing implications for co-pays, deductibles, and co-insurance would also be unaffected for those future treatments. An initial goal of the warranty construct was to pass future cost savings on to patients in the form of reduced cost sharing requirements for future costs associated with cell and gene therapy inefficacy. However, this feature remains at the discretion of the individual payers and is not specified in the warranty terms. Given that each warranty is unique to the disease state and therapy, cost sharing adjustments may also need to be therapy and warranty specific. This would require proactive plan benefit design to account for warranty coverage.

Under commercial and Medicare plans patients may be exposed to co-pays, co-insurance as well as future yearly deductible limits for treatment necessitated by cell and gene therapy performance. These might trigger the warranty. This could lead to payers receiving partial double payment from both the patient AND the warranty.

WHAT MAKES A PRODUCT A GOOD WARRANTY CANDIDATE?

The warranty construct is extensible by design to accommodate the unique characteristics of each therapy and disease state. Despite the flexibility of the model, not every disease state or therapy is a viable candidate for warranty coverage. When evaluating a therapeutic class/therapy there are a number of factors considered to determine if a warranty is the appropriate outcomes-based contracting model (note - the warranty model can apply to therapies outside of the gene and cell therapy space, therefore some considerations may not apply to the durable therapy market.)

When applied to a specific therapy warranty, the matrix shown in Table 5 produces a profile that can be used to determine if a warranty is recommended. While all factors deserve consideration, assessing the three bolded items below are critical for success. Additionally, the output helps to identify possible riders or addendums to the warranty that may align with existing constructs such as the life insurance rider, or potential new riders that may be needed for a novel therapy.

As mentioned in previous sections related to warranty candidacy, not all therapies or disease states are suited for warranty coverage. For example, Hemophilia as a disease state is an ideal warranty candidate due to a few key components:

- **Therapy Characteristics:** The therapy will likely be delivered in a few, well-controlled Centers of Excellence
- **Standard of Care (SOC):** Prophylactic and event-driven use of replacement clotting factor
- **BioMarker/Clinical Endpoint:** Clear, quantifiable endpoints from molecular markers (clotting factor levels) to clinical phenotypes (bleeding events) and eliminated alternative standard-of-care costs (factor VIII and IX replacement therapies and hospitalizations) exist

Alternatively, therapeutic areas in the ultra-orphan archetype (such as SMA and DMD) may present warranty policy coverage challenges due to the absence of clear clinical endpoints or the highly invasive nature of measuring clinical endpoints. However, there is still an opportunity to construct warranties that will create value for patients and payers by helping to protect them against healthcare costs associated with therapy inefficacy.

Table 5. Warranty criteria matrix

Topic	Inputs/Considerations
Characteristics of Therapeutic Area	<p>Population size of therapeutic area</p> <p>Therapeutic area description i.e. what are the clinical and non-clinical components of the disease, are there multiple types, common comorbidities, etc.</p> <p>Population description i.e., patient demographics, incidence and prevalence rates.</p>
Therapy Characteristics	<p>Clinical description of how the therapy addresses the disease, genetic deficiency, or other conditions</p> <p>Developer's therapy promise i.e., how does the developer define the clinical and non-clinical efficacy</p> <p>If the therapy has not launched - clinical trial inclusion/exclusion criteria as well as primary, secondary, tertiary outcomes of the study</p> <p>If existing, what is the market data on efficacy, label and indications</p> <p>Therapy considered chronic or durable/curative</p> <p>Therapy administration method/modality</p> <p>Therapy administration location</p>
BioMarker/ Clinical Endpoint	<p>Clinical endpoint(s) for the disease</p> <p>Define non-clinical endpoints and/or other efficacy measures</p> <p>Define the outcome measurement method. Is it easy or invasive?</p>

Topic	Inputs/Considerations
Standard of Care (SOC)	<p>Define existing SOC if one (or many) exists</p> <p>Define the average SOC cost</p>
Therapy Cost	<p>Define the cost (actual or anticipated) of therapy being evaluated</p>
Competitive Landscape	<p>Identify other products that exist to treat the disease</p> <p>If competitors exist, how many (estimate)</p> <p>Define the characteristics of the competitive therapies</p>
Developer Goals	<p>Increase access</p> <p>Reduce rebate exposure</p> <p>Drive market adoption</p> <p>Drive competition based on efficacy</p>
Patient Goals	<p>QoL enhancement</p> <p>Survival</p> <p>Reduce reliance on chronic therapy</p>
Provider Goals	<p>Patient survival</p> <p>Patient QoL enhancement</p> <p>Patient outcomes</p>
Payer Goals	<p>Return on investment or future cost offset</p> <p>Cost savings (short and/or long term)</p> <p>Achieve greater efficacy than existing therapies</p>

WARRANTY FIT WITH PRODUCT ARCHETYPES

While each therapy deserves a thoughtful independent analysis, some generalizations are emerging regarding the usefulness and challenges of the warranty model to the FoCUS durable cell and gene therapy product archetypes. For further information regarding FoCUS Product Archetypes see the “Framework for Precision Financing” FoCUS Research Brief (link: http://newdigs.mit.edu/sites/default/files/FoCUS%20Research%20Brief_2018F203-015_0.pdf) or the “[Designing financial solutions to ensure affordable access to cures](#)” FoCUS white paper.

