Current and future payment of cell and gene therapies

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Abstract

Background: A significant increase in the number of marketed cell and gene therapies (CGTs) is expected over the next several years. Quantifying how payers currently pay for existing CGTs and understanding how they intend to reimburse and evaluate CGTs in the near future will be important to balance affordable access and cost-containment strategies for these innovations.

Objective: To understand the current and future payment methodologies for CGTs by US payers.

Methods: Xcenda fielded an online survey in October 2020 to a panel of managed care professionals from Xcenda's Managed Care Network. Respondents involved in the review and approval processes for CGT at their organization were asked about current and future payment methodologies. A total of 47 respondents, representing 63 million lives, completed the survey.

Results: For CGTs that have launched in the past 2 years, 62% of the respondents indicate they currently use formulary or utilization management tools, while 32% of respondents use single-patient agreements. Value-based or outcomes-based contracts are in use 28% of the time, and annualized or installment payment are in use 15% of the time.

Looking ahead over the next 12 months, most respondents (66%) are very/extremely likely to use reinsurance to manage the high cost of CGTs. Outcomes-based payment models and contracts directly with distributors or wholesalers each were rated as very/extremely likely to be used by 47% of respondents, while annualized or installment payments are expected to be used over the next 12 months by 28% of respondents.

More than half (51%) of respondents anticipate case rate negotiations for CGTs by indication over the next 3 years. Respondents with regional plans (n=25) anticipate individual patient-level case rate negotiations more often than national plans (n=22) (56% vs 9%, respectively; 95% CI).

Conclusions: Most payers currently manage CGTs using traditional tools like formulary or utilization management. Over the next year, the majority of payers plan to leverage reinsurance. Use of value-/outcomes-based contracting and spreading payments over time are also anticipated to increase over the next 12 months as a technique to manage CGTs.

Introduction

Cell and gene therapies (CGTs) have tremendous promise to provide potentially curative treatments to a host of long-term, congenital, and chronic diseases. The initial group of CGTs entering the market a few years ago had price tags that gave many payers "sticker shock." As a result, payers are still examining how to evaluate and reimburse CGTs.1 Payers are also aware of the potential influx of new CGTs on the cusp of regulatory approval that may exacerbate the issues and require new approaches to evaluate them.

Even with published reports supporting the long-term value of high-cost therapies like CGTs,[2] payers still struggle to absorb these high-cost, high-value treatments and provide access and appropriate reimbursement for them.

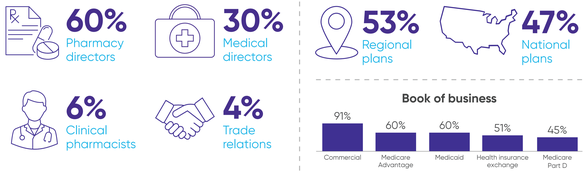
This study focuses on understanding current methodologies payers use to manage and reimburse CGTs and probes the possible changes to CGT payment and reimbursement over the next few years.

Methods

A double-blind, 15-minute, online, quantitative survey consisting of both closed-ended and open-ended questions regarding the current and future review and management of CGTs that have been approved over the past 2 calendar years was fielded October 19 through October 28, 2020 to Xcenda's Managed Care Network advisors. A total of 47 advisors from both national and regional health plans, integrated delivery networks (IDNs), and pharmacy benefit managers (PBMs) responded to the survey. Responses were tabulated and evaluated using z-tests with 95% confidence intervals.

Results

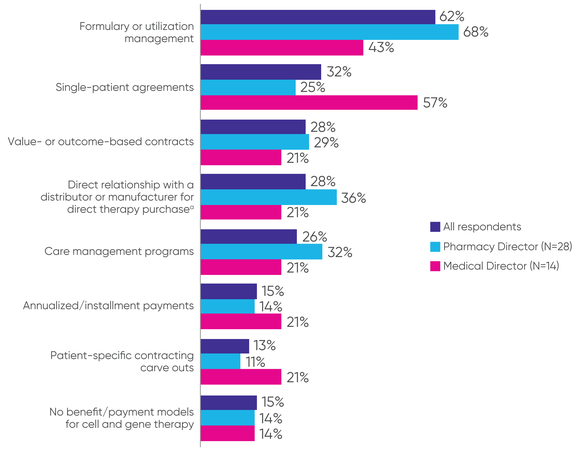
A total of 47 advisors responded to the survey:



Survey questions were written to understand current coverage evaluation methods and potential drivers in future coverage evaluation for CGTs. A total of 42 questions were fielded and a subset of those questions were evaluated and presented in Appendix A.

Figure 1 shows that for CGTs launched in the past 2 years, 62% of the respondents indicate they currently use formulary or utilization management tools, while 32% of respondents use single-patient agreements. Of note is that significantly more medical directors were likely to cite single-patient agreements than pharmacy directors (57% vs 25%; P<0.05). Value-based or outcomes-based contracts are in use 28% of the time, and annualized or installment payments are in use 15% of the time.

