Coverage and Reimbursement of Cell and Gene Therapies: Population Health Versus Individual Patient Perspectives

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PLAIN LANGUAGE SUMMARY

- Cell and gene therapies (C&GTs) introduce the promise of individualized cures and long-term health benefits
- Existing health benefit plans, built on population health principles, are not optimally designed to address a quantum shift in treatment toward individualized therapy
- The risk to patients from inappropriate benefit designs is that access and patient costs follow the trend of orphan products, in which out-of-pocket costs have limited patient access
- Payers have begun to introduce design changes to manage risk that are insurance or debt focused
  - Current implementation is mostly limited to managing population risk using reinsurance
- To appropriately manage this new paradigm, apart from expanding the implementation of existing and/or new insurance and risk-sharing payment models, we suggest there is a need for new, patient-centric thinking
- This includes redefining the very definition of "value" and developing and implementing new methods to incorporate the patient perspective (the perspective of the ultimate payer) into both coverage and reimbursement decision making as well as benefit design itself

BACKGROUND

- As far back as 2016, groups such as the New Drug Development ParadIGmS (NEWDIGS) collaborative partnership (with the Financing and Reimbursement of Cures in the United States initiative), currently housed at Tufts Medical Center, as well as the Value-Based Payment Consortium at the Duke-Margolis Center for Health Policy and the University of Michigan's Center for Value-Based Insurance Design (V-BID), have been working to address the financing and access of C&GT[1-3]
- NEWDIGS focuses on addressing financial challenges, creating strategies and tools for payers, and addressing necessary policy changes with the Centers for Medicare & Medicaid Services to allow for value-based agreements but avoids tackling value assessment
- The Duke-Margolis consortium summarizes the C&GT payment model environment, the role of value-based payment models specifically, and policy barriers to value-based payment
- V-BID states that precision medicine, like C&GT, requires precision benefit design
  - Consumers who diligently follow requirements for their condition should experience low cost sharing.[3] In other words, there are potential solutions to benefit design that do not necessarily focus solely on a population health approach or finance-only models
- C&GTs represent a quantum shift in the traditional drug treatment paradigm, embodying hope for patients with rare and previously untreated diseases, or even promise of a cure[4]
- Historical health benefit (insurance) plan designs and utilization management techniques employed by health care insurers and benefit providers, originally designed for an era of small-molecule drugs and population health principles, are inadequate to address this quantum shift toward individualized therapy[5]
- Patients will be exposed to possibly insurmountable cost sharing without creative solutions to appropriately address access to the new treatment paradigm for C&GT[2]
  - Traditional plans with a 20% copay or high deductible/annual maximums can make these life-saving treatments inaccessible to patients with some of the highest medical need[6]
- In addition, current methods for assessing the "value" of C&GT are lacking[7]
- Current payer-driven design change solutions continue to focus on population risk management with a reinsurance or debt-driven focus[6]
- This poster amends the priorities above, updating payer issues regarding financing C&GT and current novel financial models in practice, as well as exploring opportunities to address patient centricity (i.e., a precision/individual health focus vs the existing population health focus) and the need to expand current thinking on value assessment

OBJECTIVES

- To synthesize current siloed literature that characterizes challenges and dynamics inherent in the C&GT paradigm and assess their relevance to patients/consumers
- To suggest alternatives that could bring patient centricity into solutions for design changes and access decisions

METHODS

- We examined 4 themes in the C&GT literature to characterize the issues, dynamics, and perspectives of the new C&GT paradigm and contextualize it through an overarching patient or consumer lens:
  - Payer concerns about C&GT coverage and reimbursement
  - Innovative insurance and financing models proposed for C&GT
  - Evidence on payer coverage decisions and financing models currently used for C&GT
  - Dimensions of value to properly address C&GT
- The focus was on the extent to which payers and other related health benefit (insurance) plan stakeholders prioritize the patient perspective for C&GT

RESULTS

- By 2030, as many as 75 new C&GTs may be approved by the US Food and Drug Administration. These therapies offer the potential of life-altering treatments, and sometimes cures, for previously untreatable conditions[8,9]

