Berkley Accident and Health
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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.

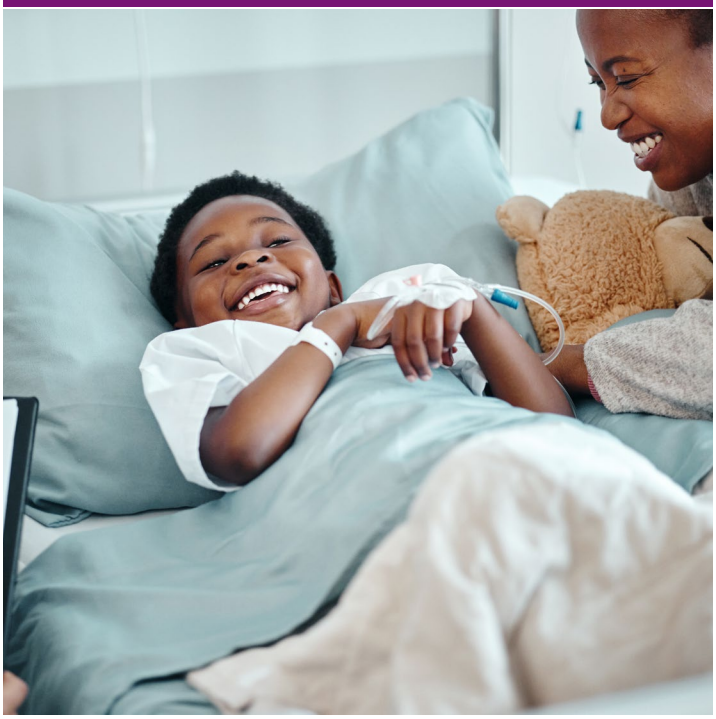
Recently Approved Drugs

Drug Name	Type	Conditions Treated	Prevalence	FDA Approval Date and Est. Price
Breyanzi (lisocabtagene maraleucel)	Cell Therapy	Chronic lymphocytic leukemia Small lymphocytic lymphoma	1 in 25,000 people	3/14/2024 **$487,477**
Abecma – Third Line	Cell Therapy	Multiple myeloma	1 in 5,847 people	4/4/2024 **$498,408**
Carvykti – Second line	Cell Therapy	Multiple myeloma	1 in 5,847 people	4/5/2024 **$522,055**
Breyanzi (lisocabtagene maraleucel)	Cell Therapy	Follicular lymphoma	1 in 25,000 people	5/16/2024 **$487,477**
Breyanzi (lisocabtagene maraleucel)	Cell Therapy	Mantle cell lymphoma	1 in 24,390 people	5/30/2024 **$487,477**
Afami-cel (afamitresgene autoleucel; afami-cel)	Cell Therapy	Synovial sarcoma	1 in 55,476 people	8/1/2024 **$727,000**
Lenmeldy (atidarsagene autotemcel; OTL-200)	Gene Therapy	Metachromatic leukodystrophy	1 in 363,981 people	3/18/2024 **$4.25 million**
Beqvez (fidanacogene elaparvovec-dzkt)	Gene Therapy	Hemophilia B	1 in 41,576 people	4/26/2024 **$3.5 million**
Elevidys (delandistrogene moxeparvovec-rokl)	Gene Therapy	Duchenne muscular dystrophy	1 in 16,667 people	6/20/2024 **$3.2 million**

Cell and gene therapy drugs hold the potential to cure previously incurable diseases, but they often come with a hefty price tag.

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

DRUG SPOTLIGHT



Elevidys

After previously approving its use for a limited population, the FDA approved Elevidys, a gene therapy treatment for Duchenne muscular dystrophy, for a broader range of child patients on June 20, 2024. Elevidys has an estimated price tag of $3.2 million.[1]

Duchenne muscular dystrophy (DMD) is a genetic condition in which a defective gene causes abnormalities in the muscle cells, causing them to break down over time. DMD symptoms usually begin between the ages of 3 and 6 and can include difficulty walking, running, learning disabilities, fatigue, and eventually, life-threatening cardiac and breathing problems.[2] DMD primarily affects boys, but can affect girls in rare cases.

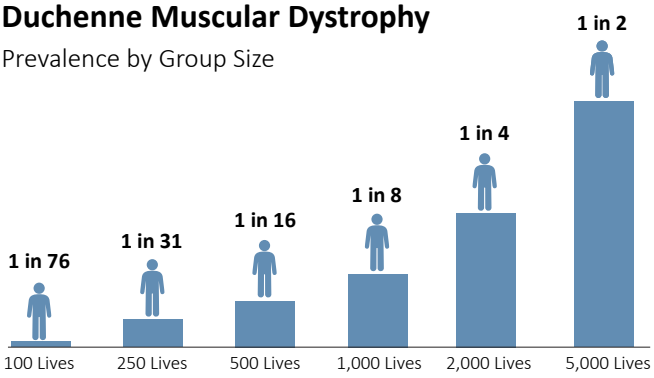
Patients receive Elevidys as a single intravenous (IV) infusion in a hospital or infusion clinic. Early clinical results indicate that children who receive Elevidys can have increased muscle strength and mobility.[3]

Financial Risks for Payers

The likelihood of having an Elevidys claim increases as the group size grows. For groups of 5,000, there is a 1 in 105 chance of having a covered member with Duchenne muscular dystrophy who qualifies to receive Elevidys.[4] Smaller groups have a lower, but still very real, chance of having a Elevidys claim.

