ACTUARIAL SPECIALTIES | HEALTH

Future of Gene Therapy Funding

Health actuaries must innovate to create new funding mechanisms to ensure health plan viability and affordability

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New curative cell and gene therapies pose several challenges to U.S. health care payers. Treatments such as chimeric antigen receptor (CAR) T-cell therapies often have large upfront costs in exchange for improved health accruing over the patient's lifetime (assuming treatment durability). New gene therapies run counter to traditional payment models where costs and benefits are spread over time and allow for yearly assessment of plan coverage, premiums and member enrollment. Many existing financial models account for new costs through plan design and premium escalation to maintain plan viability. Extreme premium increases in anticipation of high-cost care can constrain health insurance coverage or cause new treatments to be unaffordable for the patients who need them.

Understanding the payer perspective on awareness of current and new funding mechanisms and their unique challenges to delivering high-cost treatments provides insight to future health care affordability. Payers providing their patients access to medical innovations is essential to the sustainability of research and development of new cures.

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Massachusetts Institute of Technology (MIT) delivered two case studies to better understand feedback on curative treatments from various payers under the NEW Drug Development Paradigms (NEWDIGS) project. As part of this project, the Financing and Reimbursement of Cures in the United States (FoCUS) conducted surveys to further understand the cost burden of these new durable and curative therapies on the U.S. health care system. Initial interviews with 15 U.S. payers—including Medicare, Medicaid, commercial plans, integrated delivery networks (IDNs), self-insured employers and reinsurance—were conducted between August and September 2017.[1] At the time of study, 925 novel gene therapies were in development with approximately 60 therapies to be introduced to the market over the next five years.

A follow-up outreach was performed through a 15-question online survey between September 2018 and April 2019 to update the results of the initial FoCUS project and expand the number of payers surveyed.[2] While more than 400 individuals were contacted to complete it, only 77 payers—representing 153 commercial fully insured,

Medicaid, Medicare Advantage and self-insured employer plans—filled out the online survey. Results may include plans represented by more than one person; the study did not control for this.

These case studies show while many payers are eager to embrace gene therapy financing, few have implemented revised payment mechanisms. The case studies highlight these major payer concerns about curatives therapies:[3]

- **Actuarial risk.** Uncertainty of being impacted by patients with rare diseases requiring high-cost treatment is top of mind due to incidence volatility from frequently changing populations.
- **Durability.** Payers are wary of the true patient health benefit of curative treatments due to high upfront costs for savings realized over the patient's lifetime. Many new treatments have been approved based on study durations of 18 months or less.
- **Beneficiary turnover.** Patients frequently churn in and out of health plans due to simple life events like changing employers. Payers are worried patients will move onto a different plan after treatment payment before any health benefits are realized.
- **Adverse selection.** Payer coverage inconsistency encourages patient anti-selection. Payers fear patients specifically enrolling in or seeking out plans that cover gene therapy.
- **Buy and bill.** Many providers include mark-ups on medication, ranging from 25 percent to 200 percent of the original cost. Payers are concerned mark-ups are unsustainable considering the already high price tag and may need to partner with the manufacturers directly. Revenue disruptions are expected for providers.
- **Regulation.** Future regulations could potentially result in barriers to implementing revised payment models. State regulations may mandate adoption of new reimbursement models specific to gene therapy, possibly hindering implementation of any individual funding mechanism.
- **Administration.** Administration of robust funding mechanisms is burdensome for many payers as development of new systems, such as patient tracking programs, is required.

By holistically understanding payer perspectives and their unique challenges with these new curative therapies, actuaries can effectively innovate and develop new solutions to ensure patient access to these treatments while maintaining plan affordability.

