US payer perceptions of innovative contracts with cell and gene therapies

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Background

- Payers have begun collaborating with manufacturers on innovative payment models aimed at improving patient outcomes, reducing overall costs to the payer, improving data collection, and assessing the value of medicines. Examples of innovative payment models include outcomes-based contracts, installments/annuity plans, one-time payment models, etc.
- Innovative contracts have the potential to transform access for cell and gene therapies (CGT).
- Examples of CGT innovative contracts include manufacturers such as Novartis engaging with Colorado Medicaid for an outcomes-based contract for Zolgensma and bluebird bio engaging with several payers for an outcomes-based contract for Zynteglo.[1,2]
- CGTs are impactful to payers due to their high cost (ranging from $37,500 to $2 million), long term impact (expected market size of $93.78 billion by 2030), and uncertain long-term benefit.[3] These qualities have a significant impact on payer budgets and create a need for payers to understand new payment models and contracting methods.

Objective

- To understand the current US payer perspectives on and experience with innovative contracts for CGT.

Methods

- An online survey was fielded during October 2022 to payers from Xcenda's Managed Care Network (MCN).
  - The MCN is a proprietary research panel, with over 160 healthcare executives, medical and pharmacy directors, and other experienced individuals in managed care, representing over 310 million covered lives.
- Participants were screened to include individuals from organizations with >50,000 covered lives. In addition, respondents included in the survey were required to be active formulary decision makers and have familiarity and experience with CGT innovative contracts (**Figure 1**).
- The topics of the survey included the current landscape of CGT contracts, desired characteristics in a CGT innovative contract, administrative elements important for ensuring success of CGT innovative contracts, and the future of CGT innovative contracts in the next 3 to 5 years.

Figure 1. Respondent demographics

- A total of 30 advisors from Xcenda's MCN completed the survey:

Advisor demographics (N=30)

60% Health plans | **23%** IDNs | **16%** PBMs

Primary role

77% Pharmacy director | **20%** Medical director | **3%** Contracting manager/director

Enrollment

National 43% | **Regional 57%**

Results

Current landscape of CGT innovative contracts

- Outcomes-based contracts were most prevalent (77%) followed by one-time payment models (63%) and installments/annuity plans (50%) (**Table 1**).
- Outcomes-based contracts were also the most impactful for payer organizations (67%) followed by one-time payment models (43%) and installments/annuity plans (37%).

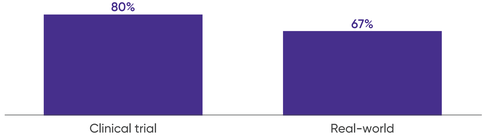
Table 1. Breakdown of current and planned CGT contracting models

	Outcomes-based contracts	One-time payment	Installments/ annuity
Currently involved in	77%	63%	50%
Plan to pursue in next 3-5 years	80%	67%	47%

Desired characteristics in a CGT innovative contract

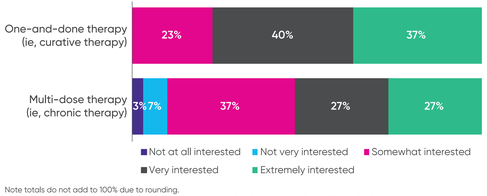
- The most valuable measures for monitoring progress and success of innovative contracts were clinical trial outcomes (80%) followed by real-world outcomes (67%) (**Figure 2**).
- 77% of payers were extremely/very interested in innovative contracts for one-and-done (ie, curative) therapies compared to only 54% who were extremely/very interested in innovative contracts for multi-dose therapies (ie, chronic therapies) (**Figure 3**).
- All payers (100%) considered durability of response to be an important element when assessing the potential of an innovative contract with a CGT.
- 80% of payers indicated that there has not been a shift towards pooled agreements vs drug-specific agreements for CGT innovative contracts.

Figure 2. Types of outcomes that are most valuable for CGT innovative contracts



Q3: Which types of outcomes are the most valuable for your organization for monitoring progress and measuring success of innovative contracts with cell and gene therapies?

Figure 3. Desired product profile for a CGT innovative contract

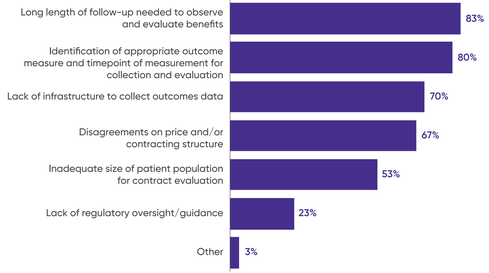


Note totals do not add to 100% due to rounding.
Q4: Please indicate your interest to participate in an innovative contract based on the cell and gene therapy product characteristics below.

Administrative elements important for ensuring success of CGT innovative contracts

- Although only 27% of payers had experience working with a third party to administer CGT innovative contracts, 70% of payers would prefer to work with a third-party administrator.
- Key elements that determine the success of a contract include the number and quality of outcomes measured for a disease state (90%) and the magnitude of rebates/discounts the manufacturer is willing to provide (90%).
- The 2 most important hinderances to innovative contracts include long length of follow-up needed to observe benefits (83%) and identification of appropriate outcome measures and time points (80%) (**Figure 4**).