Payer Concerns About C&GT Coverage and Reimbursement

- Payers must account for potential budgetary issues from possible concentrated costs of C&GT
  - A challenge from their perspective is that they may often incur the full cost of therapy at the time of treatment, while the benefits of treatment may not all accrue to them if patients receiving C&GT switch insurers
- It is unknown how much of this risk/benefit transfers as a result of plans receiving the benefits of patients treated with C&GT on other plans
  - For example, when C&GT patients transition from commercial insurance to Medicare, the Medicare program can reap the longer-term benefits of reduced medical expenditures due to C&GTs that were originally reimbursed by commercial payers or Medicaid programs
  - The value of this unique C&GT benefit to Medicare is not recognized by any of the insurance or payment models that have been proposed for C&GT
  - Correspondingly, novel solutions across payers, or requiring collaboration across typically disparate and siloed payer groups, may ultimately be required to address this paradigm shift
- From a patient perspective, access and affordability remain paramount regardless of which insurance plan covers the treatment
  - In assuming a patient perspective, plans have a responsibility to create an environment where the medical treatment of the patient can be prioritized and accommodated

Innovative Insurance and Financing Models Proposed for C&GT

- There are several current and proposed models for C&GT insurance-like (**Table 1**) and debt-like (**Table 2**) payment models
  - For each model, we include potential patient considerations that may help contextualize patient-centric value
- None of the proposed methods address patient cost-sharing risk
  - So far, we have yet to address this key patient access barrier, as the focus remains on managing payer risk via a population health/one-size-fits-all approach versus a precision approach for benefit redesign that matches C&GT precision medicine, where finance models can be adapted to a particular application[10]

Table 1. Current and Innovative Models for Mitigating Payer Risk in Covering C&GT: Insurance-like Designs[11]

Proposed model	Description	Opportunities and barriers to use	Potential patient issues
Industry pooling	Spreads risk across all payers in the pool	Requires a third-party organization to manage	Patient cost sharing is not addressed, and it could lead to disparities in coverage
Multiyear insurance	Insured is covered for multiple years instead of typical 1-year short-term policy	Difficult in US system using predominantly employer-driven health benefits with varying designs and annual balanced budget Medicaid program	Patient cost sharing is not addressed, and it may tie patients into inflexible benefit designs
Stop-loss reinsurance	Pooled insurance with a predetermined upper limit on catastrophic health care claims for self-insured employers	Although C&GT is currently widely used, growth may jeopardize viability	Patient cost sharing is not addressed. Separate risk pools for C&GT lead to similar issues, as per industry pooling

C&GT, cell and gene therapy.

Table 2. Current and Innovative Models for Mitigating Payer Risk in Covering C&GT: Debt-like Designs[11]

Proposed model	Description	Opportunities and barriers to use	Potential patient issues
Annuity payments with and without effectiveness guarantees	Payment is streamed over a longer time period. May include a warranty guarantee	Most popular of all the innovative payment models but also increasingly involves effectiveness guarantees	Patient cost sharing is not addressed. Specific patient benefit is unknown, as there are no incentives created for the patient. Ongoing patient follow-up is necessary to judge outcomes
Health currency	Initial funder is compensated a predetermined percentage of purchase price for lost future savings (health currency) should patient change insurers	Not currently operationalized. Administratively burdensome	Patient cost sharing is not addressed and appears to lack safeguards against sudden patient copay issues at the end of the term
Financial bonds without effectiveness guarantees	Similar to bonds, with coupon payments at established intervals. Principal amount is repaid at bond expiry or other trigger points[12]	Not currently operationalized. Lack of experience with financial institutions that are required to implement	Patient cost sharing is not addressed. Patient cost recapture risk is unknown if any parties default
Financial bonds with effective guarantees	Extends above model, adding an outcomes-based guarantee	Not currently operationalized	Patient cost sharing is not addressed. Ongoing patient follow-up is necessary to judge outcomes

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Evidence on Payer Coverage Decisions and Financing Models Currently Used for C&GT

- Industry pooling (reinsurance) and stop-loss are the most commonly applied methods to address C&GT finance risks[10]
  - Serre et al created 2 models, 1 in cystic fibrosis and 1 in hemophilia, to assess risk exposure to the initial payer and found that, for both disease states, risk pooling led to the highest initial risk exposure, whereas annuity payments with effectiveness guarantees led to the lowest risk[11]
  - Finance models may increasingly shift to debt-like instruments, such as annuity payments or financial bonds, with outcomes guarantees[11]
- If we consider the orphan drug experience as indicative of the future of C&GT, this suggests a different possible course
  - Access to C&GTs may transition to insurance benefit designs using increased utilization review and steerage of patients along with the existing stop-loss/reinsurance financing models
  - Such approaches have historically exposed patients to high out-of-pocket costs
- A series of structured qualitative interviews of decision makers at 15 US payers in 2019 revealed that only 27% were actively engaged in a C&GT management plan and 47% felt that new payment models, such as performance-based agreements and risk pooling, were needed[13]