INITIAL RESULTS: AUGUST–SEPTEMBER 2017[4]

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The initial NEWDIGS project involved reaching out to 40 participants from various payer segments, of which 21 interviewees from 15 payers completed the interview. Interviewees were provided with interview background and objectives including two case studies (CAR-T cell therapy and hemophilia A) and four proposed financing mechanisms (annuity, performance-based annuity, risk-pooling and no change to current). The interview results provided insight on:

- Current payer awareness
- Preference for financing and reimbursement mechanisms
- Impact by payer segment

Current Payer Awareness

Payers were asked about their knowledge of the gene therapy pipeline and the potential financial burden proposed by these high-cost drugs. Payers showed varying levels of awareness with 40 percent, 33 percent and 27 percent identifying themselves as "watchful and waiting," "early awareness" and "active management," respectively.

Payers that actively monitored the drug pipeline reviewed treatments to be approved in the next 12 to 18 months with a particular focus on high-cost treatments like gene therapy. The monitoring efforts were typically led by their financial or actuarial staff. Self-insured employers reported reliance on their pharmacy benefits manager (PBM) or medical administrator for drug pipeline information. Due to third-party reliance, self-insured employers were the least informed on anticipated new therapies and their impact.

Payers indicated the decision to cover particular gene therapies is often based on existing medical and pharmacy policy and relies on clinical, safety and economic information. Payers also relied on external review processes and internal opinion leaders to provide insight on therapy coverage.

Actuaries can effectively innovate and develop new solutions to ensure patient access to high-cost treatments while maintaining plan affordability.

New therapies expose affordability and sustainability concerns for the existing funding mechanisms. While payers are not concerned with the immediate future, they are concerned with the cumulative cost burden of multiple gene therapies to be approved over the next three to five years. Existing tools to maintain health plan viability may not be suitable to handle the new high-cost drugs. Currently, new costs are anticipated in plan design and premiums. Extreme premium escalations ultimately restrict coverage and access to the new therapies and overall health insurance coverage.

Payers showed varying opinions on near-term funding mechanisms with 53 percent, 27 percent and 20 percent preferring current tools, performance-based annuities and risk pooling, respectively.

Impact by Payer Segment

Payer segments showed differing concerns, current and future risk mitigation strategies, and barriers. All payers expressed concern about treatment durability, or the ability of the drug to postpone or delay disease progression in a safe and well-tolerated manner.

Commercial payers are defined as organizations at financial risk for health insurance policies not offered by the government. Their key characteristics and preferences include:

- Large commercial payers have substantial cash reserves that can currently cover year-to-year volatility and report satisfaction with the existing reimbursement tools. Large commercial payers have large risk pools with sophisticated tracking systems to review the long-term impact of these therapies.
- Small commercial payers are compelled to provide gene therapy coverage to maintain a competitive edge in the market, though they are concerned with adverse selection that could lead to cash-flow risk. Further, small commercial payers report a large portion of their insured lives are subject to state coverage requirements, which reduces their ability to control access and manage utilization.
- All commercial payers report a preference for the development of gene therapy centers of excellence (COEs) to improve quality and ensure treatments are performed in approved facilities to optimize outcomes.
- Commercial payers prefer to focus on direct contracting with drug manufacturers to reduce mark-ups and arranging pay-for-performance agreements with treatment outcomes tied to financial guarantees.
- Payer major concerns are cash flow, beneficiary turnover and adverse selection.

Self-insured employers are defined as employers that are financially at risk for their employee's health care. Their key characteristics and preferences include:

- Self-insured employers heavily rely on stop-loss insurance to protect against unexpected claims and use historical claims experience to predict future claims.
- Employer's ability to easily switch between stop-loss carriers allows for premium increase suppressions year-over-year.
- When stop-loss carriers require an extreme premium increase, employers opted to either increase the exposure threshold to lower premiums or reduce nonmedical employee benefits to subsidize the cost increase.
- Employers preferred expanded risk pools provided by stop-loss and reinsurance vendors to manage their financial risk.
- Major payer concerns are providing affordable access and reliance on plan administrators for high-cost drug management.

The **IDNs** interviewed were small (with less than 1 million lives) and offered multiple lines of business. They shared that:

- Similar to small commercial payers, small IDNs are compelled to cover gene therapy due to pressure to maintain a competitive edge and pressures from providers to adopt new innovations.
- IDNs express an extreme concern of adverse selection with patients enrolling only for new therapy coverage.
- IDNs report a preference for expanded risk pools, followed by performance-based annuities.