Figure 4. Factors hindering successful implementation of CGT innovative contracts

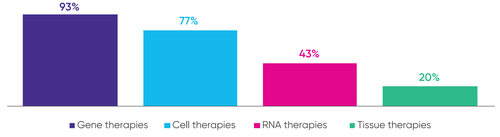


Q8: Which factors have been a hindrance to successful implementation of innovative contracts for cell and gene therapies with pharmaceutical manufacturers for your organization?
Other response: rebate guarantees (n=1)

Future of CGT innovative contracts in the next 3 to 5 years

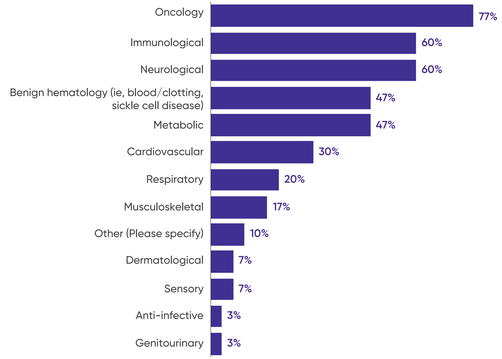
- 80% of payers were interested in outcomes-based contracts followed by 67% for a one-time payment and 47% for installments/annuity plans.
  - Refer to **Table 1** for comparison of current and future CGT innovative contract types.
- 93% of payers were interested in innovative contracts for gene therapies followed by 77% for cell therapies, 43% for RNA therapies, and 20% tissue therapies (**Figure 5**).
- Oncology (77%), neurological diseases (60%), and respiratory conditions (60%) were the top 3 therapeutic areas of interest in the next 3 to 5 years (**Figure 6**).

Figure 5. Type of CGTs of interest for innovative contracts in the next 3-5 years



Q10: What type of cell and gene therapy is your organization most interested in pursuing innovative contracts in the next 3-5 years?

Figure 6. Therapeutic areas of interest for CGT innovative contracts in next 3-5 years



Q11: Which therapeutic areas is your organization most interested in pursuing innovative contracts for cell and gene therapies in the next 3-5 years?
Other responses: rare diseases (n=1), unsure (n=1), hemophilia (n=1)

Limitations

- Survey results were descriptive in nature and based on a small number of respondents and thus may not be generalizable to all payer organizations.
- Because all respondents voluntarily completed the survey, voluntary response bias may exist, and survey results may over-represent respondents with greater involvement in innovative contracts with pharmaceutical manufacturers.
- CGTs are relatively new (first gene therapy approved in 2017),[4] and respondents may not have the depth of knowledge to navigate some of the questions, which could lead to response bias.

Conclusions

- Payers are interested in and have experience with innovative contracts for CGT.
- Outcomes-based contracts are the most common and impactful type of innovative contract.
- Payers perceive clinical trial outcomes and one-and-done therapies with a strong durability of response as important characteristics for a potential contract.
- Future engagements between manufacturers and payers will focus on outcomes-based contracts in therapeutic areas such as oncology, neurology, and respiratory diseases.
- Overall, as more novel CGTs enter the market, innovative contracts will play a crucial role in key disease states to manage costs and improve access.

Key terms

- **Outcomes-based contracts:** Reimbursement for a drug is based, in part, on observed and measurable outcomes of the drug's use in a patient population
- **One-time payment:** Up-front payment for single administration of a CGT
- **Installments/annuity plan:** Payments that are spread over a pre-determined period
- **Clinical trial outcomes:** Specific outcomes measured in the clinical trial that is utilized for FDA approval
- **Real-world outcomes:** New outcomes that are developed by the manufacturer or outcomes that better indicate the experience with the disease (eg, walk test for pulmonary arterial hypertension or pain scale for chronic pain)
- **One-and-done therapy:** Curative therapy that requires only one administration for treatment effect (eg, Zylento for beta-thalassemia)
- **Multi-dose therapy:** Chronic therapy that requires multiple administrations to initiate and sustain treatment effect (eg, Adstiladrin for high-risk Bacille Calmette-Guérin-unresponsive non-muscle-invasive bladder cancer)
- **Cell therapy:** Transfer of intact, live cells into a patient to help lessen or cure a disease; this can be patient cells or donor cells. (eg, tumor-infiltrating lymphocyte therapy, natural killer cell therapy)
- **Gene therapy:** Modify a person's genes to treat or cure a disease either by reducing levels of a disease-causing version of a protein, increase production of disease-fighting proteins, or producing/altering new proteins (eg, Zolgensma, Luxturna, etc)
- **RNA therapies:** Therapies that utilize RNA-based molecules to treat or prevent a disease (eg, patisiran, givosiran, lumasiran, etc)
- **Tissue therapies:** Combination of scaffolds, cells, and biologically active tissues (eg, artificial skin/cartilage, Rethymic)

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