Dimensions of Value Necessary to Properly Characterize Benefits of C&GT

- One-time, curative treatments can add unique value, on top of risk issues already highlighted, that must be accounted for in a broader context and across multiple payers to equitably support access and reimbursement
- **Table 3** enumerates several dimensions of value relevant for C&GT that are not generally considered in value assessments today[14]

Table 3. Additional Dimensions of Value Associated With C&GT

C&GT additional dimensions of value	Description	Measurement challenges
Value of hope	Concept that technologies offering the chance for a cure are more valuable to many patients	Requires the collection of patient preference data but is relatively straightforward
Insurance value	Reflects the value of the additional peace of mind individuals achieve by participating in an insurance pool. It combines physical risk protection with financial risk protection	Estimated that incorporating the value of insurance would add 40%-60% to the value of morbidity improvements associated with treatments[15]
Severity of disease	Implies that a given gain in health is more valuable to a consumer with a poorer baseline prognosis. This concept is intimately linked to insurance value	Key to measurement is whether individuals respond based on views about care for themselves or on what they believe society should care about
Real option value	Generated when a health technology that extends life creates opportunities for the patient to benefit from other future advances in medicine	Estimated that real option value can add an extra 25% to conventional net monetary benefit (breast cancer)[16]
Spillover effects	Concept relates to unintended positive or negative consequences from investing in a particular technology. For example, the development of a cell therapy for a particular illness might turn out to be relevant for treating other diseases or might involve the development of technology for rapid sequencing that is applicable to other diseases	Difficult to measure unforeseen benefits/costs, the risk of double counting benefits, and the opportunity cost of covering the original treatment

C&GT, cell and gene therapy.

Patients as the Ultimate Payer

- As C&GT-treated patients transition from commercial health insurance or Medicaid plans to Medicare, a sizeable potential social surplus could result as Medicare inherits previously cured patients
- As stated earlier, there are various innovations in health insurance design and policy related to C&GT that continue to take a risk-based population health approach to managing coverage and reimbursement (i.e., responsibility for decision making lies solely with private and government payers; **Table 1**)
- We propose a need for a different approach that is more centered around patients, with the premise that the patient/consumer is the ultimate payer, whether through insurance premiums, benefit plan copays, reduced wages to accommodate benefit plans as part of an overall reimbursement package, taxation, the price of goods and services, etc
  - Currently, patients have no meaningful input into benefit design, or even access policy
- **Table 4** highlights current mechanisms for patient contribution, which are limited, as well as proposed future mechanisms
  - The patient perspective currently influences value assessments, mainly through patient preferences or outcomes; however, if the patient is the ultimate payer, then patients should be consulted on defining the correct objective function (or the desired optimization target that is typically minimized or maximized), which should drive access policy decisions and health plan designs
  - Data that are critical for outcome measurements or patient preferences require patient follow-up and participation, highlighting the additional need for a patient-centric design

Table 4. Current and Proposed Mechanisms for Incorporating Consumer Perspectives in Coverage and Reimbursement Decision Making

Current mechanisms	Proposed mechanisms
Payer-centric approach, with limited consumer input, often using nonspecific, insensitive instruments in value-assessment cost-effectiveness modeling used for health technology assessment decision making	Patient-centric approach incorporating additional dimensions of value into economic evaluation. For C&GT, at a minimum, these should include the value of hope, insurance, severity of disease, real option value, and spillover effects of therapy
Consumer choice limited to off-the-shelf benefit designs. Steerage to more "efficient" treatments from the payer perspective or choice based on the individual's financial situation	Incorporate consumer and payer perspectives and offer a tailored patient-centric and value-based benefit design that does not place financial barriers on treatment
	Integrate consumer and payer perspectives into value assessment and subsequent decision making

C&GT, cell and gene therapy.

CONCLUSIONS

- C&GTs introduce the promise of individualized cures and long-term health benefits
- Existing health benefit plans, built on population health principles, are not optimally designed to address a quantum shift in treatment toward individualized therapy
- The risk to patients from inappropriate benefit designs is that access and patient costs follow the trend of orphan products, in which out-of-pocket costs have limited patient access
- Payers have begun to introduce design changes to manage risk that are insurance or debt focused
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