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FoCUS

Financing and Reimbursement of Cures in the US

WHITE PAPER

Emerging market solutions for financing and reimbursement of durable cell and gene therapies

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Contents

EXECUTIVE SUMMARY

The FoCUS project was launched in 2016 with the aim of developing precision financing solutions that address the challenges and financial impact created by durable cell and gene therapies entering the US healthcare market. While these innovative therapies can deliver significant health benefits for patients, the performance and actuarial risk associated with these products are concerns for stakeholders. When combined with the high upfront cost of treatment, the combined uncertainty means that payers face a high level of risk associated with therapy coverage.

Stakeholders are therefore exploring alternative, innovative approaches to address the barriers of cost and uncertainty associated with cell and gene therapy to facilitate patient access and sustainable reimbursement. With an aim towards identifying and describing existing and emerging products available in the healthcare market that address the financing and reimbursement challenges of these durable therapies, FoCUS conducted a request for information (RFI) survey with organizations known to offer these products.

The survey comprised 45 questions covering details such as product history/description, financial challenges addressed, impact on patients and providers, data tracking, quality assurance and performance guarantees. Responses were received from 12 companies representing 16 services or products: Audaire Health; August Care; BCS Financial; BlueCross Blue Shield Association; CVS Health (2 products); Emerging Therapy Solutions (3 products); Evernorth; MedImpact Healthcare Systems; OptumRx; OutcomeRx (2 products); PayRx; and Real Endpoints.

Broadly, the products fall into four categories: payer reinsurance/stop-loss/risk carve out solutions (7 products); contract negotiation and data management services for payers and pharmaceutical companies (2 products); provider contract negotiation services (2 products); or financial and pharmaceutical company warranty services (3 products). Nearly all products covered durable gene therapies and around half covered cell therapies (i.e., approved CAR-Ts) as well; one solution covered cell therapies only. Many products address multiple financial challenges related to cell and gene therapies, however, there was no single, system changing product or service that would address all the concerns and needs of all stakeholders. Challenges addressed by these products included:

- Risk: actuarial/financial risk, performance risk, payment timing
- Cost: product costs, ancillary costs
- Administration: data tracking, contracts handling
- Patient/provider: Centers of Excellence (COE) access, patient access, patient care

Most market solutions have established methodologies or systems to track patient outcomes over time, regardless of the type of product. Five organizations included performance guarantees as part of their solution. Specific details of the performance guarantees were generally not made available, although it was clarified by most respondents that their guarantees are customized by product and/or payer specification.

None of the products require additional payment from the patient over and above their usual plan benefit cost share for the durable therapy. However, several products required both patients and providers to agree to additional responsibilities upon treatment with a cell or gene therapy, such as data sharing or clinical follow-up to assess outcomes.

Overall, the survey has shown a wide range of products are already available in the market that provide diverse approaches to addressing stakeholder needs. These market solutions are in various stages of maturity. Because the market is still evolving and relatively few cell and gene therapies have been approved to date, stakeholders may not yet be motivated to move away from individually negotiated arrangements. However, as more cell and gene therapies emerge over the next decade, these market solutions may become more popular, and a shift toward particular solutions may appear.

FoCUS anticipates providing a resource within the FoCUS Toolkit (https://payingforcures.mit.edu/toolkit/) that will summarize key considerations for stakeholders wishing to understand how to approach evaluating market solutions in this arena. FoCUS is dedicated to education in this arena and will continue monitoring of the market for key trends.

INTRODUCTION

The Financing and Reimbursement of Cures in the US (FoCUS) project was launched in 2016 with the aim of developing precision financing solutions that address the challenges and financial impact created by durable cell and gene therapies entering the US healthcare market.

While these innovative therapies can deliver substantial health benefits for patients, long-term safety and efficacy outcomes, particularly related to the durability of response or cure, have often not been fully established upon the drug's approval. This evidence gap, combined with the high upfront cost of treatment, means that stakeholders face uncertainty regarding appropriate therapy coverage. In addition, payers may face considerable actuarial risk, meaning there is uncertainty around the number of members or patients who might require cell or gene therapies in their plan populations.

