



# ADMINISTRATIVE POLICY STATEMENT

## Nevada Marketplace

Policy Name & Number	Date Effective
Cellular, Gene, and Cell-Based Gene Therapy-NV MP-AD-1624	05/01/2026
Policy Type	
<b>ADMINISTRATIVE</b>	

Administrative Policy Statements 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 or Certificate of Coverage documents, Medical Policy Statements, Provider Manuals, Member Handbooks, and/or other plan policies and procedures.

Administrative Policy Statements do not ensure an authorization or payment of services. Please refer to the plan contract (often referred to as the Evidence of Coverage or Certificate of Coverage) for the service(s) referenced in the Administrative Policy Statement. Except as otherwise required by law, if there is a conflict between the Administrative Policy Statement and the plan contract, then the plan contract 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.

### Table of Contents

A.	Subject .....	2
B.	Background .....	2
C.	Definitions .....	3
D.	Policy .....	3
E.	Conditions of Coverage .....	3
F.	Related Policies/Rules .....	3
G.	Review/Revision History .....	4
H.	References .....	4

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

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

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. Services, procedures, and pharmaceuticals, when a covered benefit, must be medically necessary and may be subject to prior authorization.

### E. Conditions of Coverage

NA

### F. 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.

### G. Review/Revision History

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

### H. References

1. American Society of Gene and Cell Therapy. What is Cell Therapy? Updated December 18, 2023. Accessed December 9, 2025. [www.patienteducation.asgct.org](http://www.patienteducation.asgct.org)
2. Arabi F, Mansouri V, Ahmadbeigi N. Gene therapy clinical trials, where do we go? An overview. *Biomedicine & Pharmacotherapy*. 2022;153:113324. doi:10.1016/j.biopha.2022.113324
3. Argiro A, Ding J, Adler E. Gene therapy for heart failure and cardiomyopathies. *Rev Esp Cardiol*. 2023;76(12):1042-1054. doi:10.1016/j.rec.2023.06.009
4. Centers for Disease Control and Prevention. Prevention and Treatment of SCD Complications. October 3, 2024. Accessed November 25, 2025. [www.cdc.gov](http://www.cdc.gov)
5. Centers for Disease Control and Prevention. Treatment of Thalassemia. January 2, 2025. Accessed November 25, 2025. [www.cdc.gov](http://www.cdc.gov)
6. Dietrich J, Frigault MJ. Immune effector cell-associated neurotoxicity syndrome (ICANS) and other neurologic toxicities of CAR-T cell and related therapies. UpToDate. Updated August 7, 2025. Accessed December 12, 2025. [www.uptodate.com](http://www.uptodate.com)
7. Flomenberg P, Daniel R. Overview of gene therapy, gene editing, and gene silencing. UpToDate. Updated October 22, 2025. Accessed November 25, 2025. [www.uptodate.com](http://www.uptodate.com)
8. Genetic Testing Registry. National Center for Biotechnology Information, U.S. National Library of Medicine. Accessed November 25, 2025. [www.ncbi.nlm.nih.gov](http://www.ncbi.nlm.nih.gov)
9. Hill JA. Human herpesvirus 6 infection in hematopoietic cell transplant and CAR-T cell therapy recipients. UpToDate. Updated December 3, 2024. Accessed December 12, 2025. [www.uptodate.com](http://www.uptodate.com)
10. Issa S, Shaimardanova AA, Solovyeva VV, et al. Various AAV serotypes and their applications in gene therapy: an overview. *Cells*. 2023;12,785. doi:10.3390/cells12050785
11. Jiang L, Wang D, He Y, et al. Advances in gene therapy hold promise for treating hereditary hearing loss. *Mol Ther*. 2023;31(4):934-950. doi:10.1016/j.ymthe.2023.02.001
12. Kitawi R, Ledger S, Kelleher AD, et al. Advances in HIV gene therapy. *Int J Mol Sci*. 2024;25(5):2771. doi:10.3390/ijms25052771
13. Li YR, Zhu Y, Fang Y, et al. Emerging trends in clinical allogeneic CAR cell therapy. *Med*. 2025;6(8):100677. doi:10.1016/j.medj.2025.100677
14. National Human Genome Research Institute. Coverage and Reimbursement of Genetic Tests. February 6, 2024. Accessed November 25, 2025. [www.genome.gov](http://www.genome.gov)

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

15. Porter DL, Maloney DG. Cytokine release syndrome (CRS). UpToDate. Updated April 2, 2024. Accessed December 12, 2025. [www.uptodate.com](http://www.uptodate.com)
16. Raby BA, Blank RD. Genetics: Glossary of terms. UpToDate. Updated November 5, 2024. Accessed November 25, 2025. [www.uptodate.com](http://www.uptodate.com)
17. Shahid S, Prockop SE, Flynn GC, et al. Allogeneic off-the-shelf CAR T-cell therapy for relapsed or refractory B-cell malignancies. *Blood Adv*. 2025;9(7):1644-1657. Doi:10.1182/bloodadvances.2024015157
18. Shoushtari AN, Johnson DB. Principles of cancer immunotherapy. UpToDate. Updated July 16, 2024. Accessed December 12, 2025. [www.uptodate.com](http://www.uptodate.com)
19. Uddin F, Rudin CM, Sen T. CRISPR gene therapy: applications, limitations, and implications for the future. *Front Oncol*. 2020;10:1387. doi:10.3389/fonc.2020.01387
20. US Department of Health and Human Services, Food and Drug Administration, Center for Biologics Evaluation and Research. Long term follow-up after administration of human gene therapy products: guidance for industry. January 2020. Accessed November 26, 2025. [www.fda.gov](http://www.fda.gov)
21. US Food and Drug Administration: Cellular & Gene Therapy Products. March 20, 2023. Accessed November 26, 2025. [www.fda.gov](http://www.fda.gov)
22. US Food and Drug Administration: What is gene therapy? July 25, 2018. Accessed November 26, 2025. [www.fda.gov](http://www.fda.gov)
23. US Food and Drug Administration: Approved Cellular and Gene Therapy Products. Updated November 26, 2025. Accessed November 26, 2025. [www.fda.gov](http://www.fda.gov)
24. Zhang L, Wang Y. Gene therapy in epilepsy. *Biomedicine & Pharmacotherapy*. 2021;143:112075. doi:10.1016/j.biopha.2021.112075

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