Managed Medicaid plans highlighted these areas of concern, risk mitigation strategies and potential barriers:

- A major concern for managed Medicaid is the regulatory restrictions with escalating drug costs since the state—rather than the plan—dictates premiums. Annual premiums that do not account for new drug treatment approvals during the year can lead to financial loss. Some states require that all oncology treatments approved by the U.S. Food and Drug Administration (FDA) be covered.
- A current method to handle high-cost drugs is through carve-outs, which occur when payers exclude specific services from the plan. Despite the use of carve-outs, the liability is difficult to anticipate since drug exclusions are addressed on a case-by-case basis. Other methods include states withholding a portion of premiums to reimburse plans with higher-cost patients.

- Another major payer concern is beneficiary turnover since managed Medicaid has high patient churn. Managed Medicaid is unlikely to realize the benefits of any medical costs offset by the durable therapies.
- Managed Medicaid plans prefer expanded risk pools, carve-out or plan consolidation.

The key characteristics and preferences for **Medicare** plans include:

- Similar to Medicaid, Medicare is subject to mandatory drug coverage requirements, such as oncology drugs.
- Bid submissions for Medicare plans, including formulary, utilization, premiums and plan design, have a long lead time (seven to eight months), which makes setting accurate premiums difficult. Underestimating premiums can lead to financial loss, while overestimating premiums results in reduced competitiveness.
- Catastrophic coverage for Medicare Part D plans (PDP) is provided by the government, which acts like a reinsurer. PDPs are interested in short-term performance-based agreements.
- Medicare Advantage is at risk for both high medical and pharmacy costs due to no catastrophic coverage from the government. These plans must obtain reinsurance and prefer expanded risk pools to mediate reinsurance premiums.

As far as **stop-loss and reinsurance**:

- Many payers report relying on stop-loss and reinsurance to mitigate claims volatility.
- As experience drives the rates, new curative therapies can lead to higher reinsurance and stop-loss premiums.
- Future strategies to maintain costs include expanded risk-pooling or carved-out risk pool for gene therapy.

UPDATED RESULTS: SEPTEMBER 2018–APRIL 2019[5]

Following the first FoCUS survey in 2017, a secondary 15-question online survey was performed between September 2018 and April 2019. The respondents of this second survey were 77 payers of varying sizes from less than 5,000 to 50 million insureds. Payers represented 153 plans, including commercial fully insured plans, self-insured employers, Medicaid and Medicare Advantage. Most respondents were in a pharmacy-related role (53 percent); other roles represented are medical (31 percent), human resources and benefits (9 percent) and finance and actuarial (7 percent). The three key findings of the results were:

1. Payers are concerned about financial risk and the impact of high-cost durable therapies.

2. Payers are motivated to manage the financial risk associated with high-cost durable one-time treatments differently, making this area a high priority over the next two years.

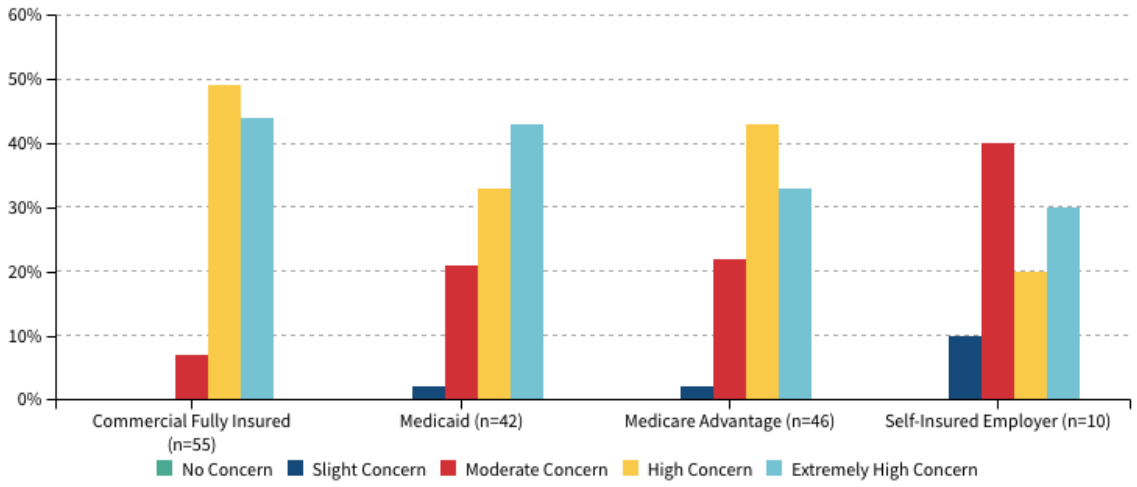
3. Addressing contract terms and barriers will matter.