While payers have tools to manage healthcare costs and utilization (such as benefits coverage, prior authorization and step therapy), most of these traditional techniques would result in limited access for patients who would benefit from cell and gene therapy. Stakeholders are therefore exploring alternative, innovative products or programs that could address the barriers of cost and uncertainty associated with durable cell and gene therapy and facilitate patient access and sustainable reimbursement. The FoCUS project has developed multiple precision financing solutions to meet the needs of stakeholders wishing to engage in innovative contracts for high-cost gene and therapies. These models include:

1. Milestone-based contracts: a type of performance-based contract in which a pharmaceutical company guarantees to refund the cost of therapy (partially or fully) to the payer if an agreed outcome is not achieved.
2. Warranty: a pharmaceutical company purchases a patient-specific warranty policy that reimburses treatment-related costs for suboptimal performance to payers over an agreed time period. The value is related to covered healthcare costs and is not a refund for the cost of the treatment (1).
3. Performance-based annuities: a type of performance-based contract in which payments for a cell or gene therapy are spread over multiple years and linked to therapy performance. If a therapy fails to deliver an agreed outcome, no further payments are made.
4. Orphan reinsurer and benefit manager (ORBM): a risk pooling solution to manage actuarial risk and executional challenges, including contracting, reimbursement and care coordination (2).
5. Subscription model: a pharmaceutical company provides treatment for a set fee regardless of the number of patients treated or a set price per patient (3).

Multiple implementation challenges have also been identified by FoCUS as part of the development and assessment of these financial models. These include tracking of performance outcomes; access to performance data, patient mobility (i.e., when a patient switches payers); and federal polices, such as Medicaid best price rules, HIPAA rules, and the Anti-Kickback Statute.

As of May 2021, seven durable cell and gene therapies have been approved and launched in the US market: Abecma, Breyanzi, Kymriah, Luxturna, Tecartus, Yescarta and Zolgensma (4). With an aim towards identifying and describing existing and emerging products available in the healthcare market that address the financing and reimbursement of these recently launched cell and gene therapies, FoCUS conducted a request for information (RFI) survey with organizations that offer these products. This paper provides an overview of the products identified, based on details provided from respondent companies and aims to provide stakeholders with practical information about the availability of solutions in the real world.

METHODOLOGY

A specialist FoCUS research group was created with the aim of identifying and describing existing and emerging products available in the healthcare market that address the unique challenges of financing and reimbursement of durable cell and gene therapies. A request for information (RFI) survey with a total of 45 questions in the following major categories was developed:

- Respondent's organization information
- Product history and description
  - Therapies included
  - Targeted customers
  - Eligibility requirements
- Financial challenges addressed by product
- Impact on patients and ability to address patient mobility
- Data tracking of financial and clinical information
- Quality assurance
- Patient, provider and customer educational support
- Patient or provider requirements

Organizations that offer market solutions were identified by the research group. Standard stop-loss and reinsurance providers were included if they offered a product specifically designed to cover durable cell and/or gene therapies. Respondents had 4 weeks to respond. Responses were open-ended; there were no requirements to answer all questions or provide a specific level of detail.

After review of the responses, the research group agreed to follow-up with respondents for further questions requesting more details regarding how a financial product solved for the challenges identified by respondents, and specifically clarifying whether the product assumed financial risk.

Twelve companies were invited to participate in the survey; all agreed to participate and completed the survey and follow-up questions.

OVERVIEW OF SURVEY RESPONSES AND PRODUCTS AVAILABLE

Responses were received from 12 companies representing 16 services or products aimed at addressing the challenges associated with the financing and reimbursement of durable cell and gene therapies: Audaire Health; August Care; BCS Financial; BlueCross Blue Shield Association; CVS Health (2 products); Emerging Therapy Solutions (3 products); Evernorth; MedImpact Healthcare Systems; OptumRx; OutcomeRx (2 products); PayRx; and Real Endpoints.

As most of the survey questions were open-ended, the extent of information received for each product/solution varied. In some cases, responses were limited by confidentiality. Most of the products are new, having launched since 2020. Solutions from BlueCross Blue Shield Association, OutcomeRx and Emerging Therapy Solutions have been available since 2019.

