CELLULAR AND GENE THERAPIES OFFER PROMISING TREATMENTS, BUT AT HIGH COSTS

Emerging cellular and gene therapies hold promise for managing a wide range of diseases. These treatments are typically given one time to manage a disease that previously was untreatable and that in some cases can now be cured.

Genetic diseases are caused when a gene is missing or defective from birth, or sometimes when a gene has changed over time. Gene therapy targets a gene that's causing a medical problem and replaces it or inserts an additional gene to treat, cure or prevent a disease or medical condition, like spinal muscular atrophy.

Cellular therapy is the introduction of new cells to grow, replace or repair damaged tissue or cells. With new technologies and innovative products, various types of cells may be used as part of a therapy or treatment. Cellular therapy is largely focused on treating blood and solid organ cancers.

The value and role of genetic testing

Genetic testing is an emerging diagnostic tool used for the identification and treatment of genetic diseases or conditions in which mutations are common, like cancer.

Several genetic tests have well-documented, evidence-based value and are covered by Blue Cross Blue Shield of Michigan and Blue Care Network. In order to be considered for coverage, genetic tests must focus on guiding the treatment or care plan of our members. The use of genetic testing has expanded as more targeted therapies become available to identify genetic differences present in a patient and then use medication to target those specific differences.

FDA-approved therapies available

In the United States, the Food and Drug Administration has approved the first cellular and gene therapies, and others may be approved in the future.

Drugs	Use	Cost	Covered benefit
Kymriah® and Yescarta®	Cellular immunotherapy, or CAR-T, for treating blood cancers	$525,000 to $700,000 for drugs and administration costs	Yes, when medical policy criteria met
Luxturna®	Gene therapy for child blindness, Treats RPE65 mutation-associated retinal dystrophy, a condition that causes progressive blindness early in life	$850,000 one-time therapy	Yes, when medical policy criteria met
Zolgensma®	Gene therapy for spinal muscular atrophy, caused by a defect in the SMN1 gene	$2.125 million one-time therapy	Yes, when medical policy criteria met

Blue Cross and BCN actively manage drug costs

- New therapies are proactively evaluated by doctors and pharmacists in the Blue Cross and BCN multifunctional Emerging Therapies Workgroup and then reviewed by the Pharmacy & Therapeutics Committee to:
  - o Review the safety and effectiveness of the drug
  - o Determine the clinical criteria for appropriate use of the drug
  - o Determine whether the drug will be covered under pharmacy or medical benefits
- There are medical policies and clinical guidelines in place for each FDA-approved drug.
- Clinicians review medical records, including genetic testing results when applicable, to make sure these treatments are appropriate for patients, according to our medical policy.
- We negotiate outcomes-based contracts with drug manufacturers to tie the cost of medication to its performance over time.

On the horizon

Cellular and gene therapies that are still in clinical trials are showing promise in the treatment of lifelong diseases. Blue Cross and BCN will continue to monitor these developments and advocate for high-value treatments — high-quality care at the lowest net cost — for our members.

Drugs	Use	Estimated Cost	Anticipated approval
JCAR017 (lisocabtagene)	CAR-T, blood cancers	$350,000 to $500,000 for drugs and administration costs	2020
Zynteglo®	Gene therapy for transfusion-dependent beta thalassemia	$2 million one-time therapy	2020
Hemophilia gene therapies	Gene therapy for hemophilia A and B	$2 million to $3 million one-time therapy	2020