Payers Concerned About High-Cost Durable Drugs

Since the 2017 survey, payers showed increased concerns about the financial impact of high-cost durable drugs with 80 percent of payers rating their concern as high or extremely high.

Commercial fully insured plans expressed the highest level of concern with 93 percent of those in the payer segment citing high or extremely high concern. In contrast, of the self-insured payer segment evaluated, half expressed slight to moderate concern while the remaining half expressed high to extremely high concern (see Figure 1).

Figure 1: Payer Concern With Financing High-Cost Durable Drugs



Commercial Fully Insured(n=55)Medicaid (n=42)Medicare Advantage (n=46)Self-Insured Employer (n=10)0%10%20%30%40%50%60%No ConcernSlight ConcernModerate ConcernHigh ConcernExtremely High Concern

Source: FoCUS Payer Perspectives: MIT NEWDIGS White Paper: Survey Results: Payer Perspectives on Financing and Reimbursement of One-time High-cost Durable Treatments. *NEWDIGS FoCUS*, October 11, 2019.

Common reasons for high or extremely high concern include:

- The total cost is material for the plan
- Product performance risk (effectiveness and durability)
- Actuarial risk (likelihood of encountering a rare case)
- Payment timing relative to benefit realization (health benefit does not offset high-cost treatment)

Other reasons for high concern include possibility of having the burden of multiple high-cost therapies, impact of these costs over three to five years, potential off-label use, operational management of the larger pipeline and adverse selection.

Most payers currently use stop-loss or reinsurance arrangements to handle high-cost treatments.

Most payers (70 percent of the 76 surveyed) currently use stop-loss or reinsurance arrangements to handle these high-cost treatments. At the time of the survey, the majority of payers (99 percent) cover one-time high-cost durable treatments—46 percent covered the entire treatment while 53 percent covered some of it. For the payers covering the entire treatment, 48 percent cover high-cost therapies as specified on the FDA-approved label, 49 percent cover high-cost therapies as specified on the FDA-approved label with utilization management (additional restrictions), and 3 percent cover with fewer restrictions than what's on the label.

Commercial fully insured payers were most restrictive with these treatments with 62 percent of these payers requiring coverage management stricter than the FDA-approved label. Medicare Advantage plans were the least restrictive with 67 percent of plans managing these drugs consistent with the FDA label. During treatment, case management is used by 82 percent of payers, and 64 percent of payers require COEs.

High-Cost Treatment Financial Management Priority in the Near Term

Payers are interested in a solution specific to one-time high-cost durable treatments. Payers did not show similar interest or concern for high-cost chronic treatments, such as SPINRAZA (nusinersen) or OPATTRO (patisiran).

A majority of payers (57 percent of 77) expect to implement new cost management strategies in the next one to two years, while 13 percent of payers already have. Examples of new strategies are typically outcome- or value-based agreements. Another example of a new cost management technique is changes to provider contracting where the treatment cost is absorbed by a third party or the treatment is subject to a global case rate.