A diverse range of products were represented in the responses. Broadly, the products fell into four categories, although some solutions offered services that overlapped multiple categories (Table 1). Eligibility for access to most products was relatively open; 10 respondents stated that their products were available to all prospective customers. Blue Cross Blue Shield Association and CVS Health products are for current customers only. OptumRx products are currently available for new or existing OptumRx customers only.

Payer reinsurance/stop-loss/risk carve out solutions

Reinsurance/stop-loss/risk carve out solutions included the largest number of products in the survey – a total of seven were identified. Targeted customers are self-insured employers, commercial health plans, Medicare and Medicaid.

BCS Financial, CVS Health and OptumRx offer stop-loss products for self-insured employers that provide coverage for specified high-cost gene therapy claims in exchange for a fixed per member or employee per month (PMPM or PEPM) fee. BCS Financial's product is intended for jumbo employers who would typically self-insure with no stop-loss coverage. CVS Health's stop loss is for Caremark or Aetna self-insured employer groups without stop loss; there are additional eligibility requirements associated with the product. OptumRx's Gene Therapy Risk Protection currently requires customers to have pharmacy benefits through OptumRx; the product also provides cost and quality management services, including outcomes-based contracts, to lower drug costs and ensure clinically appropriate access.

MedImpact Healthcare Systems and OutcomeRx offer specialty carve-out reinsurance products: MedShield and Patient Access to Costly and Curative Therapies (PACCT), respectively. Both products charge a PMPM or PEPM premium to take over the risk of unexpected high-cost gene therapy claims.

Category	Characteristics of the solutions
Payer reinsurance/ stop-loss/ risk carve out solutions	• Carve out of financial responsibility or risk for specified cell and/or gene therapies, or other specified high-cost therapies. • May or may not have financial attachment points; carve out is specific to the covered therapy and not total patient medical and drug costs • Do not offer performance guarantees, but assume all actuarial/financial risk
Contract negotiation and data management services for payers and pharmaceutical companies	• Act as third parties to negotiate contracts for cell and gene therapies • Provide data/outcomes tracking services • Contracts may include performance guarantees, but the service company themselves do not assume financial risk
Provider contract negotiation services	• Oriented towards providers and patient care pathways • Multiple services offered, including COE network creation and contracting, data analytics and cost containment • Performance guarantees are oriented towards clinical services and outcomes rather than financial guarantees
Financial and pharmaceutical company warranty services	• Include payment plans, with or without performance guarantees, and warranties for purchase by pharmaceutical companies that can provide protection to payers for suboptimal product performance

Table 1. Defining categories of products current available for managing the impact of cell and gene therapies

Evernorth's Embarc Benefit Protection Program brings together health services, medical management, and specialty pharmacy expertise in a risk carve-out designed to shield health plans and customers from the high cost of gene therapies and ensure patient access.

PayRx offers a product called Benefit Protection, which expands traditional pharmaceutical company payment assistance programs via a proprietary analytics model. The model determines

who would benefit most, and then assumes the risk, allowing stakeholders to collaborate and ensure patients have access to high-cost therapies.

Contract negotiation and data management services for payers and pharmaceutical companies

Two products were identified in this category: Audaire's Gene & Cell Therapy Outcomes Management Service and Real Endpoints' Marketplace. Both products include pharmaceutical companies as potential customers, as well as self-insured employers, commercial health plans, Medicaid and Medicare.

Audaire's Gene & Cell Therapy Outcomes Management Service offers a range of services, including: negotiation of performance-based contracts on a plan's behalf; automatic data capture of patients and providers (clinical and patient-reported outcomes); data aggregation; contract administration on behalf of the plan; patient tracking (via a patient registry) and patient portability (if a member leaves the plan). If a healthcare payer prefers to negotiate their own outcomes-based agreement (OBA) and manage their own contract with the pharmaceutical company, Audaire can provide the automated outcomes capture and portable patient registry services to ensure the payer receives the maximum value from their OBA.