Orphan condition (orphan disrupters)

Refers to a diagnosed population with significant historical costs per patient which may vary by type and severity of the disease (e.g. Hemophilia, beta thalassemia). Therapies that fit into this archetype typically represent the best candidates for product warranties due to the known populations and standard of care costs. Additionally, these therapeutic areas often have easily measurable clinical endpoints. Moreover, the known efficacy and variability in the standard of care support warranty language that provides parity with the standard of care.

These conditions also introduce a number of considerations when leveraging a warranty in conjunction with durable therapies. One such consideration is adjustment to efficacy risk over time. At launch, gene and cell therapies generally have limited clinical trial data concerning durability and efficacy. This creates opportunities for outcomes-based tools such as the warranty to manage the uncertainty. Over time as more data become available, the warranty policies issued in subsequent years may materially change inclusion/exclusion criteria as well as premium amounts. This raises questions on how stakeholders may be impacted over time as the warranty terms change and/or the premium fluctuates to account for increased or decreased efficacy risk.

Additionally, the warranty construct provides a viable solution to the cost sharing as a function of benefit design for the therapies. Because payers are protected against some or all of the downside performance risk for durable therapies, they may use the warranty as a justification for reducing or removing patient cost sharing requirements. Currently, when a warranty is available, this is a common discussion topic, and offering, from payers - but an example has not emerged. And questions remain regarding the overall implications across stakeholders of such changes to benefit design.

Ultra-orphan condition (novel breakthroughs)

These conditions are dominated by incidence, and include therapies for populations under about 1,000 cases per year with few existing treatments such as Spinal Muscular Atrophy (SMA), Duchenne Muscular Dystrophy (DMD), and Liposomal Storage

Disorders (LSD)¹⁵ Existing treatments may be either high or low cost. Where other treatments do exist, they typically only address subsets of the diagnosed populations. Additionally, there is often wide variability in the impact of the diseases across different patient sub-populations which are often clinically characterized by disease severity or molecular mutation. For example, SMA is clinically divided into types 1-4 where each type has a different severity and time frame for diagnosis. Due to the variability in these disease states, the indications of the therapies available, and the possible severe consequence of therapy inefficacy, the warranty construct provides the flexibility necessary to address a range of considerations.

This archetype provides significant opportunity to pressure test the flexibility and extensibility of the warranty construct as well as introduce new concepts regarding benefit and formulary design.

Larger population (quantum leaps)

Refers to larger population disease states (e.g. Wet Age related Macular Degeneration (Wet AMD), Sickle Cell Disease) that often have multiple standards of care and treatment options. Similar to the other disease states, they may also be characterized by various disease states that impact patients morbidity and mortality and their treatment options. Due to the variety of treatment options (or lack thereof) across disease states, there is a need for highly targeted warranties or broad-based warranty coverage depending on the developer promise and indication for each therapy. Additionally, developing warranties for these therapeutic areas may need to especially consider issues such as patient mobility and different value propositions across payer segment.

Cell-based oncology products (CAR-T/TCR)

This durable therapy class includes CAR-T therapies, as well as solid tumor cell therapies (TCR) and transplant/transfusion related therapeutics. To date, these product costs have been lower than gene therapies for ultra-rare conditions, albeit the ancillary medical costs due to ablation, cell harvesting, and adverse event treatment may equal the therapeutic cost. The developer warranty is typically constrained to the cost of the therapy itself, which introduces complexity when considering this class of therapy, due to the likely use of adjuvant or other therapies over time. Moreover, the initial efficacy determination period for outcomes-based contracts has been as short as 90 days. However, the 3- to 5-year efficacy durability experienced by some patients presents an opportunity for developers to warranty costs for a longer period. The non-responder window rider enables the warranty to begin after an initial short-term milestone-based rebate or refund period.

Oncology can exacerbate certain challenges for longer-term warranties.

¹⁵ SMA: Spinal Muscular Atrophy; DMD: Duchenne Muscular Dystrophy; LSD: Lysosomal Storage Diseases)

- **Sustained Durability Metric:** The complex, polypharmacy and multi-modal treatment regimens in oncology can make defining a triggering metric attributable to the therapy difficult.
- **Distinguishing therapeutic failure costs from Standard of Care costs** can similarly be difficult to distinguish. This could lead to higher administrative charges and adjudication disputes.
- **Relative therapy cost share:** Related to the above two points, the cell therapy cost may not be the majority cost of care over the multi-year period. Payers and providers may not find the warranty reimbursement amounts large enough to isolate in a risk sharing agreement.