Generally, payer segments prefer short-term solutions with measurable milestones where the treatment is paid for upfront and performance is assessed over a period of two years or less (see Figure 2). Compared to other payers, self-insured employers showed a preference for population risk-pooling and stop-loss/reinsurance.

Figure 2: Preferred High-Cost Drugs Financing Solutions

Approach	Commercial Fully Insured (n=55)	Medicaid (n=42)	Medicare Advantage (n=46)	Self-Insured Employer (n=10)	All (n=153)
Population risk-pooling	69%	40%	52%	50%	55%
-term MBC	75%	50%	61%	20%	60%
Long-term MBC	58%	36%	43%	30%	46%
Annuity: Payment spread >2 years	33%	14%	24%	20%	24%
Performance-based annuity: Payment spread >2 years, tied to performance	47%	21%	35%	30%	35%

Note: MBC is a milestone-based contract—pay for therapy upfront and refunds tied to performance over the short term (<2 years) or long term (>=2 years).

Source: FoCUS Payer Perspectives: MIT NEWDIGS White Paper: Survey Results: Payer Perspectives on Financing and Reimbursement of One-time High-cost Durable Treatments. *NEWDIGS FoCUS*, October 11, 2019.

Contract Terms and Barriers

Payers agree that alternative financing mechanisms have several benefits, such as reducing the upfront burden of the treatment by smoothing the cost over time, aligning therapy costs with benefits and only paying for treatments that work by including performance-based components.

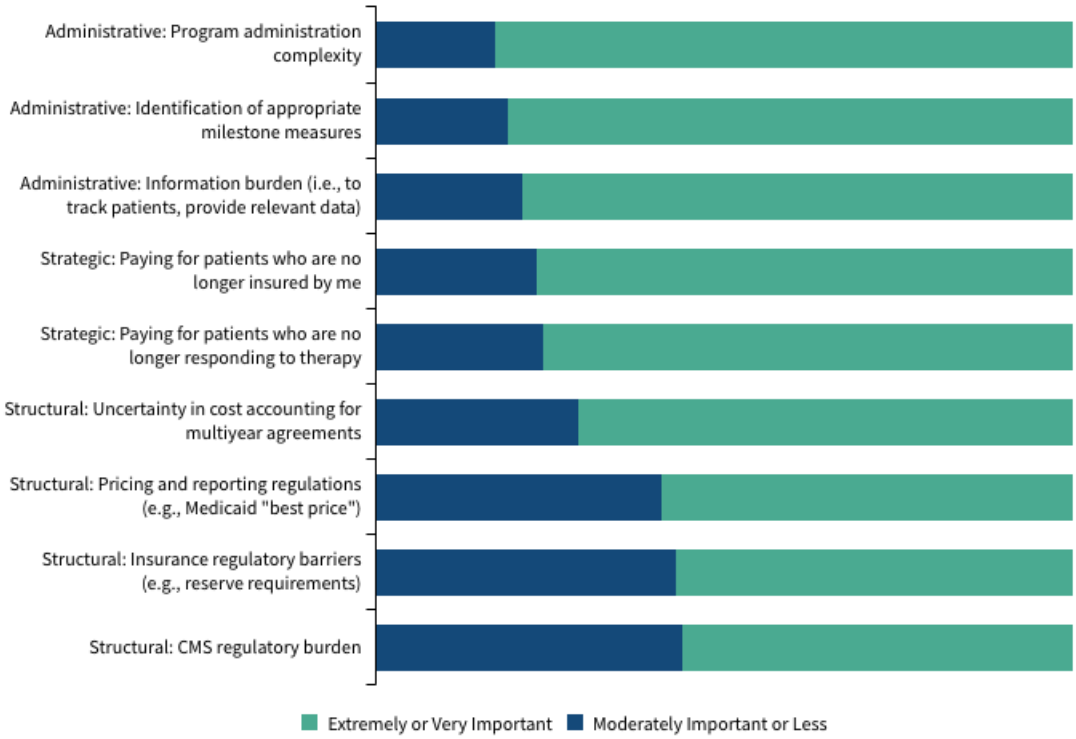
Paying only for treatments that work was ranked as the most beneficial outcome of using an alternative funding arrangement with 83 percent of the 77 payers rating this aspect as very or extremely beneficial. A majority of payers highlighted several necessary—and some nonnegotiable—components to a multiyear performance-based agreement, such as:

