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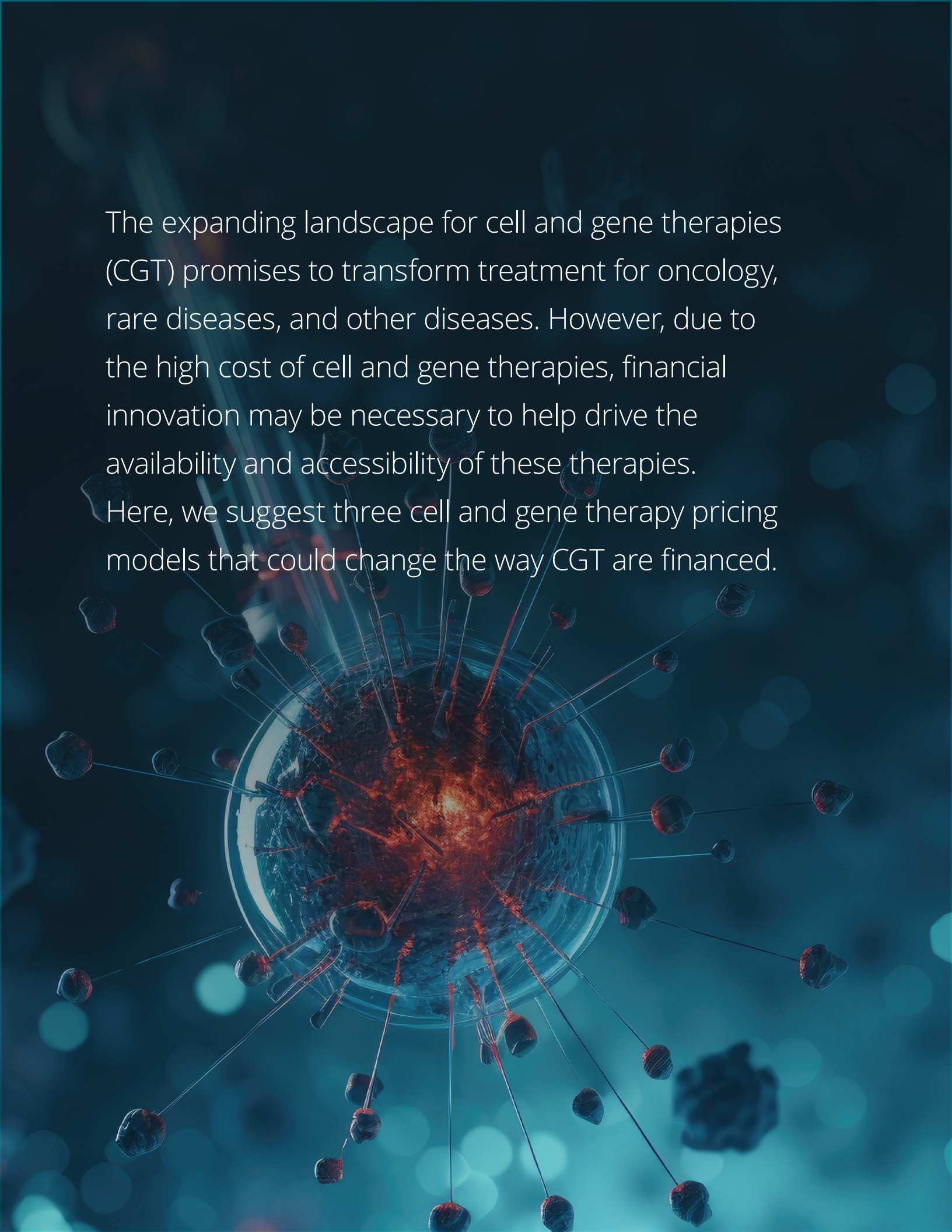
Transformative therapies require transformative financing models

The expanding landscape for cell and gene therapies (CGT) promises to transform treatment for oncology, rare diseases, and other diseases. However, due to the high cost of cell and gene therapies, financial innovation may be necessary to help drive the availability and accessibility of these therapies. Here, we suggest three cell and gene therapy pricing models that could change the way CGT are financed.