WARRANTY IMPLEMENTATION CONSIDERATIONS

As referenced in the previous sections the OBCA develops unique warranty policies for each therapy supported. Though therapies can be classified into different archetypes that leverage similar warranty terms, the combination of warranty coverage types and specific developer promises will vary. The following sections outline some key implementation considerations and challenges when developing unique warranty policies.

Patient mobility

Patient mobility creates challenges of patient data access and raises issues about sharing or transferring outcomes-based contract obligations and benefits. The warranty model also faces these issues.

Patient mobility reduces the warranty value by impairing the patient monitoring ability of the original payer as well as shifting the incurred costs to a subsequent payer.

FoCUS has proposed a possible solution for performance-based annuities in which voluntary payer consortiums shift both obligations and benefits. [see [Impact of Patient Mobility on Annuity/Performance-Based Contracting.](#)] Similarly, warranties might employ transferability riders by payer segment to minimize regulatory and reimbursement rate disparities. For example, a Medicaid plan consortium either among states or among the MCOs within or among states. Similarly, commercial plans in regional subsets may be well positioned to leverage the standardized warranty coverage structure to develop mutually beneficial agreements. As issuers of the warranties, developers could play a catalytic role in organizing such consortia.

Simple warranty transfers (having the warranty ‘follow the patient’) create subsequent challenges of potential windfalls for payers who did not incur the original cost of therapy. With larger patient numbers, payers may expect to gain as much as they lose. But with most cell and gene therapies treating rare conditions, this financial balancing is unlikely. Consortia could choose to sell the warranty among each other when patients move. But high transaction costs from negotiating the transfer rates and operational administration costs may prevent the evolution of such a warranty transfer mechanism.

In the absence of a patient mobility solution, the perceived warranty value by payers will simply be reduced according to their expectations of member retention in their plans.

COMBINING WARRANTIES WITH OTHER FINANCING TOOLS

The financial challenges associated with innovative cell and gene therapies are not constrained to cost and durable benefit alignment but also the upfront cost itself. Current cell and gene therapy list prices range from ~\$300k to \$2.1M based on the therapy, and it is anticipated that emerging therapy costs may reach or exceed \$3M. The warranty model focuses on the therapy durability and regulatory challenges that exist in today’s current regulatory landscape. Previously proposed models inextricably tied therapy performance financial remediation to price, which resulted in Medicaid Best Price Reporting barriers. The warranty model is a performance-based financing solution designed to align with other financing solutions that addresses the one-time high cost considerations in the cell and gene therapy market. The following sections outline the alignment of the warranty model with existing and evolving precision financing strategies.

Installment plan combination

An installment plan or more traditional financing mechanism that allows developers to recognize the full cost of therapies at the point of sale, while allowing payers to pay over time, provides a viable payment option to support payers that cannot absorb full therapy costs at one time. When combined with the warranty, this type of model not only allows patients to gain access to costly therapies but also allows payers (i.e., self-insureds) to spread their costs over time. Using the Hemophilia disease state as an example, this type of model allows payers to accept the full cost as payable, but also align the funds they reserved in anticipation of ongoing Factor VIII/IX costs with the potentially durable benefits (cost offset) recognized over time, as a gene therapy reduces or eliminates the need for supplemental factor. If the treated patient does need to utilize supplemental factor, the warranty covers the cost for the payer, thus keeping their financial payments standard and eliminating additional cost exposure.

Benefits:

- Patients get access to therapy by removing one-time cost burden from payers
- Developers recognize revenue upfront
- Payers align cost with durable therapy benefit
- Warranty protects payers from future cost exposure while they continue to pay down financing arrangement

Challenges

- Payers will likely need to absorb interest payments associated with the financing mechanism
- May require a need for a third party to service the financing vehicle (a role that can be played by the OBCA) or payer intermediary
- If the patient leaves the plan post therapy, the payer must still continue with payments based on the financing contract (loan)

Performance-based subscription model

A performance-based subscription model presents a viable alternative for addressing the durable therapy financial challenges while also providing efficacy protection when combined with the warranty. This model could take on a few forms and presents an opportunity to service multiple payer segments simultaneously i.e., government sponsored, commercial, self-insured. At a high level this model allows payers to pay a “subscription” fee for unlimited access to covered therapies based on qualified need. As a risk mitigation strategy, this is a superior model as it addresses the actuarial risk along with the one-time cost exposure. However, it does not address therapy durability, which could result in additional cost to the payer if the therapy does not work as promised by the developer.