- Inclusion of performance-based requirements for payments
- Termination of payment obligation upon death of patient
- Access to data on specific measures
- Definition of term and the number of years over which the payouts are stretched
- Ability to track patient performance after patient leaves the plan

Patient longevity within plans varies by payer, ranging from less than two years for 38 percent of Medicaid members to more than five years for 70 percent of self-insured employers. Contracts with longer terms will need to address patients leaving plans prior to the contract expiration or include the ability to track patients among different plans.

While alternative financing methods may be preferred and have satisfied several concerns around high-cost durable therapies, payers also have cited barriers to implementation. Among the most concerning barriers is that of administrative complexity. Other top barriers are strategic and structural in nature (see Figure 3).

Figure 3: Payer Barriers to Alternative Financing by Importance (n=77)



Administrative: Program administrationcomplexityAdministrative: Identification of appropriatemilestone measuresAdministrative: Information burden (i.e., totrack patients, provide relevant data)Strategic: Paying for patients who are nolonger insured by meStrategic: Paying for patients who are nolonger responding to therapyStructural: Uncertainty in cost accounting formultiyear agreementsStructural: Pricing and reporting regulations(e.g., Medicaid "best price")Structural: Insurance regulatory barriers(e.g., reserve requirements)Structural: CMS regulatory burdenExtremely or Very ImportantModerately Important or Less

Source: FoCUS Payer Perspectives: MIT NEWDIGS White Paper: Survey Results: Payer Perspectives on Financing and Reimbursement of One-time High-cost Durable Treatments. *NEWDIGS FoCUS*, October 11, 2019.

OBSERVATIONS

Payers are interested in alternative financing mechanisms and prefer short-term solutions with measurable milestones with a two-year term or less.

Since the 2017 survey, concerns for these high-cost durable treatments increased, likely due to heightened general awareness and additional drugs approved in the pipeline. Commercial payers show the highest level of concern, while self-insured employers show the least amount. The reason for this lack of concern from self-

insured employers is unclear—it could be driven by the absence of knowledge or strong confidence in existing solutions, such as stop-loss and reinsurance.

The study shows all payers are interested in alternative financing mechanisms and prefer short-term solutions with measurable milestones with a two-year term or less. While all payers observe the benefits of new solutions, such as paying only for working treatments, administrative barriers—such as patient tracking, program maintenance and milestone determination—prove to be burdensome. Despite administrative, strategic and structural barriers, most payers identify with having high concerns for these expensive durable drugs and plan to implement new cost strategies over the next couple of years.

THE ACTUARIAL PERSPECTIVE

Both case studies highlight a strong need for alternate funding mechanisms for gene therapies in the near future with keen interest from payers looking to implement new cost management strategies in the next one to two years. Potential solutions have been proposed or analyzed by various health care industry stakeholders, such as PBMs, academic centers like MIT and professional organizations like the Society of Actuaries (SOA).[6] Examples of proposed solutions (some may be implemented already) include:

