

# Medicaid Coverage Practices for Approved Gene and Cell Therapies: Existing Barriers and Proposed Policy Solutions

## Article Summary

The American Society of Gene and Cell Therapy (ASGCT) commissioned a study in 2022 to identify Medicaid coverage policies for currently approved gene and cell therapies in response to anecdotal reports of variations in policies resulting in inconsistent access and time-to-treatment for patients. Under the federal Medicaid statute, the medically accepted indication for coverage by the program is defined as “any use of a covered outpatient drug which is approved [by the FDA], or the use of which is supported by one or more citations included or approved for inclusion in any of the compendia described in section 1927(g)(1)(B)(i).”<sup>1,2</sup>

This study was conducted against the backdrop of a robust pipeline of gene therapies in development for conditions that will have large Medicaid populations. The one-time nature of these therapies that result in durable benefits by treating the underlying cause of disease result in high upfront high costs that will be difficult for state budgets to handle despite the long-term savings.

In addition to the policies, we sought to quantify common barriers to coverage access from a multi-stakeholder perspective and discuss federal policy options that could improve coverage to ensure equitable patient access in the Medicaid program.<sup>3</sup>

### Methods

- Studied 16 states and 3 MCOs (46 million individuals) across geography, population size, political leadership, and program structure<sup>1</sup>
- Assessed publicly available coverage policies for three products
  - KYMRIAH<sup>®</sup>, a CAR T-cell therapy to treat adult relapsed/refractory diffuse large B-cell lymphoma as well as pediatric and young adult relapsed/refractory B-acute lymphoblastic leukemia.
  - LUXTURNA<sup>®</sup>, a gene therapy for children and adults with an inherited retinal dystrophy.
  - ZOLGENSMA<sup>®</sup>, a gene therapy to treat children with spinal muscular atrophy.

### Results

	KYMRIAH	LUXTURNA	ZOLGENSMA
<b>Coverage Broader than FDA Labeled Indication</b>	1 [OR]	0	0
<b>Coverage to FDA Labeled Indication</b>	8 [CA, CO, FL, MA, NY, NC, OK, TX]	4 [MA, NY, United, Anthem]	0
<b>Coverage more Restrictive than FDA Labeled Indication</b>	4 [MS, United, Anthem, Centene]	9 [CA, FL, IN, MS, NC, OK, OR, TX, Centene]	14 [CA, CO, FL, IN, MA, MS, NY, NC, OK, OR, TX, United, Anthem, Centene]
<b>No Policy Available</b>	6 [AZ, AR, GA, IL, IN, MI]	6 [AZ, AR, CO, GA, IL, MI]	5 [AZ, AR, GA, IL, MI]

<sup>1</sup> 42 U.S.C. § 1396(a).

<sup>2</sup> Includes the American Hospital Formulary Service Drug Information, United States Pharmacopeia-Drug Information (or its successor publications), and the DRUGDEX Information System

<sup>3</sup> The full article can be found: [https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501\(23\)00077-3](https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501(23)00077-3)

Payor requirements determined to be...	
in accordance with the labeled indication	not in accordance with the labeled indication
Confirmation of diagnosis or genetic mutation(s) for which the product is approved	Age limitations are narrower than the label (unless the payor does not cover such ages)
Confirmation of physical attributes required for the product to work (e.g., confirmation of viable retinal cells to be transduced, tumor marker expression required for drug mechanism of action, etc.)	Severity of condition thresholds (visual acuity, advanced disease, physical performance scores, expectation of outcomes)
Requirements to monitor or assess physiological markers or functionality recommended in the label	Limitations on use based on pregnancy or being of childbearing age, even if not recommended in pregnancy in section 8.1 of the FDA package insert
Requirements to have failed previous lines of therapy consistent with the indications and usage section of the label	Limiting use in populations not included in the clinical trial, even if the lack of data from such populations is noted in the indications and usage section of the label
Limitations that match the contraindications included in the indications and usage section of the label	

### Takeaways

States are not always adhering to the requirements to provide coverage for products to their “medically accepted” indication.

- States are narrowing coverage to populations covered in clinical trials, despite the broader labeled indication
  - FDA has wide scientific and legal latitude to establish the labeled indication based on “substantial evidence,” and does not obligate a clinical trial population to match the patient population once approved
  - To demonstrate product benefit, clinical trial eligibility criteria may be much narrower than the population FDA approves for use in the indication
  - Gene and cell therapies frequently have small clinical trial populations by the nature of the rare diseases they aim to treat
  - A state’s determination of medical necessity should not substitute for the federal definition of medically accepted indication for coverage, nor the judgment of the treating physician
- States require additional information or eligibility criteria that is not indicated in the drug’s label
  - Despite Medicaid patients meeting the “medically accepted indication” standard, ASGCT has heard reports of these patients receiving an initial denial of insurance approval
  - Many Medicaid denials are accompanied by requests for additional information, likened to “eligibility criteria”, that is above and beyond simply requesting confirmation that a patient’s condition corresponds to the labeled indication of the commercial product

### **Additional Barriers to Coverage**

- States have difficulty anticipating the timing of new advanced product approvals
  - States claim they are unaware of therapies with high, frontloaded costs coming to market, yet limit meetings with manufacturers to discuss pipeline products
  - Yearly state budgets have little flexibility to accommodate new products approved afterwards, and consider other fiscal pressures (infrastructure, education, etc.)
- Appeal processes are slow and burdensome, compounding issues with delays in time to treatment
  - While processes exist, they are unclear and burdensome for patients and providers
  - These delays in treatment may cause a patient to “age out” of the medically accepted indication or develop additional non-reversible comorbidities

The American Society of Gene & Cell Therapy is the primary professional membership organization for gene and cell therapy. The Society's members are scientists, physicians, patient advocates, and other professionals. Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, and biotechnology and pharmaceutical companies.

For more information about cell and gene therapy payment policies, please contact [advocacy@asgct.org](mailto:advocacy@asgct.org).