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The rise and impact of transformative treatments

The Cell and Gene Therapy (CGT) landscape continues to change at an unprecedented pace: 18 CGTs are in the US market today and an estimated 30 to 40 manufacturers have CGTs in their near-term pipelines.[1] Seven of the top 10 pharmaceutical manufacturers have CGTs in the market or in development.[2] The FDA has made decisions on several CGTs in 2023 and is expected to approve anywhere from 30 to 60 CGTs by 2028.[3] Some estimates suggest that more than 100,000 patients will be treated with CGTs by 2030, with an estimated annual spend of $20 billion in the United States.[4] CGTs are unique when compared to traditional therapies—they are potentially curative, will likely only require a one-time administration, and typically come with a high upfront cost. While there are differences in costs, administration, and coverage for gene versus cell therapies, the personalized nature of CGTs, complex manufacturing processes, and limited longitudinal data have contributed to access challenges for patients.

Most of the CGTs in the market today are to treat oncology or rare diseases. However, treatments are beginning to come to market for more prevalent diseases such as sickle cell anemia, beta thalassemia, and hemophilia.[5] As more treatments come to market, move to earlier lines of therapy, and target more prevalent diseases, the eligible patient population will meaningfully increase. Health plan sponsors (e.g., health plans, employers, government) that opt to offer coverage—are at risk of claims that can be sudden, unexpected, and sizable. These claims, or the potential for these claims, will likely drive increases in per member per month (PMPM) coverage costs. The decision to cover CGTs will be balanced with other operational budgetary considerations, including salaries, bonuses, and investments in the business. The *2023 Deloitte State of the Cell and Gene Therapy Industry* survey revealed that "payer and reimbursement challenges" was the top force respondents expected to significantly impact commercialization of CGTs in the next 12 to 18 months.

Legacy health care financing models are insufficient

The current one-time administration or single-dose price tag for a CGT treatment is significantly higher than traditional, typically chronic treatments that "amortize" the cost of care over months or years. This creates a single, high dollar, or "lightning strike," claim that makes legacy financing models inadequate as they are built on the expectation of chronic care and more moderate chronic payments. While the cost of CGTs has gotten press given the price point, ICER, when reviewing the recent sickle cell disease products from Vertex and bluebird bio, found "that current evidence is adequate to demonstrate a net health benefit for exa-cel (and lovo-cel) when compared to standard of care."[6]

These products demonstrate a health benefit and value case, but they still create a challenge to the legacy health care financing models. Both the burden associated with data collection and unclear regulatory guidelines were among the top three most significant barriers for effective real-world evidence collection according to respondents of the *Deloitte State of the Cell and Gene Therapy Industry* survey. While all health plan sponsors (commercial and government) face exposure, small and midsize self-insured employers in the United States—representing approximately 40 million beneficiary lives—are especially vulnerable to the overall rising cost of care and, in particular, "lightning strike" claims. The large and unpredictable budget impact associated with these therapies may lead these employers to reconsider their benefit designs (e.g., Will I be able to make payroll if I have to pay a CGT claim?). Today, financial friction areas for financers—those funding coverage or treatment costs—include the size of the risk pool, the fluidity of people entering and leaving the risk pool, and increasing overall budget exposure as the eligible population increases and more treatments come to market.

Patients who have coverage through existing benefits will likely feel the added burden of higher premiums and rising out-of-pocket costs as additional CGTs come to market. However, the share of patients who do not have access to these transformative therapies is likely to increase given the budget challenges for health plan sponsors. Without meaningful financing innovation, many plans and employers may carve out coverage for CGTs, leaving patients without access to these transformative and life-changing, or life-saving, therapies.

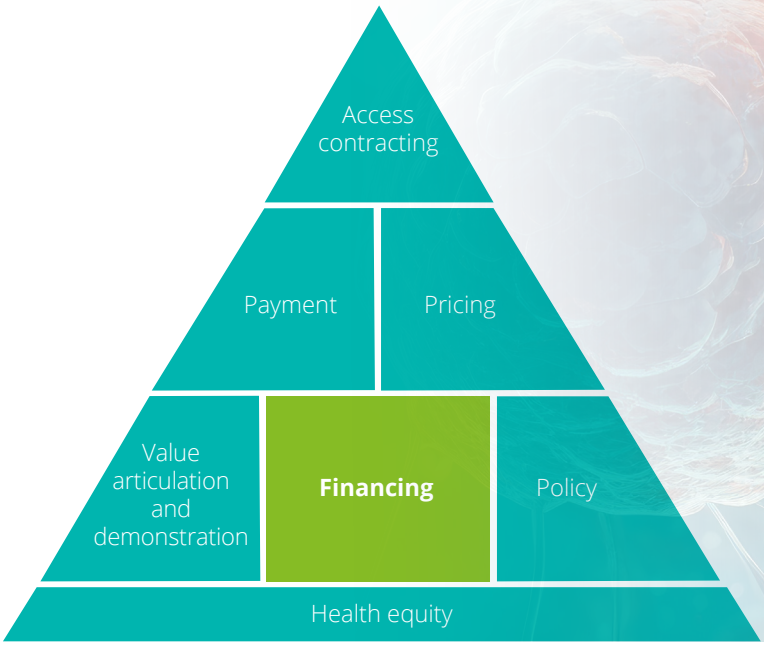
Today, limited in-market programs offer coverage solutions for health plan sponsors (see below for more information on current offerings). Moreover, patients face financing challenges that vary by type of coverage (including a disproportionate impact on patients already disadvantaged in today's health care system). Patient financing frictions include out-of-pocket (OOP) costs—both immediate OOP (e.g., deductibles and co-insurance) and total OOP (e.g., co-insurance, premiums). While ICER cites that gene therapies for sickle cell disease will meaningfully address society's goal of reducing health inequities,[7] it cannot be possible without innovation in financing models. Without novel models, the growth of CGTs in the market and the associated financing frictions will likely exacerbate existing health equity and access challenges already in the system.