Figure 1. Benefit/payment models for CGT launched in past 2 years



* As opposed to provider reimbursement.

Figure 2 shows reinsurance as the most likely payment approach to be used over the next 12 months, with 66% of advisors rating it as "extremely" and "very likely" to use reinsurance. This is compared to outcomes-based payment models and contracts directly with distributors or wholesalers (47% each) or spreading payment over multiple years (28% each) as methods to mitigate high-cost CGTs over the next year.

Figure 2. Likely use of listed CGT payment approaches in the next 12 months

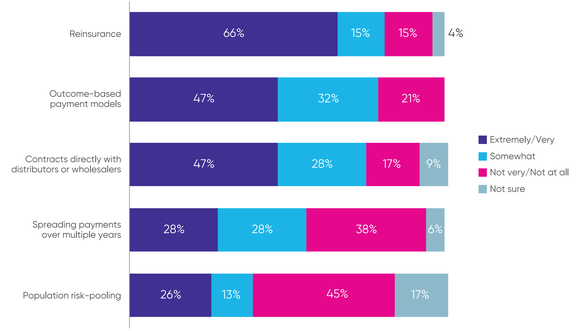
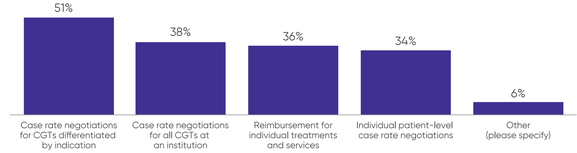


Figure 3 illustrates that most advisors (51%) believe that over the next 3 years, reimbursement for CGTs will evolve into indication-based case rates. This is compared to case rate reimbursement by institution, reimbursement by individual treatments and services, and individual patient-level case rate negotiations (38%, 36%, 34%, respectively). Other responses included expansion of extended payment plans, government engagement in CGT reimbursement (eg, subsidization of current markets), and carve-out programs to supplement current payer benefits.

Figure 3. Anticipated reimbursement for CGTs in next 3 years



Discussion

The key for CGT reimbursement is navigating the challenge of aligning the high humanistic value of these high-cost treatments with potentially long-term clinical outcomes in a system designed to focus on shorter-term outcomes and lower costs. Payers have gravitated toward traditional models such as formularies or utilization management to control access to CGTs. Given the extreme high costs of CGTs and movement of members from one insurance to another, it is not surprising that payers anticipate using reinsurance, value- and outcomes-based contracting, and installment payments more frequently in the near term to address financial outlay concerns.

Case-rate CGT payments based on indication fit nicely into evidence-based methods of assessing value. However, the regulatory landscape1 will need to be addressed before case-rate payments by indication and value-based options reach their full potential to make CGTs accessible to medically appropriate patients.

In summary, most payers currently manage CGTs using traditional tools like formularies or utilization management. The anticipated CGT reimbursement evolution over the next 3 years will likely include a mix of solutions such as reinsurance, value-/outcomes-based contracting, and installment plans. Whatever methodologies payers implement in the future, they will need to address the array of complex issues surrounding CGT and provide the hope of curative treatments to patients and their families.

References

1. Salzman R, Cook F, Hunt T, Malech HL, Reilly P, Foss-Campbell B, Barrett D. Addressing the Value of Gene Therapy and Enhancing Patient Access to Transformative Treatments. *Mol Ther*. 2018 Dec 5;26(12):2717-2726.
2. Marsden G, Towse A, Pearson SD, Dreitlein B, Henshall C. Dreitlein B, Henshall C. Gene therapy: understanding the science, addressing assessing the evidence, and paying for value. Accessed November 1, 2020. https://www.ohe.org/system/files/private/publications/ICER-Gene-Therapy-White-Paper.pdf

Appendix A

Q20. Which of the following **benefits/payment models** between your organization and the provider has your organization developed for **cell and gene therapies** launched in the past 2 years? *Please select all that apply.*

- Formulary or utilization management
- Care management programs
- Patient-specific contracting carve-outs
- Value-based or outcomes-based contracts
- Annualized/installment payments
- Single-patient agreements
- Direct relationship with a distributor or manufacturer for direct therapy purchase (as opposed to provider reimbursement)
- Other (please specify)
- No benefit/payment models for cell and gene therapy

Q29. How likely is it that the following payment approaches be used by your organization within next 12 months to manage the high cost of cell and gene therapies?

- Extremely, very, somewhat, not very, not at all
- Reinsurance
- Outcomes-based payment models
- Contracts directly with distributors or wholesalers
- Spreading payments over multiple years
- Population risk-pooling

Q23. How do you anticipate reimbursement for cell and gene therapy evolving in the next 3 years? Please select all that apply.

- Individual patient-level case rate negotiations
- Case rate negotiations for cell and gene therapy differentiated by indication
- Case rate negotiations for all cell and gene therapy at an institution
- Reimbursement for individual treatments and services
- Other (please specify)

Presented at 2021 AMCP Virtual Meeting
April 12 - 16, 2021

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