Duchenne Muscular Dystrophy

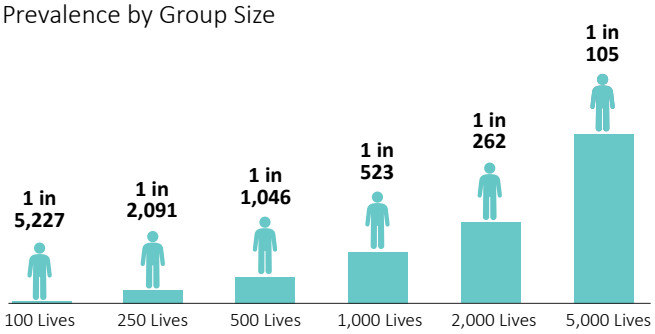
Prevalence by Group Size



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

Elevidys

Prevalence by Group Size



Likelihood of having a covered member eligible for Elevidys.

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 2025-2026

The FDA has stated its intent to accelerate the approval pathway for cell and gene therapies. For the first half of 2024, 7 therapies have already been approved and at this point, there are currently over 500 therapies in different stages of development. Research suggests that, by 2025, the FDA will approve 10 to 20 more gene and cell therapies.[5]

[1] BioPharmaDive, ***Sarepta prices Duchenne gene therapy at $3.2M***, https://www.biopharmadive.com/news/sarepta-duchenne-elevidys-price-million-gene-therapy/653720/

[2] MDA, ***Duchenne Muscular Dystrophy (DMD)***, https://www.mda.org/disease/duchenne-muscular-dystrophy

[3] Boston Children's Hospital, ***Elevidys***, https://www.childrenshospital.org/treatments/elevidys

[4] Berkley Accident and Health internal data

[5] PartnerRe Perspectives, ***The Future is Now: Reimagining the Impact of Gene & Cell Therapies***, https://www.partnerre.com/perspectives/the-future-is-now-reimagining-the-impact-of-gene-cell-therapies/

Expected Approvals for 2025-2026

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
Ryoncil (remestemcel-L)	Cell Therapy	Acute graft-versus-host disease	1 in 55,556 people	1/7/2025
Tab-cel (tabelecleucel)	Cell Therapy	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	1 in 5,280 people	1/15/2025
CAP-1002 (Deramiocel)	Cell Therapy	Duchenne muscular dystrophy	1 in 16,667 people	2025
NurOwn	Cell Therapy	Amyotrophic lateral sclerosis	1 in 11,111 people[6]	2025
Lete-cel (letetresgene autoleucel)	Cell Therapy	Synovial sarcoma	1 in 55,476 people	2026
Lete-cel (letetresgene autoleucel)	Cell Therapy	Myxoid/round cell liposarcoma	1 in 100,000 people	2026
Upstaza (elandocageneexuparvovec: PTC-AADC)	Gene Therapy	Aromatic l-amino acid decarboxylase	1 to 3 in 100,000 newborns[7]	11/13/2024
RP-L102	Gene Therapy	Fanconi anemia	1 in 725,926 people	2024-2025
RGX-121	Gene Therapy	Mucopolysaccharidosis type II	1 in 274,400 people	2025
AAV-RPGR (botaretigene sparoparvovec)	Gene Therapy	X-linked retinitis pigmentosa	1 in 18,293 people	2025
Lumevoq (lenadogene nolparvovec)	Gene Therapy	Leber hereditary optic neuropathy	1 in 50,000 people[8]	2025

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

There are currently over 500 therapies in different stages of development.

[6] Healthline, ***Understanding the Prevalence of ALS***: How Common Is It?, https://www.healthline.com/health/als-prevalence

[7] NORD Rare Disease Database, ***Aromatic L-Amino Acid Decarboxylase Deficiency,*** https://rarediseases.org/rare-diases/aromatic-l-amino-acid-decarboxylase-deficiency/

[8] NORD, ***Leber Hereditary Optic Neuropathy,*** https://rarediseases.org/rare-diseases/leber-hereditary-optic-neuropathy/

Research suggests that, by 2025, the FDA will approve 10 to 20 more gene and cell therapies.

Expected Approvals for 2025-2026 – *continued*

Drug Name	Type	Conditions Treated	Prevalence	Expected FDA Approval Date
giroctocogene fitelparvovec	Gene Therapy	Hemophilia A	1 in 8,315 people	2025
DTX401 (pariglasgenebrecaparvovec)	Gene Therapy	Glycogen storage disease type Ia	1 in 20,000 people	2025
DTX 301 (avalotcagene ontaparvovec)	Gene Therapy	Ornithine transcarbamylase deficiency	1 in 77,000 people[9]	2025
MCO-010 (sonpiretigeneisteparvovec)	Gene Therapy	Retinitis pigmentosa	1 in 100,000 people[10]	2025-2026
RGX-314	Gene Therapy	Wet age-related macular degeneration	1 in 197 people	2025-2026
Zolgensma (onasemnogeneabeparvovec-xioi)	Gene Therapy	Spinal muscular atrophy (expanded indications)	1 in 166,667 people	2025-2026
LYS-SAF302 (olenasufligenerelduparvovec)	Gene Therapy	Mucopolysaccharidosis type IIIa	1 in 100,000 people	2025-2026

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

[9] Children's Hospital of Philadelphia, Ornithine Transcarbamylase Deficiency, https://www.chop.edu/conditions-diseases/ornithine-transcarbamylase-deficiency

[10] Cleveland Clinic, Retinitis Pigmentosa, https://my.clevelandclinic.org/health/diseases/17429-retinitis-pigmentosa

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

www.BerkleyAH.com

BAH AD 2024-253 2/25

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