Financing innovation is at the core of driving access and equity

We developed a conceptual framework (figure 1) to explain the relationship between foundational access requirements—health equity, value articulation and demonstration, financing, and policy—in driving the availability of and access to CGTs. In this model, if even one "block" is compromised, then patients will not be able to access CGT therapies. While each of these areas are critical, much work has been published on the need for health equity solutions,[8] as well as the impact of value articulation and demonstration, and policy[9] to address access.

While health equity concerns underpin access for all therapies, CGT financing challenges must be addressed, or there could be potential implications to the other building blocks of the market access pyramid. In this article, we address financing for CGTs as a focus for action while recognizing the importance of other elements in driving access and availability of CGTs.

Figure 1. CGT access framework



Current approaches may not be enough

Traditional approaches, such as stop-loss, are unlikely to be sustainable financing solutions long term, especially for small and midsize employers, given the average cost of employer stop-loss coverage for companies of this size is about equivalent to the cost of one gene therapy. Stop-loss costs are likely to increase if they become a common mechanism to finance CGTs. As a result, one approach being considered by stop-loss insurers is to carve out the cost of certain individuals (called "lasering")[10] or reduce coverage to mitigate the financial risk (especially as the number of CGTs in the market increases).

Health plans have also launched CGT financing programs.[11] Most of these programs focus solely on gene therapy coverage, with only a handful also covering CAR-Ts. Many of these programs provide limited coverage, such as discounts for using approved treatment centers. Furthermore, they do not directly address the added burdens on patients and caregivers, such as travel costs or time away from work to be near a treatment center.

Therefore, as more CGTs come to market—and for more prevalent diseases—the likelihood an employer will have an employee or dependent who is eligible for and requires a CGT will increase. In 2029, for example, an employer with 7,500 members will incur a roughly 25% chance of having at least one gene therapy claim that year.[12] The coverage gaps that persist will need to be addressed to drive access, uptake, and equitable care. Innovative financing will play a pivotal role in driving equitable and timely access to cutting-edge treatments for patients.

Transforming financing solutions to meet patient needs for CGT treatments

Recognizing there is no one-size-fits-all solution that can meet the various aspects of the financing challenges and types of coverage that exist, addressing the complexities of CGT financing necessitates a tailored and multifaceted approach. Patients and health plan sponsors need innovative and financially sustainable solutions that anticipate the financial exposure and help mitigate CGT financing frictions. Opportunities to create innovative financing models lie both within and outside of the current health care value chain. These opportunities can include supplementing existing offerings or creating new offerings.

Exploring potential solutions requires mitigating the current market frictions and creatively considering novel roles and opportunities for existing and, potentially, new stakeholders. Bringing solutions to market will require a shift from the traditional health care paradigm. There will be a need for cross-industry collaboration, there may be a need for government or regulatory support, and there might be opportunities for nontraditional players.

Putting aside the idea of potential government intervention in this space, we have explored three innovative models that we believe could fundamentally change the way CGTs are financed (figure 2).