Real Endpoints' Marketplace product is a forum for payers and pharmaceutical companies to efficiently develop and implement performance-based contracts. Real Endpoints has aggregated millions of lives at small and mid-sized payers. The payers receive access to innovative contracts across a collection of medical benefit drug products through a single contract with the RE Marketplace. Pharmaceutical companies in turn can reach millions of patient lives through negotiation of one contract with the Marketplace. Real Endpoints acts as the third party to analyze data, assess performance, and manage reconciliation and reporting.

Provider contract negotiation services

Two companies offer services in this category: Blue Cross Blue Shield Association and Emerging Therapy Solutions. These products launched the earliest overall, in 2018 and 2019, respectively.

Blue Cross Blue Shield Association offers the Blue Distinction Center for Cellular Immunotherapy program, which is designed specifically for CAR-T therapies. The program aims to provide optimal patient-centered care at designated COE, improving outcomes and costs for members undergoing CAR-T treatment. The program recognizes providers for their excellent expertise and efficiency in delivering complex care via a robust criteria evaluation. The Blue Distinction Center for Cellular Immunotherapy program is for self-insured employers and commercial health plans and is only available for current Blue Cross Blue Shield Association members.

Emerging Therapy Solutions offers three products (ETS Programs of Excellence, ETS Analytics and ETS Buyer's Group) that provide insights, data, and expert analysis that payers need to manage the high costs, potential risks and intricate processes associated with treating rare and complex medical conditions. This is achieved by proactive monitoring of the emerging therapies pipeline and engagement with physician experts to provide medical policy and underwriting support services. Cost containment and negotiation services are also offered. Emerging Therapy Solutions' customers include a wide range of stakeholders: self-insured employers, commercial health plans, Medicaid, Medicare, care management organizations, pharmaceutical companies, and stop-loss reinsurers.

Financial and pharmaceutical company warranty services

Three products were identified in this category, which targeted different customers. Two products included performance guarantees.

August Care Health offers an Outcomes-based Financial Solutions platform that provides the opportunity to move from high upfront costs to paying for achieved performance only, by offering cost spreading over multiple budget years and financial protection against treatment failure. The product is designed for self-insured employers, commercial health plans, Medicaid, Medicare, care management organizations, pharmaceutical companies, and pharmacy benefit managers.

CVS Health offers an installment payment plan for gene therapies that allows health plans to pay over several years, smoothing the impact of an immediate large, incurred cost. The product is for self-insured employers and commercial health plans who are utilizing CVS Caremark or CVS Specialty services and when CVS Specialty dispenses the gene therapy.

OutcomeRx's Specialty Therapy Warranty is an outcomes-based contract mechanism that serves as a risk sharing mechanism between payers and pharmaceutical companies by guaranteeing the efficacy/durability of cell and gene therapies. Warranties are sponsored by the pharmaceutical company (the only direct customer for the product) and administered by OutcomeRx, who manages all underwriting, claims, data, and payer communication.

ADDRESSING FINANCIAL CHALLENGES OF DURABLE/POTENTIALLY CURATIVE THERAPIES

Addressing the financial challenges of cell and gene therapies is a key aspect of FoCUS research. Results from research and modelling initiatives undertaken by the FoCUS project have identified multiple areas of risk and other challenges that would ideally be addressed by innovative contracts with pharmaceutical companies. Table 1 highlights the challenges that respondents reported are addressed by the products detailed in the survey. The challenges are grouped by type: risk, costs, administration, and those specific to patients or providers:

Risk

- **Actuarial/financial risk**: product addresses the uncertainty in the number of patients likely to be treated with a durable cell or gene therapy and the resulting financial risk associated with the cell or gene therapy.
- **Performance risk**: product addresses the uncertainty over effectiveness and durability of the cell or gene therapy, which may not be well-established at approval.
- **Payment timing risk**: product addresses the high upfront cost of treatment relative to benefit realization.

Cost

- **Product cost**: product covers the cost of cell or gene therapy alone.
- **Ancillary cost**: product covers additional costs of treatment with cell or gene therapy.

Administration

- **Data tracking**: product addresses need to track outcomes for patients receiving cell or gene therapy, solely or as related to a performance-based agreement.
- **Contract administration**: product assists with contracting between stakeholders, including initiation, management, and/or adjudication of contract.