Benefits:

- Addresses actuarial risk for payers
- Provides a viable solution across all payer segments
- Aligns subscription cost with actual risk (note: this depends on the actuarial rating model and the entity offering the subscription)
- When combined with the warranty, provides comprehensive coverage for all three major financing challenges while limiting Medicaid Best Price reporting implications

Challenges:

- Depending on the structure of the subscription agreement it may cause confusion as to who benefits from the warranty (see role of stop-loss and reinsurance).
- The subscription model may require a third party to service the subscription vehicle
- Payers are exchanging subscription fees for future coverage of events that may not occur depending on the prevalence of the disease and geographic variables

Milestone-based contract combination

Some existing and emerging therapies (CAR-T, TIL, etc.) produce a more immediate, measurable result (<6 months) that allows developers and payers to leverage creative billing practices that address cost and avoid Medicaid Best Price reporting requirements. For example, with some CAR-Ts, efficacy can

be measured over a short period of time which allows the developers to delay payer invoices until certain clinical milestones are reached. If the patient does not meet those milestones, the developer can either withhold the invoice altogether or invoice the payer and then offer a product refund for damaged goods. In either case, there is no impact to Medicaid Best Price reporting based on the current regulatory exclusions (and supplemented by reasonable assumptions regarding reporting requirements submitted by developers). For non-responders (patients that do not meet the initial clinical milestones), this is an effective mechanism. However, payers remain at risk for patients that meet the initial clinical milestones but fail to experience a durable benefit after the initial milestone period. If the warranty were paired with the milestone model, it would address two key issues: immediate response and long-term durability. Additional benefits and challenges are as follows:

Benefits:

- Sets pre-defined monitoring periods and clear outcomes
- Addresses immediate non-responders and longer-term durability (1-5 year duration)
- May reduce the need to monitor specific claims and allows the warranty to cover more general damages

Challenges:

- Requires longer term durability data on behalf of manufacturers to accept risk—this may not be available from clinical trials at launch
- It is more difficult to align the warranty to specific medical, physician, treatment costs associated with unmet durability milestones
- Proactive patient and provider engagement may be required to track data over time, however this is a benefit when leveraging an OBCA that can perform the wrap around monitoring service with the warranty, thus removing administrative burden.

CONCLUSION

Future directions

The warranty model offers significant opportunity to push the boundaries of outcomes-based contracting beyond single patient and single therapies. Looking forward as the cell and gene therapy market expands and competition emerges for conditions such as Hemophilia, there is an opportunity to focus on the disease state as a whole and aggregate warranty coverage around standard metrics. As mentioned in regards to the individual therapy warranty model, an aggregated model will also work better with some therapeutic areas than others. The goal of the warranty model (and all Precision Financing Solutions) is to drive patient access and align risk between developers and payers. Operating on this premise, the healthcare ecosystem should support comprehensive solutions that focus innovative and durable therapy proliferation. Some additional opportunities that should

drive future action as more durable therapies enter the market are as follows:

- Structured warranty portability driven by payer coalitions similar to the one proposed for the performance-based annuity pilot in Massachusetts
- Extend warranties to traditional specialty therapies to help drive impactful innovation and therapy differentiation
- Incorporate validated patient-reported outcomes measurement methodologies to extend warranty coverage beyond purely clinical measures and to provide risk sharing mechanisms for therapies whose benefits are not easily measured clinically
- Leverage warranties to:
 - Drive treatment regimens that are best aligned with the patient's needs
 - Reduce the volume rebates with an outcomes approach that aligns net cost with therapeutic benefit
 - Protect the spirit of Medicaid Best Price rebate program, while ensuring that patients gain expedited access to therapies and achieve meaningful outcomes

The warranty model is designed to reduce barriers to patient access, to improve performance uncertainty risk sharing between developers and payers, and to provide a flexible structure that could be implemented under current Medicaid Best Price rules and Anti-Kickback Statutes.

Pressure-testing by the FoCUS consortium revealed implementation challenges that are common themes for all performance-based contracting solutions, including agreeing on performance and patient mobility impacts across payer segments. However, the warranty provides a flexible construct that is useful for conditions in which clear performance metrics can be established. The warranty model provides a mechanism for developers to stand behind innovative therapies while allowing payers to reevaluate benefit design and reduce reliance on rebates as a cost containment mechanism.

Moreover, the warranty model presents an opportunity to combine Precision Financing Solutions, such as milestone-based payments, performance-based annuities, and subscription models, to support improved, sustainable patient access to durable cell and gene therapies.

Overall, the warranty model provides the healthcare ecosystem a viable tool to help place patient therapy access and outcomes at the center of the adaptive biomedical innovation process.

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.