- **Pooling or carve-outs.**[7] Participants provide consistent payments to an entity to spread the cost of treatment across an entire group to reduce financial burden. Some examples include gene therapy specific pool, industry pooling, state-level pooling and plan-level pooling. Pools can also be voluntary or nonvoluntary, for profit or nonprofit, or small employers or large employers. Some third-party providers currently offer risk pools specific to gene therapy.
- **Warranty model.**[8] The warranty model operates similarly to a manufacturer car warranty where core benefits of the therapy are guaranteed to the payer over a specified time period. The warranty is a specified efficacy or durability agreement between the manufacturer and payer outlining which health care costs should no longer be incurred by a patient on the curative therapy. In the event the patient incurs a health care cost covered by the warranty, the payer is reimbursed for these costs. Some third-party providers currently offer warranty models. In CMS' latest final rule on value-based payments, specific warranty structures are supported and have amended Medicaid "best price" reporting requirements that support more comprehensive risk-sharing among manufacturers and payers.[9]
- **Multiyear insurance**.[10] This is an insurance duration exceeding the typical one year of coverage to spread the treatment cost over years.
- **Annuity payments.**[11] High-cost treatment is converted into multiple payments spread over a specified term, which can depend on a specific clinical result duration. The annuity can be executed by the drug manufacturer or a third party (such as a PBM). Additional layers of risk-sharing also can be implemented by including a drug efficiency guarantee. For example, annuity payments are only continued when specified levels of clinical results are achieved.
- **Health currency.**[12] Health currency funding arrangements help with beneficiary turnover barriers. Health currency is established when the therapy is administered. When the patient changes insurers or payers, the initial payer receives a portion of the expected savings to be realized by the benefit treatment from the subsequent payer. The claw-back period depends on the expected therapy value at the time the patient is transferred to the new insurer. Payment responsibilities to the initial payer are transferred each time the patient migrates to a new insurer.
- **Financial bonds.**[13] Payment of the treatment from the payer is to occur at the end of the treatment instead of the beginning to address risks of durability. The payer provides interest payments for the treatment until a predetermined future date. The treatment amount is paid in full at the time of bond expiry. Contract language will need to address special events like patient death or turnover. Additional layers of risk-sharing can be implemented into financial bonds based on the clinical outcome of the treatment. For example, if the patient does not respond to the treatment, then the entire bond is forfeited or would only be partially paid. The manufacturer can be paid upfront with the involvement of a third-party administrator that coordinates the interest payments until bond expiration. Financial bonds can propose challenges for manufacturers in meeting Medicaid best price requirements.
- **Value-based contracting.**[14] A portion of the treatment cost is refunded to the payer if the patient does not demonstrate clinical improvement or response. Payers and manufacturers are committed to tracking the patient over a long duration. Contractual language will be required to address when the patient transfers to a new payer or dies.
- **Development of COEs for gene therapy.**[15] Many insurers have excellence programs to help patients navigate complex diseases. Excellence programs allow patients to receive treatments at leading facilities with favorable reported clinical experience, quality of outcomes and economic factors. These facilities must meet rigorous

benchmark quality factors and certifications. While publicly reported gene therapy outcomes are limited, development of excellence programs for gene therapy can help direct patients to facilities with a history of effective outcomes, improving the likelihood treatment success.

- **Administration of funding mechanisms through an invested third party.**[16] The introduction of third-party entities, such as PBMs, can facilitate new and innovative financial payment models to reduce administrative burden and provide expertise. For example, self-insured payers are unlikely to have extensive knowledge of new therapies to effectively negotiate contracts with a drug manufacturer.
  While some barriers, such as actuarial risk and adverse selection, cannot be mitigated with alternate funding mechanisms, Figure 4 shows how these funding techniques can help address payer curative therapy concerns.

Figure 4: Payer Barriers and Funding Mechanisms

	Easy to Administer/ Implement	Addresses Durability Concerns	Addresses Patient Turnover Concerns	Simplistic Future Cost Accounting	Regulation and Reporting Challenges	Prevents Buy and Bill	Leverages Existing Mechanisms
Pooling							
Warranty							
Multiyear Insurance							
Annuity							
Annuity Payments With Guarantees							
Health Currency							
Financial Bonds							
Milestone/Value-based Contracting							
Develop Centers of Excellence							
Third-party Administrator							

In MIT's initial study in 2017, some payers relied on financial and actuarial staff to monitor the incoming pipeline of new curatives therapies. As more gene therapies are approved, health actuaries will need to be prepared to innovate new alternative funding mechanisms to ensure health plan viability and affordability. We will need to consider the cost equity of these new therapies, either through relying on third parties, such as reinsurers, or managing the new costs through revised pricing models.

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