

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.

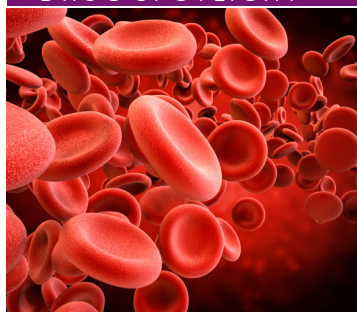
Berkley Accident and Health is actively monitoring the gene therapy market and is pleased to offer pricing solutions and data analytics to our policyholders through our relationship with Emerging Therapy Solutions (ETS). This is the second white paper in our cell and gene therapy series, and the first one of 2023.

Recently Approved Drugs

Drug Name	Type	Conditions Treated	Prevalence	FDA Approval Date and Est. Price
Yescarta (axicabtagene ciloleucel)	Cell Therapy	Large B-cell lymphoma (LBCL) in the second-line setting	1 in 3,899 people	4/1/2022 \$424,000
Kymriah (tisagenlecleucel)	Cell Therapy	Follicular lymphoma	1 in 16,750 people ¹	5/27/2022 \$399,110
Breyanzi (lisocabtagene maraleucel)	Cell Therapy	Diffuse large B-cell lymphoma in the second setting	1 in 3,899 people	6/24/2022 \$410,300
Zynteglo (betibeglogene autotemcel)	Gene Therapy	Beta-thalassemia	1 in 5,000 people	8/17/2022 \$2.8 million
Skysona (elivaldogene autotemcel)	Gene Therapy	Cerebral adrenoleukodystrophy	1 in 15,000 newborns ²	9/16/2022 \$3 million
Hemgenix (etranacogene dezaparvovec)	Gene Therapy	Hemophilia B	1 in 41,576 people	11/22/2022 \$3.5 million
Adstiladrin (nadofarogene firadenovec-vncg)	Gene Therapy	Bladder cancer	1 in 1,251 people	12/16/2022 \$160-260K³

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

DRUG SPOTLIGHT



Zynteglo

Zynteglo is a gene therapy drug that treats beta-thalassemia, an inherited blood disorder, that causes anemia, delayed growth, and brittle bones.⁴ People born with this condition need regular blood transfusions and may suffer heart failure in their teens or early 20s.

With an estimated price of \$2.8 million, Zynteglo uses the patient's modified blood stem cells to restore hemoglobin production, eliminating the need for blood transfusions.⁵

¹ NORD Rare Disease Database, Follicular Lymphoma, <https://rarediseases.org/rare-diseases/follicular-lymphoma/>

² Adrenoleukodystrophy, Prevalence, <https://adrenoleukodystrophynews.com/prevalence/>

³ Reuters, U.S. FDA approves Ferring Pharma's first gene therapy for bladder cancer, <https://www.reuters.com/world/us/us-fda-approves-ferring-pharmas-first-gene-therapy-bladder-cancer-2022-12-16/>

⁴ Johns Hopkins Medicine, Beta Thalassemia, <https://www.hopkinsmedicine.org/health/conditions-and-diseases/beta-thalassemia>

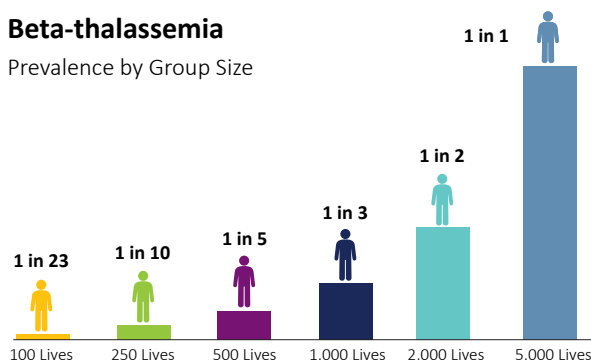
⁵ Zynteglo, A one-time treatment that addresses beta-thalassemia at the genetic level, <https://www.zynteglo.com/how-zynteglo-gene-therapy-works>

Financial Risks for Payers

The likelihood of having a Zynteglo claim increases as the group size grows. For groups of 5,000, there is a 1 in 56 chance of having a covered member with beta-thalassemia qualify to receive Zynteglo.⁶ Smaller groups have a lower, but still very real, chance of having a Zynteglo claim.

Beta-thalassemia

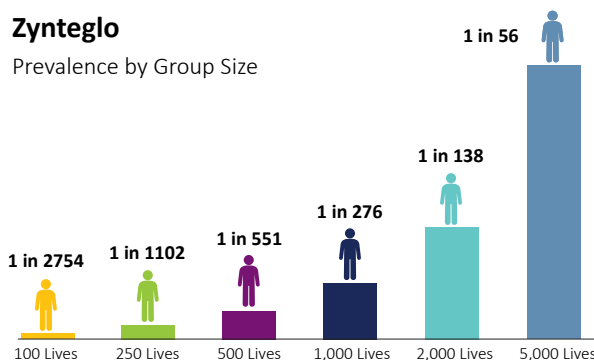
Prevalence by Group Size



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

Zynteglo

Prevalence by Group Size



Likelihood of having a covered member eligible for Zynteglo.

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 2023

Drug Name	Type	Conditions Treated	Prevalence	Expected FDA Approval Date
Roctavian (valoctocogene roxaparvovec)	Gene Therapy	Hemophilia A	1 in 8,315 people	2023
SRP-9001 (delandistrogene moxeparvovec)	Gene Therapy	Duchenne muscular dystrophy	1 in 100,000 people	2023
PTC-AADC⁷ (eladocogene exuparvovec)	Gene Therapy	Aromatic L-amino acid decarboxylase (AADC)	1 to 3 in 100,000 newborns	2023
Libmeldy (atidarsagene autotemcel)	Gene Therapy	Metachromatic leukodystrophy	1 in 363,981 people	2023
RP-L201	Gene Therapy	Leukocyte adhesion deficiency type I	1 in 1,000,000 births ⁸	2023
Lovo-cel (lovotibeglogene autotemcel)	Gene Therapy	Sickle cell disease	1 in 4,226 people	2023
LUMEVOQ (lenadogene nolparvovec)	Gene Therapy	Leber hereditary optic neuropathy	1 in 50,000 people ⁹	2023
EB-101	Gene Therapy	Recessive dystrophic epidermolysis bullosa	1 in 100,000 people	2023
Afami-cel (afamitresgene autoleucel)	Cell Therapy	Synovial sarcoma	1 in 55,476 people	2023
Afami-cel (afamitresgene autoleucel)	Cell Therapy	Myxoid/round cell liposarcoma	1 in 66,667 people	2023
Tab-cel (tabelecleucel)	Cell Therapy	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	1 in 5,280 people	2023
Lifeucel (LN-144)	Cell Therapy	Metastatic melanoma	1 in 1,413 people	2023

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

⁶ Berkley Accident and Health internal data

⁷ NORD Rare Disease Database, Aromatic L-Amino Acid Decarboxylase Deficiency, <https://rarediseases.org/rare-diseases/aromatic-l-amino-acid-decarboxylase-deficiency/>

⁸ ScienceDirect, Molecular Therapy Methods & Clinical Development, <https://www.sciencedirect.com/science/article/pii/S2329050122001073>

⁹ NORD, Leber Hereditary Optic Neuropathy, <https://rarediseases.org/rare-diseases/leber-hereditary-optic-neuropathy/>

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

www.BerkleyAH.com

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