Patients and providers

- **COE access**: product assists stakeholder with access to COE for administration of cell or gene therapy.
- **Patient access**: product addresses the barriers to patient access to cell and gene therapies.
- **Patient care**: product is focused on delivering optimal patient care throughout treatment with cell or gene therapy.

Table 2. Challenges addressed by current market solutions for managing the financial impact and reimbursement of cell and gene therapies

Company *Product*	RISK			COST		ADMINISTRATION		PATIENT/PROVIDER		
	Actuarial/ financial	**Performance**	**Payment timing**	**Product**	**Ancillary**	**Data tracking**	**Contracts**	**COE access**	**Patient access**	**Patient care**
Payer reinsurance/stop-loss/carve-out										
BCS Financial *Stop-Loss Gene Therapy*	●		●	●	●					
CVS Health *Gene Therapy Stop-Loss*	●			●	●				●	
Evernorth *Embarc Benefit Protection Program*	●	●	●	●	●	●	●	●	●	●
MedImpact Healthcare Systems *MedShield*	●			●					●	
OptumRx *Optum Gene Therapy Risk Protection*	●	●	●	●		●	●		●	●
OutcomeRx *Patient Access to Costly and Curative Therapies*	●		●	●		●			●	
PayRx *PayRx Benefit Protection*	●	●	●	●	●	●				
Contract negotiation & data management services for payers and pharma companies										
Audaire Health *Gene & Cell Therapy Outcomes Management Service*		●	●	●		●	●			

Company *Product*	RISK			COST		ADMINISTRATION		PATIENT/PROVIDER		
	Actuarial/ financial	Performance	Payment timing	Product	Ancillary	Data tracking	Contracts	COE access	Patient access	Patient care
Real Endpoints *RE Marketplace*	●	●		●		●	●		●	
Provider contract negotiation										
BlueCross Blue Shield Association *Blue Distinction Center for Cellular Immunotherapy*		●	●	●	●	●	●	●	●	●
Emerging Therapy Solutions *ETS Programs of Excellence, ETS Analytics & ETS Buyer's Group*	●		●	●	●	●	●	●		
Financial and pharma company warranty services										
August Care *Outcomes-based Financial Solutions*	●	●	●	●		●	●	●	●	●
CVS Health *Gene Therapy Payment Plan*			●	●					●	
OutcomeRx *Specialty Therapy Warranty*		●				●	●		●	

THERAPY COVERAGE

A summary of the durable cell and gene therapies covered by all products is presented in Table 3. Most solutions covered the approved gene therapies Luxturna (for inherited retinal disease) and Zolgensma (for spinal muscular atrophy). Coverage for approved cell therapies included all approved CAR-Ts: Abecma, Breyanzi, Kymriah, Tecartus and Yescarta.

ADDRESSING PATIENT MOBILITY AND DATA TRACKING FOR PRODUCT PERFORMANCE

Patient mobility

Patient mobility (i.e., patients moving from one payer to another) is a key challenge when considering innovative financing solutions for cell and gene therapies. Patients moving plans results in the first payer bearing the upfront one-time costs of gene therapy while a later payer potentially reaps the downstream benefits. For example, a payer may set up a payment plan for a gene therapy, which they remain responsible for even if a patient leaves their plan. In another example, a product failure that triggers a refund from a pharmaceutical company may be

Company *Product*	**Approved cell therapies**	**Approved gene therapies**	**Pipeline[a]**	**Other therapies[b]**
Audaire Health *Gene & Cell Therapy Outcomes Management Service*	●	●	●	●
August Care *Outcomes-based Financial Solutions*	●	●		
BCS Financial *Stop-loss Gene Therapy*		●	●	●
BlueCross Blue Shield Association *Blue Distinction Center for Cellular Immunotherapy*	●			
CVS Health *Gene Therapy Stop Loss*		●	●	
CVS Health *Gene Therapy Payment Plan*		●[c]	●	
Emerging Therapy Solutions *ETS Programs of Excellence, ETS Analytics & ETS Buyer's Group*	●	●		
MedImpact Healthcare Systems *MedShield*		●	●	●
OptumRx *Optum Gene Therapy Risk Protection*		●	●	●
OutcomeRx *Patient Access to Costly and Curative Therapies*		●		●
OutcomeRx *Specialty Therapy Warranty*	●	●		
PayRx *PayRx Benefit Protection*				●
Real Endpoints *RE Marketplace*	●	●	●	●

