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Cell and Gene Therapy State of the Market

The cell and gene therapy market continues to grow, with the number of drugs in the clinical pipeline increasing every year. Cell and gene therapy drugs hold the potential to cure previously incurable diseases, but they often come with a hefty price tag. That's why it's important for health care payers to have a strategy in place to manage costs.

Berkley Accident and Health is actively monitoring the cell and gene therapy market and is pleased to offer pricing solutions along with data analytics to our policyholders through our relationship with Emerging Therapy Solutions (ETS). We collaborate with ETS to bring you these regular cell and gene therapy updates.

Recently Approved Drugs

Drug Name	Type	Conditions Treated	Prevalence	FDA Approval Date and Est. Price
Lifeucel (LN-144)	Cell Therapy	Metastatic melanoma	1 in 1,413 people	2/16/2024 **$515,000**
Lyfgenia (lovotibeglogene autotemcel)	Gene Therapy	Sickle cell disease	1 in 4,226 people	12/8/2023 **$3.1 million**
Casgevy (exagamglogene autotemcel)	Gene Therapy	Sickle cell disease	1 in 4,226 people	12/8/2023 **$2.2 million**
Casgevy (exagamglogene autotemcel)	Gene Therapy	Transfusion-dependent beta-thalassemia	1 in 5,000 people	1/16/2024 **$2.2 million**

Source: Emerging Therapy Solutions, https://emergingtherapies.com/ and Berkley Accident and Health internal data

DRUG SPOTLIGHT

Casgevy

On December 8, 2023, Vertex Pharmaceuticals received FDA approval for Casgevy, a gene therapy for sickle cell disease in patients 12 years and older. It has an estimated price of $2.2 million. Sickle cell disease is an inherited blood disorder that affects the ability of hemoglobin in red blood cells to carry oxygen throughout the body.[1] Red blood cells with normal hemoglobin are smooth and disk-shaped, easily moving through the blood vessels. Cells with misshapen sickle cell hemoglobin stick together and cause blockages in the small blood vessels, which leads to anemia, pain, and other life-threatening health problems.

Patients receive Casgevy via a one-time IV infusion. Casgevy uses CRISPR/Cas9 gene-editing technology to modify the patient's blood stem cells to produce healthy hemoglobin. Over time, Casgevy reduces the number of sickle cells in the body to a negligible amount, which can reduce symptoms in the long-term.[2] In January 2024, the FDA approved Casgevy for a second blood disorder, called transfusion-dependent beta-thalassemia.

[1]National Heart, Lung, and Blood institute, ***What Is Sickle Cell Disease?***, https://www.nhlbi.nih.gov/health/sickle-cell-disease

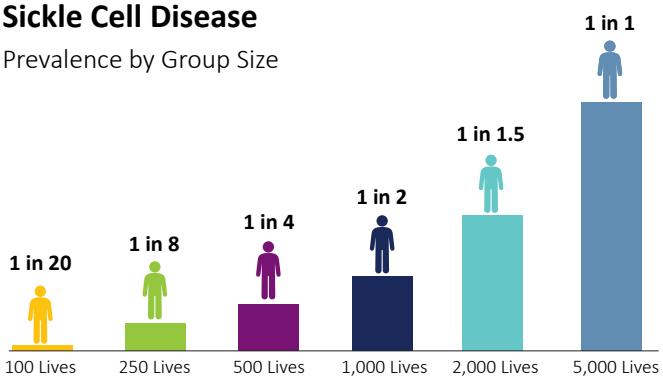
[2]Sickle Cell Disease News, ***Casgevy for sickle cell disease,*** https://sicklecellanemianews.com/ctx001-sickle-cell-disease

Financial Risks for Payers

The likelihood of having a Casgevy claim increases as the group size grows. For groups of 5,000, there is a 1 in 1,138 chance of having a covered member with sickle cell disease qualify to receive Casgevy.[3] Smaller groups have a lower, but still very real, chance of having a Casgevy claim.

Sickle Cell Disease

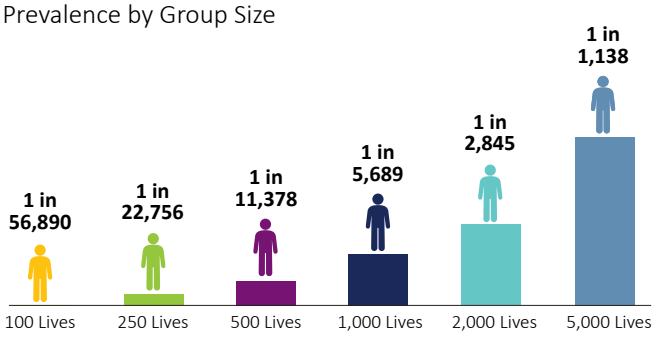
Prevalence by Group Size



Likelihood of having a covered member diagnosed with the above condition.