Figure 2. Three novel CGT financing solutions

	1	2	3
Model	**Supplemental risk pool**	**New insurance product**	**Theraputic health credit**
Description	Sponsors (e.g., self-insured employers) contribute funds (e.g., PMPM) to a nationally available risk pool which is used to cover the cost of CGT treatments for patients	Generate new financing opportunities through new insurance policies or offering riders on existing insurance plans	Develop financial instruments for CGTs which can be traded in the market with some guarantee to protect investments
Value prop	Create a large risk pool in which risks and costs are distributed	New insurance offering to protect against high costs	Introduce new players to share in risk and repayment
Target audience	Sponsors	Parents, group insurance companies, and employers	Self-insured employers, financial services Institutions

1. Supplemental risk pool

Existing risk pooling solutions cover only some therapies (typically gene therapies) and are available only to members of the health plan provider that offers the risk pooling solution. Based on our work, some see the PMPM cost of these models as too high to be viable long term, especially as the number of therapies on the market increases and the cost to access these risk pooling solutions increases accordingly.[13]

We propose the creation of a supplemental insurance risk pool for commercially covered patients managed by a public benefit corporation, not-for-profit, or other neutral third party. This could reduce the profit margin expectations of the managing entity, align incentives, and reduce the overhead costs of providing this service while increasing the size of the risk pool. In this model, a sponsor would carve out CGT coverage for all its members and instead contribute a PMPM fee to the third party. This fee would be lower than existing risk pooling solutions in the market as it would be covered by an entity with aligned incentives, open across health plan provider networks. In the event of a patient needing a cell or gene therapy, the diagnostic test results are provided to the third party, confirming the patients' eligibility, and the necessary funds to cover the cost of the treatment are released. Depending on the design of the solution, additional funds could be released to cover patient out-of-pocket costs related to the diagnostic test, travel, logistics, etc. It could also improve the prior authorization process and how employers determine if coverage should be provided.

2. New insurance product model

Individual or group coverage for CGTs could resemble existing group life insurance products. These would likely be provided by employers to prevent selection bias, but individual market solutions could also be created. Specific examples include accelerated benefit products, supplemental insurance products, or an extended/optional warranty model for patients.

- In the accelerated benefit product, a patient would receive a premature payout upon CGT diagnosis to cover the cost of treatment.
- The supplemental insurance product could be a rider on a policy, available at an additional premium, to cover the patient out-of-pocket costs as well as indirect costs (e.g., cost of caring for a family member, loss of capital and economic productivity) of receiving treatment.
- The extended/optional warranty concept could be purchased at the time of care to ensure repayment or a cash outlay to the patient in the event the therapy does not work as intended (duration or effectiveness). Note: This would differ from other warranty models in the marketplace today as it would be purchased at the time of care as an option.

This model would primarily benefit patients and their families. While likely not enough to cover the full cost of treatment (including the therapy), this would provide an added benefit to patients as they undergo therapy and need additional financing support. This is especially important because the patient may have already exhausted short-term disability funds or need additional help over and above what is provided to them.

3. Therapeutic health credit

The third model we considered drives access to therapies through the financial markets. Using financial instruments that exist today could allow for the diversification of the risk associated with the "lightning strike" nature of CGT claims.

This could take the shape of futures contracts that could be acquired by health plan sponsors to cover the cost of an unexpected future claim. If the claim does not materialize, a secondary market would allow the sponsor to sell the contract to another sponsor who has an immediate need for a treatment.

Alternatively, loan contracts could be structured to amortize the payments related to CGTs. A low-interest loan would make the one-time nature of a CGT more like the traditional chronic care model that health plan sponsors are more familiar with. Those loans with repayment terms could become commodities. If the employee/member left the company after receiving a CGT, the asset could travel with them much like a preexisting solution. Employers would need to agree to take this obligation upfront—perhaps as a precondition of being eligible for the loan program more generally. This could be a role for financial services organizations—from new startups harnessing the power of technology (e.g., blockchain) to industry stalwarts that can provide financial offerings geared toward CGTs.

Transforming financing will require the whole ecosystem

A combination of these (and other) solutions is likely required to drive more equitable access, affordability, and sustained coverage of these life-changing and life-saving products. We also believe that these solutions could be designed to reduce the impact on health plan sponsors, and potentially reduce patient fluidity or member churn across sponsor plans. Since the solutions are employer-driven, clauses could be created allowing employees who remain with the company to access the benefits (e.g., employee must remain for two years post-benefit or they would be required to pay back some of the cost). Additionally, if payments were spread out over time akin to traditional chronic treatments (e.g., through a loan program) and the loans were transferrable to other employers, the question of patient fluidity would matter less. Finally, some of these solutions can harness the adoption of technologies (e.g., artificial intelligence, blockchain) that could efficiently and sustainably address patient portability and data-sharing challenges, and ultimately contribute to a more efficient and accessible CGT ecosystem.