Table 3. Summary of coverage provided by current market solutions

[a]Pipeline products include those that are in late-stage trials and/or undergoing FDA review, but are not yet approved. [b]Other therapies include non-cell or gene therapies that are high cost. [c]Select gene therapies dispensed through CVS Specialty.
Evernorth responded as 'Confidential'

overlooked if the data are not reported back to the original payer after a patient leaves a plan. In the survey, respondents were asked how their product address patient mobility and whether the product is transferable. Responses varied considerably.

Three solutions described their approaches for handling patient mobility. Audaire Health follows all patients for the duration of an OBA, regardless of their current payer. In the event of a failure trigger, Audaire Health operates as an independent third party, reporting back to the original payer and capturing all documentation of the triggering failure event on behalf of the original payer. Real Endpoints responded that patients can be tracked between participating payers if both payers agree to tracking. OptumRx stated they will continue to track outcomes and collect outcomes-based reimbursements (as applicable) for members treated under a product's coverage that subsequently change plans, when provided access to the necessary outcomes data, such as patient and provider surveys and medical claims data.

Emerging Therapy Solutions agreements may cover patient mobility, but it is dependent on the products and the structure of the agreement with the risk taker.

Two solutions stated that patient mobility does not affect their product or benefits for their customers. With MedImpact Healthcare Systems' reinsurance, payers are responsible for a PMPM premium payment irrespective of any claims filed for covered therapies. Therefore, patient mobility risk is eliminated for the payer. Similarly, BCS Financial allows patients to change payers as long as the employer continues the stop loss coverage. August Care offers payer-agnostic outcomes tracking, which means patient mobility does not affect the agreement.

Data tracking

Most solutions have established methodologies or systems to track patient outcomes over time (Table 4). Reinsurance products from OutcomeRx (Patient Access to Costly and Curative Therapies) and MedImpact Healthcare Systems (Medshield) do not track patient outcomes.

PERFORMANCE GUARANTEES AND FINANCIAL CHARACTERISTICS

Five organizations included performance guarantees as part of their solution for cell and gene therapies: Blue Cross Blue Shield Association; Real Endpoints; August Care; PayRx; and OutcomeRx (Specialty Therapy Warranty). Although Audaire Health does not offer performance guarantees themselves, their service allows for implementation of performance-based contracts.

Details of the performance guarantees were generally not made available, although it was clarified by most respondents that their guarantees are customized by product and/or payer specification. Real Endpoints' performance guarantees are based on refunds that are paid from pharmaceutical companies to payers in the event of a product failure. Blue Cross Blue Shield Association's performance guarantee is oriented towards offering a high standard of specialized care across the treatment pathway for CAR–T therapies.

Specific requirements for adjudication of performance guarantees were not made available, except for noting that outcomes data must be made available for analysis.

Details of fees for most products identified were generally not available. Real Endpoints retains a set percentage of any value payments to payers. For OutcomeRx's specialty therapy warranty, it was noted that the premium paid for the warranty coverage is subject to Medicaid Best Price reporting. All respondents stated that customers have the right to audit transactions.

IMPACT ON PATIENTS AND PROVIDERS

None of the products require additional payment from the patient over and above their usual plan benefit cost share. However, both patients and providers may be required to agree to additional responsibilities upon treatment with a durable cell or gene therapy.

Audaire, for instance, requires patients and providers to continue follow-up assessments should they leave their original payer or plan. Similarly, OptumRx requires patients to comply with prior authorization activities and participate in outcomes tracking. The tracking will vary by therapy, but may include responding to surveys, providing current provider information, reporting outcomes, and providing consent for OptumRx to access medical records. The treating provider must also agree to same. OutcomeRx solutions require providers to supply additional data to payers to support coverage. These requirements for patients and providers to continue follow-up may result in additional administrative burden for both groups.