Casgevy

Prevalence by Group Size



Likelihood of having a covered member eligible for Casgevy.

Assumes each enrolled employee represents 2.2 members and the birth rate per employee is consistent with the national average. Likelihood is defined as the percentage chance in any calendar year. Source: Emerging Therapy Solutions, https://emergingtherapies.com/

Looking Forward: Expected Approvals for 2024-2025

The FDA has stated its intent to accelerate the approval pathway for cell and gene therapies.[4] For 2023-2024, the FDA is expected to approve 73 new therapies, an increase of more than 160% over the 28 approvals in 2021-2022.[5] Here is a snapshot of the growing pipeline of therapies awaiting approval:

Drug Name	Type	Conditions Treated	Prevalence	Expected FDA Approval Date
Obe-cel (Obecabtagene autoleucel)	Cell Therapy	Acute lymphoblastic leukemia	1 in 14,925 people	11/16/2024
Breyanzi (lisocabtagene maraleucel)	Cell Therapy	Chronic lymphocytic leukemia Small lymphocytic lymphoma	1 in 25,000 people	2024
Tab-cel (tabelecleucel)	Cell Therapy	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	1 in 5,280 people	2024
Afami-cel (afamitresgene autoleucel)	Cell Therapy	Synovial sarcoma	1 in 55,476 people	2024
Afami-cel (afamitresgene autoleucel)	Cell Therapy	Myxoid/round cell liposarcoma	1 in 100,000 people	2024
Lete-cel (letetresgene autoleucel)	Cell Therapy	Synovial sarcoma	1 in 55,476 people	2024
Lete-cel (letetresgene autoleucel)	Cell Therapy	Myxoid/round cell liposarcoma	1 in 100,000 people	2024
Lifeucel (LN-144)	Cell Therapy	Cervical cancer	1 in 6,218 people	2024-2025
Zevor-cel (zevorcabtagene autoleucel)	Cell Therapy	Multiple myeloma	1 in 5,847 people	2024-2025

[3]Berkley Accident and Health internal data

[4]Reuters, *US FDA to take steps to help gene therapies get accelerated approval,* https://www.reuters.com/world/us/us-fda-official-says-agency-needs-start-using-accelerated-approval-gene-2023-03-20/

[5]Emerging Therapy Solutions, actual 2021-2022 approvals and expected approvals for 2023-2024, https://emergingtherapies.com/

Drug Name	Type	Conditions Treated	Prevalence	Expected FDA Approval Date
Kresladi (marnetegragene autotemcel)	Gene Therapy	Leukocyte adhesion deficiency type I	1 in 380,952 people	3/31/2024
Pz-cel (prademagene zamikeracel)	Gene Therapy	Recessive dystrophic epidermolysis bullosa	1 in 50,000 people	5/25/2024
Libmeldy (atidarsagene autotemcel)	Gene Therapy	Metachromatic leukodystrophy	1 in 363,981 people	2024
Fidanacogene elaparvovec	Gene Therapy	Hemophilia B	1 in 41,576 people	2024
RP-L102	Gene Therapy	Fanconi anemia	1 in 725,926 people	2024
PTC-AADC[6] (eladocagene exuparvovec)	Gene Therapy	Aromatic L-amino acid decarboxylase (AADC)	1 to 3 in 100,000 newborns	2024
RGX-121	Gene Therapy	Mucopolysaccharidosis type II	1 in 74,400 people	2024
AAV-RPGR (botaretigene sparoparvovec)	Gene Therapy	X-linked retinitis pigmentosa	1 in 18,293 people	2024
Lumevoq (lenadogene nolparvovec)	Gene Therapy	Leber hereditary optic neuropathy	1 in 50,000 people[7]	2024

[6]NORD Rare Disease Database, ***Aromatic L-Amino Acid Decarboxylase Deficiency,*** https://rarediseases.org/rare-diases/aromatic-l-amino-acid-decarboxylase-deficiency/
[7]NORD, ***Leber Hereditary Optic Neuropathy***, https://rarediseases.org/rare-diseases/leber-hereditary-optic-neuropathy/

Source: Source: Emerging Therapy Solutions, https://emergingtherapies.com/ and Berkley Accident and Health internal data

For more information about risk strategies to manage cell and gene therapy claims, contact your Berkley Accident and Health representative.

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