Cell and gene therapies are transformative treatments that require novel financing approaches. Multiple stakeholders, both within the health care ecosystem today and parties from other industries, need to come to the table to create solutions that enable access to and affordability of these innovative therapies. This includes manufacturers, health plan sponsors, and new health care market entrants. Each of these parties has a critical role to play:

- **Manufacturers:** As the source of the scientific innovation, manufacturers must remain on the forefront of creating and adopting novel solutions as they come to market:
  - Develop the evidence to demonstrate the value to the patient and the cost-effectiveness of the therapy, and work with other ecosystem stakeholders to develop innovative contracts to demonstrate the commitment to the therapy.
  - Pool risk across the leading self-funded pharmaceutical manufacturers to start working to set the standard for a novel risk pool.
  - Encourage collaboration among other market players like group insurers and employer benefit consultants to educate them on the intricacies and impact of this emerging class of therapies.
- **Health plan sponsors:** It may be easy to overlook the approaching impact from CGTs, as sponsors are inundated with rising availability, utilization, and cost of specialty therapies (e.g., GLP-1s, oncolytics), and other business priorities.
  - Learn more about the importance and impact that CGTs will have on patient outcomes.
  - Explore novel financing models like those discussed in this article, and work with benefit consultants to include them as options in the requests for proposal and benefit packages for upcoming benefits enrollment cycles.
- **New health care market entrants (e.g., financial services, group insurers, technology companies):** CGTs represent a major shift in how care is delivered. There are opportunities for new market entrants to carve off accretive roles in financing these therapies.
  - Financial services and group insurance companies should explore new models that draw on their expertise (e.g., financial products, supplemental insurance) and apply those to CGTs.
  - Technology companies can provide the capabilities required to implement these solutions (e.g., blockchain) and elevate the patient experience by enabling seamless access to financing solutions.

We encourage appropriate collaboration and innovation across the ecosystem to match the level of scientific innovations by biopharmaceutical companies. This cross-sector and cross-industry approach will require collaboration and an iterative, test-and-learn methodology. We have established a cross-sector working group (i.e., NextGen Therapies Industry Working Group) to support the industry transformation that will be required to support these transformative therapies. In our interactions with this group, financing for CGTs has remained top of mind across participants. It will continue to be discussed in upcoming meetings by this cross-functional consortium of leaders as we consider how we, as an industry, can collaborate to ensure patient access to transformation treatments.

Multiple solutions will be required and can coexist in the ecosystem given the diversity of financing frictions and needs. In developing these models, we can drive more equitable access to life-changing therapies for patients in need today, while simultaneously fostering a more sustainable and efficient health care system for tomorrow. As more therapies move toward market approval, time is of the essence to ensure patients have access to transformational cell and gene therapies.

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Endnotes

1. Evaluate Pharma database, accessed July 15, 2023
2. Ibid.
3. Based on 2022 global sales, DRG; Casey Quinn et al., "Gene therapies and their potential economic impact on US healthcare system," *Value in Health 22*, no. 6 (June 2019): pp. 621–26.
4. Quinn et al., "Gene therapies and their potential economic impact on US healthcare system"; Chi Heem Wong et al., "The estimated annual financial impact of gene therapy in the United States," *Gene Therapy* 30 (2023): pp. 761–73.
5. Based on 2022 global sales, DRG.
6. Institute for Clinical and Economic Review (ICER), "ICER publishes final evidence report on gene therapies for sickle cell disease," press release, August 21, 2023.
7. Ibid.
8. Andy Davis et al., "US health care can't afford health inequities," Deloitte Insights, June 22, 2022; Drew Wilkins et al., "Achieving health equity in life sciences," Deloitte Insights, May 18, 2023; Kulleni Gebreyes et al, "Hiding in plain sight: The health care gender toll," Deloitte, 2023.
9. Eric Faulkner, Michael Werner, and Robert Falb, "Roadmap for navigating cell and gene therapy value demonstration in U.S. managed care," *Journal of Managed Care Medicine*, NAMCP Medical Directors Institute, 2019.
10. Rob Bachler et al., *Managing risks related to gene and cell therapies for self-insured employers with stop-loss coverage*, Milliman, January 2020.
11. Cigna Healthcare, Embarc Benefit Protection, accessed May 2024; CVS Health, "Aetna launches Gene-based, Cellular, and Other Innovative Therapies™ (GCIT) designated network," press release, October 27, 2021; Optum, "Gene therapy: How to prepare for a growing pipeline," accessed May 2024.
12. Brown & Brown, *2023 Employee Benefits Market Trends*, 2023.
13. Bachler et al., *Managing risks related to gene and cell therapies for self-insured employers with stop-loss coverage*.

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