Benefits for patients and providers provided by current market solutions include improved patient access and treatment coordination, educational resources, and dedicated customer service.

Real Endpoints accelerates patient access to therapy because their contracting and prior authorization is aligned to drug labeling. Optum Rx provides patient navigators to help patients with treatment coordination for the complex therapies they include in their solution. For example, there is a comprehensive hemophilia program that would be available should gene therapy be approved for this condition.

BlueCross Blue Shield Association and Emerging Therapy Solutions report educating all stakeholders, including patients and providers, on the cell and gene therapies they cover. Emerging Therapy Solutions includes educational content regarding

Company *Product*	**How are outcomes tracked?**	**What is the source of data?**
Audaire Health *Gene & Cell Therapy Outcomes Management Service*	Proprietary, customizable, full automated, self-service system	Providers Data collected include product, disease state and agreed clinical endpoints as per OBAs
August Care *Outcomes-based Financial Solutions*	Decentralized infrastructure	De-identified patient data from integrated sources
BCS Financial *Stop-loss Gene Therapy*	Self-insured medical and pharmacy administrators	Self-insured medical and pharmacy administrators
BlueCross Blue Shield Association *Blue Distinction Center for Cellular Immunotherapy*	n/a	COE, providers Data collected are based on the Blue Distinction Center criteria and local plan requirements
CVS Health *Gene Therapy Stop Loss*	Standard product performance dashboards	n/a
CVS Health *Gene Therapy Payment Plan*	Standard product performance dashboards	n/a
Emerging Therapy Solutions *ETS Programs of Excellence, ETS Analytics & ETS Buyer's Group*	Proprietary software	Variable, tracking is based on customer needs
OptumRx *Optum Gene Therapy Risk Protection*	Process varies by condition and/or therapy OptumInsight has the capability to administer patient and provider surveys and access to medical claims across several payers that may be used to track outcomes	Varies by condition and/or therapy May include, but is not limited to patient surveys, provider surveys, medical claims, pharmacy claims
OutcomeRx *Specialty Therapy Warranty*	n/a	Payers or providers, depending on the warranty Data collected are based on agreed warranty structure
PayRx *PayRx Benefit Protection*	n/a	Payers Data collected are per payer requirements
Real Endpoints *RE Marketplace*	Periodic reports	Payer pharmacy and medical claims Data collected are used to reconcile contracts

Table 4. Summary of performance tracking methodologies used by current market solutions

COE, Centers of Excellence; n/a: information not provided or no response; OBA, outcomes-based agreement.
Evernorth responded as 'Confidential'.

approved conditions, therapies and the pipeline of potential treatments. OptumRx has customer service call centers to answer provider's questions about the prior authorization and appeals process. Outcome Rx provides educational information on the therapies and pipeline; however, the education is better suited to the payer than the provider and patient.

DISCUSSION

This paper summarizes the results of a FoCUS RFI survey of known organizations that offer products or services aimed at managing the challenges and financial impact of cell and gene therapies with potentially curative efficacy and high upfront costs. The purpose of the survey was not to compare or make

