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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.

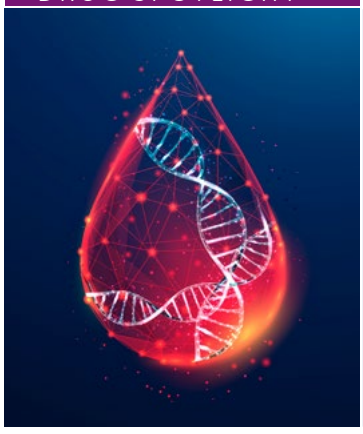
Berkley Accident and Health is actively monitoring the cell and gene therapy market and is pleased to offer pricing solutions along with data analytics to our policyholders through our relationship with Emerging Therapy Solutions (ETS). We collaborate with ETS to bring you these regular cell and gene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

Recently Approved Drugs

Drug Name	Type	Conditions Treated	Prevalence	FDA Approval Date and Est. Price
Omisirge (omidubicel-only)	Cell Therapy	Hematologic malignancies (Blood cancers)	1 in 1,000,000 people	4/17/2023 **$338,000**
Lantidra (donislecel-jujn)	Cell Therapy	Diabetes Type 1	1 in 25,000 people	6/28/2023 **TBD**
Vjuvek (beremagene geperpavec)	Gene Therapy	Dominant and recessive dystrophic epidermolysis bullosa (DDEB and RDEB)	1 in 1,000,000 (DDEB)/ 50,000 (RDEB) people	5/19/2023 **$631,000**
Elevidys (delandistrogene moxeparvovec-rokl)	Gene Therapy	Duchene muscular dystrophy	1 in 100,000 people	6/22/2023 **$3.2 million**
Roctavian (valoctocogene roxaparvovec-rvox)	Gene Therapy	Hemophilia A	1 in 8,316 people	6/29/2023 **$2.9 million**

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

DRUG SPOTLIGHT



Roctavian

On June 29, 2023, BioMarin Pharmaceuticals received FDA approval for Roctavian, a gene therapy for Hemophilia A. Hemophilia A is a genetic disorder caused by a missing or defective Factor VIII, a blood clotting protein.[1] People with Hemophilia A can face a life-threatening emergency if bleeding cannot be stopped following an injury or surgery.[2]

This is the first gene therapy approved to treat Hemophilia A and is administered via a one-time IV infusion. With an estimated price of $2.9 million, Roctavian uses a modified virus vector (AAV5) to deliver a copy of the Factor VIII gene to liver cells, enabling the body to produce its own clotting protein.[3]

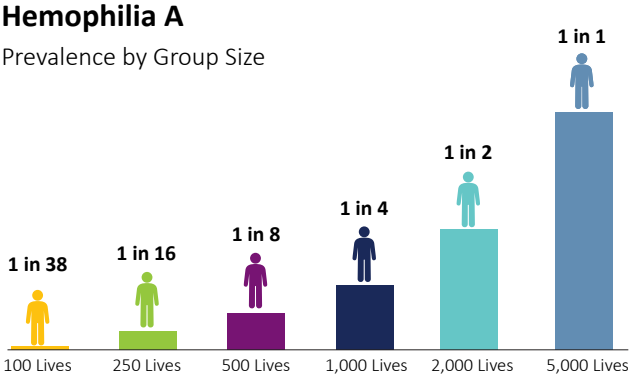
[1]National Bleeding Disorders Foundation (NBDF), ***Hemophilia A***, https://www.hemophilia.org/bleeding-disorders-a-z/types/hemophilia-a
[2]CDC, ***What is Hemophilia?***, https://www.cdc.gov/ncbddd/hemophilia/facts.html
[3]Roctavian, ***What is Roctavian***, https://www.roctavian.com/en-us/

Financial Risks for Payers

The likelihood of having a Roctavian 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 Hemophilia A qualify to receive Roctavian.[4] Smaller groups have a lower, but still very real, chance of having a Roctavian claim.

Hemophilia A

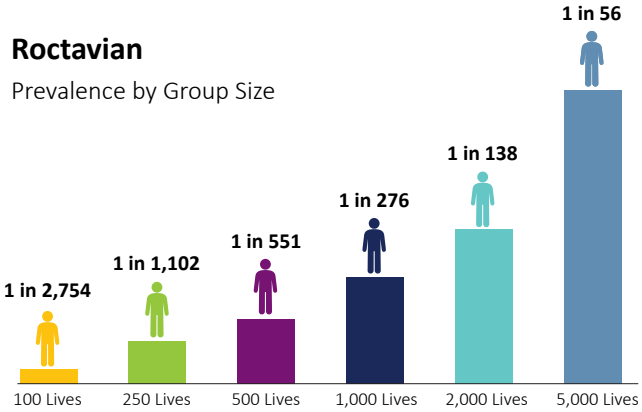
Prevalence by Group Size



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

Roctavian

Prevalence by Group Size



Likelihood of having a covered member eligible for Roctavian.

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 2023-2024

The FDA has stated its intent to accelerate the approval pathway for cell and gene therapies.[5] 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.[6] Here is a snapshot of the growing pipeline of therapies awaiting approval:

Drug Name	Type	Conditions Treated	Prevalence	Expected FDA Approval Date
Lifeucel (LN-144)	Cell Therapy	Metastatic melanoma	1 in 1,413 people	11/25/2023
NurOwn	Cell Therapy	Amyotrophic lateral sclerosis	1 in 13,720 people	2023
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 66,667 people	2024
Obe-cel (Obecabtagene autoleucel)	Cell Therapy	Acute lymphoblastic leukemia	TBD	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 66,667 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 5,848 people	2024-2025

[4]Berkley Accident and Health internal data

[5]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/

[6]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
Remestemcel-L	Cell Therapy	Acute graft-versus-host disease	1 in 54,880 people	2024-2025
Exa-cel (exagamglogene autotemcel)	Gene Therapy	Sickle cell disease	1 in 4,226 people	12/8/2023
Exa-cel (exagamglogene autotemcel)	Gene Therapy	Transfusion-dependent beta-thalassemia	1 in 5,000 people	3/30/2024
Lovo-cel (lovotibeglogene autotemcel)	Gene Therapy	Sickle cell disease	1 in 4,226 people	12/20/2023
RP-L201	Gene Therapy	Leukocyte adhesion deficiency type I	1 in 1,000,000 births[7]	2023
Fidanacogene elaparvovec	Gene Therapy	Hemophilia B	1 in 41,576 people	2024
EB-101	Gene Therapy	Recessive dystrophic epidermolysis bullosa	1 in 100,000 people	2024
RP-L102	Gene Therapy	Fanconi anemia	1 in 725,926 people	2024
Libmeldy (atidarsagene autotemcel)	Gene Therapy	Metachromatic leukodystrophy	1 in 363,981 people	2024
PTC-AADC[8] (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 274,400 people	2024
AAV-RPGR (botaretigene sparoparvovec)	Gene Therapy	X-linked retinitis pigmentosa	1 in 18,294 people	2024
Lumevoq (lenadogene nolparvovec)	Gene Therapy	Leber hereditary optic neuropathy	1 in 50,000 people	2024

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

[7]ScienceDirect, ***Molecular Therapy Methods & Clinical Development***, https://www.sciencedirect.com/science/article/pii/S2329050122001073
[8]NORD Rare Disease Database, ***Aromatic L-Amino Acid Decarboxylase Deficiency***, https://rarediseases.org/rare-diases/aromatic-l-amino-acid-decarboxylase-deficiency/

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

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