ADMINISTRATIVE POLICY STATEMENT
Marketplace

Policy Name & Number	Date Effective
Cellular, Gene, and Cell-Based Gene Therapy-MP-AD-1623	05/01/2026
Policy Type	
ADMINISTRATIVE	

Administrative Policy Statement prepared by CareSource and its affiliates are derived from literature based on and supported by clinical guidelines, nationally recognized utilization and technology assessment guidelines, other medical management industry standards, and published MCO clinical policy guidelines. Medically necessary services include, but are not limited to, those health care services or supplies that are proper and necessary for the diagnosis or treatment of disease, illness, or injury and without which the patient can be expected to suffer prolonged, increased or new morbidity, impairment of function, dysfunction of a body organ or part, or significant pain and discomfort. These services meet the standards of good medical practice in the local area, are the lowest cost alternative, and are not provided mainly for the convenience of the member or provider. Medically necessary services also include those services defined in any Evidence of Coverage documents, Medical Policy Statements, Provider Manuals, Member Handbooks, and/or other policies and procedures.

Administrative Policy Statements prepared by CareSource and its affiliates do not ensure an authorization or payment of services. Please refer to the plan contract (often referred to as the Evidence of Coverage) for the service(s) referenced in the Administrative Policy Statement. If there is a conflict between the Administrative Policy Statement and the plan contract (i.e., Evidence of Coverage), then the plan contract (i.e., Evidence of Coverage) will be the controlling document used to make the determination.

According to the rules of Mental Health Parity Addiction Equity Act (MHPAEA), coverage for the diagnosis and treatment of a behavioral health disorder will not be subject to any limitations that are less favorable than the limitations that apply to medical conditions as covered under this policy.

This policy applies to the following Marketplace(s):			
☒ **Georgia**	☒ **Indiana**	☒ **Ohio**	☒ **West Virginia**

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A. Subject
Cellular, Gene, and Cell-Based Gene Therapy

B. Background
Genetic disorders including inherited conditions and acquired conditions such as cancer and infections cause a significant burden on society in cost, resources, and patient care. Treatment for most genetic conditions relies on management of symptoms rather than cure. However, new molecular techniques are being developed in an attempt to cure specific genetic diseases as well as cancers and certain types of infections.

Gene therapy, also called gene editing, aims to create sequence-specific alterations in the DNA of a cell using molecular methods that take advantage of site-directed DNA repair after strand breakage. Current techniques utilize CRISPR and a viral vector, such that a specific sequence of a person's DNA can be excised and replaced with a different DNA sequence. Gene therapy may be used for several purposes (eg, replace a missing gene, bypass the role of a missing gene, re-establish the function of a gene, augment therapy for a disease). Diseases caused by a gene or protein deficiency can potentially be cured using this technique. Gene therapy is currently being studied for a variety of disorders, including cancers, sickle cell disease, hemophilia, Duchenne muscular dystrophy, idiopathic macular telangiectasia type 2, type 1 diabetes, and retinal dystrophy. However, there are several concerns regarding the utilization of gene therapy. Since gene therapy is designed to result in a permanent change, the potential for off-target genome modifications that lead to aberrant gene expression, chromosomal translocations, and malignancy induction are all potential outcomes, as well as the risk for insertional mutagenesis from vectors delivering genome editing components and the risk of tumorigenicity. In addition, there is the potential for an immune response to the genome-editing products. Gene therapy is a complex process which needs to appropriately select the target cells, genomic site to modify, technology used to edit the genome, vector used to deliver the modified material, and the route of administration. Long term follow-up is also necessary, to ensure oncogenesis and immune responses do not occur. As such, most gene therapies are still being tested in clinical trials.

Cellular therapy aims to replace damaged cells or boost the immune system by using living cells either modified or directly acquired from donors. While cellular therapy includes both stem cell transplants and engineered immunotherapies like CAR T-cells, this policy is not meant to apply to stem cell transplants where no genetic manipulation is performed on the donor cell. In CAR-T therapy, a patient's T-cells are collected, genetically modified to target specific cancer cells, then reinfused. Examples of FDA-approved CAR-T therapies include Kymriah and Yescarta for some lymphomas, and Abecma and Carvykti for multiple myeloma. While current CAR T-cell therapies are derived from the patient's cells (ie, autologous), a number of complications can arise. Cytokine release syndrome (CRS) occurs in 25% to 50% of patients treated with CAR T-cell therapy for relapsed/refractory acute lymphoblastic leukemia/lymphoma, but occurs at a lower frequency for other cancers. CRS is an acute, systemic inflammatory syndrome characterized by fever and multiple organ dysfunction which may result in

hospitalization. Immune effector cell-associated neurotoxicity syndrome (ICANS) is a neuropsychiatric syndrome that occurs in up to 70% of patients receiving immunotherapies, including CAR T-cell therapy. Other complications include cytopenia, infection, and development of secondary neoplasms.

The FDA Center for Biologics Evaluation and Research (CBER) regulates cellular therapy products and human gene therapy products as biologics, as well as some devices related to cellular and gene therapy. CBER evaluates new biological products based on scientific and clinical data submitted by the manufacturer. Approval decisions are based on a risk-benefit analysis for the intended population and the product's intended use. According to CBER, "although medical products are required to be safe, safety does not mean zero risk…a safe biological product is one that has reasonable risks, given the patient's condition, the magnitude of the benefit expected, and the alternatives available."

C. Definitions

- **Cell Therapy** – A therapy in which specific cell types are injected, grafted, or implanted into a patient to treat or prevent a disease.
- **CRISPR** – Clustered regularly interspaced short palindromic repeats is a method used for gene editing, adapted from a component of a bacterial defense system in combination with an endonuclease.
- **Drug** – A medication or substance which induces a physiologic effect on the body of a member (eg, medication, agent, drug therapy, treatment, product, biosimilar drug).
- **Gene Therapy** – Changes in gene expression, achieved by replacing or correcting a disease-causing gene, inactivating a target gene, or inserting a new or modified gene, using a vector or delivery system of genetic sequence or gene, genetically modified microorganisms, viruses, or cells.

D. Policy

I. Refer to the member's Evidence of Coverage (EOC) and Schedule of Benefits (SOB) for coverage eligibility, and CareSource medical/pharmacy policies on cellular, gene, and cell-based gene therapy, if active, for coverage criteria. Each Marketplace plan's coverage, limitations, and/or exclusions may vary, as stated in the member's EOC/SOB. Services, procedures, and pharmaceuticals, when a covered benefit, must be medically necessary and may be subject to prior authorization.

E. State-Specific Information

N/A

F. Conditions of Coverage

N/A

G. Related Policies/Rules

Medical Necessity Determinations
Medical Benefit Medication

The ADMINISTRATIVE Policy Statement detailed above has received due consideration as defined in the ADMINISTRATIVE Policy Statement Policy and is approved.

H. Review/Revision History

	DATE	ACTION
Date Issued	01/28/2026	New policy, approved at Committee.
Date Revised		
Date Effective	05/01/2026	
Date Archived		

I. References

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