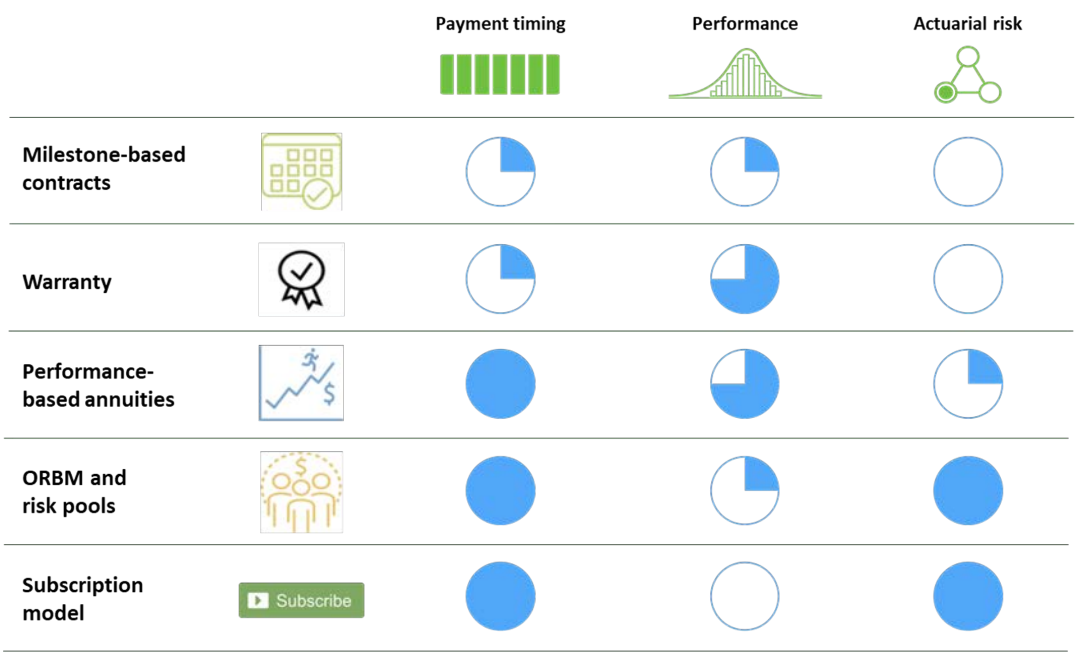

Figure 1. FoCUS precision financing solutions and their ability to address key challenges associated with cell and gene therapies

Blue circles represent the proportion of the associated challenge (payment timing, performance, actuarial risk) addressed by the precision financing solution. A full blue circle indicates the challenge is fully addressed; an empty circle indicates the solution has not addressed the challenge.

assessments of individual products, but to report the responses regarding the level of coverage and types of products available. In published literature, including research by FoCUS, multiple models of precision financial solutions for durable cell and gene therapies have been reviewed (1-3, 5, 6). To our knowledge, this is the first publication that offers details of the real-world healthcare marketplace for specific products and services that support reimbursement and coverage of durable cell and gene therapies.

Overall, the survey has shown a wide range of products are already available in the healthcare market that provide diverse approaches to addressing stakeholder needs. These market solutions are in various stages of maturity. Because the market is still evolving and relatively few cell and gene therapies have been approved to date, stakeholders may not yet be motivated to move away from individually negotiated arrangements. However, as more cell and gene therapies emerge over the next decade, these market solutions may become more popular, and a shift toward particular solutions may appear.

Currently, there is no single, system-changing product that is likely to address all stakeholder concerns and needs. In fact, to be fully covered for certain scenarios, stakeholders may need to use multiple products to manage their risk. This result is aligned with FoCUS precision financing solutions – no solution proposed by FoCUS addresses all three key challenges associated with durable cell and gene therapies (Figure 1).

FoCUS has previously conducted real-world evidence research with payers which indicated that different payer segments need and want different solutions, and that payers want pharmaceutical companies to propose solutions (7, 8). Our survey shows that the market is responding to these needs. While most solutions are offered in the reinsurance/stop-loss space, more complex solutions are also available. It remains to be seen if pharmaceutical companies will align with individual solutions for their products. In 2018, it was reported that Novartis was exploring working with global reinsurers to help health systems bear the cost of their cell and gene therapies (9).

The survey was administered to known organizations/products from within the group's experience. No systemized search methodology was used to identify products, and additional solutions may be available and not included in this survey. For most questions, respondents were allowed to respond as they wished, or not respond at all. This resulted in variability in the amount of detail provided.

CONCLUSIONS

The aim of this survey was to provide an overview of products currently available in the healthcare market that are designed to address the unique needs of stakeholders associated with the reimbursement of durable cell and gene therapies. Overall, the survey indicated a variety of products are already available to stakeholders, and these products provide diverse approaches to addressing stakeholder needs.

FoCUS anticipates providing a resource within the FoCUS Toolkit (https://payingforcures.mit.edu/toolkit/) that will summarize key considerations for stakeholders wishing to understand how to approach evaluating products in this arena. FoCUS is dedicated to education in this arena and will continue monitoring of the market for key trends.

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FoCUS wishes to thank all survey respondents.

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