Approved & Investigational Cell and Gene Therapies Pipeline

Updated July 2024

Emerging Therapy Solutions® (ETS) closely monitors the cell and gene therapy pipeline to identify noteworthy prospective treatments that our clients should be aware of, with a focus on near-term therapies that are estimated to be coming in the next 15 to 24 months. Below is a brief and limited overview of several investigational cell and gene therapy clinical trials that Emerging Therapy Solutions® (ETS) is monitoring in the United States. For combined administrative and therapy (total) cost estimations, gene therapies are expected to range from between $1.5 and $4 million; cell therapies, such as chimeric antigen receptor (CAR) T-cell therapies, are expected to range from $500,000 to $1 million in total cost estimations.

Cell Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Kymriah® (tisagenlecleucel; tisa-cel) Novartis Pharmaceuticals	Acute lymphoblastic leukemia	Multidrug regimen, chimeric antigen receptor (CAR) T-cell therapy	Approved 8/30/2017 $581,895
Yescarta® (axicabtagene ciloleucel; axi-cel) Kite Pharma	Diffuse large B-cell lymphoma Non-Hodgkin lymphoma Follicular lymphoma	Chemotherapy, hematopoietic stem cell transplantation (HSCT), CAR-T therapy	Approved 10/18/2017 $462,000
Kymriah (tisagenlecleucel; tisa-cel) Novartis Pharmaceuticals	Diffuse large B-cell lymphoma Non-Hodgkin lymphoma	Chemotherapy, HSCT, CAR-T therapy	Approved 5/1/2018 $456,941
Tecartus® (brexucabtagene autoleucel; brexu-cel) Kite Pharma	Mantle cell lymphoma Non-Hodgkin lymphoma	Radiation, chemotherapy, autologous HSCT, CAR-T	Approved 7/24/2020 $462,000
Breyanzi® (lisocabtagene maraleucel; liso-cel) Bristol Myers Squibb	Diffuse large B-cell lymphoma Follicular lymphoma Non-Hodgkin lymphoma	Chemotherapy, HSCT, CAR-T therapy	Approved 2/5/2021 $487,477
Abecma® (idecabtagene vicleucel; ide-cel) Bristol Myers Squibb/BBB	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Approved 3/26/2021 $498,408
Tecartus (brexucabtagene autoleucel; brexu-cel) Kite Pharma	Acute lymphoblastic leukemia	Multidrug regimen, CAR-T therapy	Approved 10/1/2021 $462,000
Rethymic® (Allogeneic Processed Thymus Tissue) Enzyvant Therapeutics	Congenital athymia	Strict isolation, prophylactic infection control, immunoglobulins and in some situations, immunosuppression	Approved 10/8/2021 $2,811,385
Carvykti™ (ciltacabtagene autoleucel; cilta-cel) Janssen/Legend Biotech	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Approved 2/28/2022 $522,055
Yescarta (axicabtagene ciloleucel; axi-cel) Kite Pharma	Large B-cell lymphoma in the second-line setting	Chemotherapy, HSCT, CAR-T therapy	Approved 4/1/2022 $462,000
Kymriah (tisagenlecleucel; tisa-cel) Novartis Pharmaceuticals	Follicular lymphoma	Chemotherapy, HSCT, CAR-T therapy	Approved 5/27/2022 $456,941
Breyanzi (lisocabtagene maraleucel) Bristol Myers Squibb	Large B-cell lymphoma in the second-line setting	Chemotherapy, HSCT, CAR-T therapy	Approved 6/24/2022 $487,477
Omisirge® (omidubicel-onlv) Gamida Cell	Hematologic malignancies (Blood cancers)	Umbilical cord blood transplantation (UCBT)	Approved 4/17/2023 $338,000
Lantidra® (donislecel-jujn) CellTrans, Inc.	Diabetes Type 1	Insulin, and/or other injected medicines	Approved 6/28/2023 TBD
Amtagvi™ (lifileucel) Iovance Biotherapeutics	Metastatic melanoma	Surgical excision, removal of affected lymph nodes, chemotherapy, radiation, checkpoint inhibitor immunotherapy or targeted therapy drugs, TIL therapy	Approved 2/16/2024 $515,000
Breyanzi (lisocabtagene maraleucel) Bristol Myers Squibb	Chronic lymphocytic leukemia Small lymphocytic lymphoma	Targeted therapy, chemotherapy, HSCT, CAR-T therapy	Approved 3/14/2024 $487,477
Abecma – Third line Bristol Myers Squibb/BBB	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Approved 4/4/2024 $498,408
Carvykti – Second line Janssen/Legend Biotech	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Approved 4/5/2024 $522,055
Breyanzi (lisocabtagene maraleucel) Bristol Myers Squibb	Follicular lymphoma	Chemotherapy, HSCT	Approved 5/16/2024 $487,477
Breyanzi (lisocabtagene maraleucel) Bristol Myers Squibb	Mantle cell lymphoma	Chemotherapy, HSCT	Approved 5/30/2024 $487,477
Afami-cel (afamitresgene autoleucel; ADP-A2M4) Adaptimmune Therapeutics	Synovial sarcoma	Surgical excision, chemotherapy, radiation therapy	PDUFA 8/4/2024
Obe-cel (Obecabtagene autoleucel) Autolus Therapeutics	Acute lymphoblastic leukemia	Multidrug regimen, CAR-T therapy	PDUFA 11/16/2024
Ryoncil™ (remestemcel-L) Mesoblast Limited	Acute graft-versus-host disease	Immunosuppressive prevention; corticosteroid	PDUFA 1/7/2025
Tab-cel® (tabelecleucel; ATA129/EBV-CTL) Atara Biotherapeutics	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	Immunotherapy, rituximab, radiation & antiviral therapy, immunochemotherapy	PDUFA 1/15/2025
CAP-1002 (Deramiocel) Capricor Therapeutics	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, I*n-vivo* gene therapy; otherwise, medical management	2025
NurOwn® BrainStorm Cell Therapeutics	Amyotrophic lateral sclerosis	Supportive therapies	2025
Lete-cel (letetresgene autoleucel) Adaptimmune Therapeutics	Myxoid/round cell liposarcoma	Surgical excision, chemotherapy, radiation therapy	2026
Lete-cel (letetresgene autoleucel) Adaptimmune Therapeutics	Synovial sarcoma	Surgical excision, chemotherapy, radiation therapy	2026
Lifileucel (LN-144) Iovance Biotherapeutics	Cervical cancer	Surgery, radiation, chemotherapy	Delayed
Zevor-cel (zevorcabtagene autoleucel, CT053) CARsgen Therapeutics	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Delayed
Afami-cel (afamitresgene autoleucel; ADP-A2M4) Adaptimmune Therapeutics	Myxoid/round cell liposarcoma	Surgical excision, chemotherapy, radiation therapy	Delayed

Approved & Investigational Cell and Gene Therapies Pipeline

Updated July 2024

Gene Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Luxturna® *in-vivo* (voretigene neparvovec-rzyl) Spark Therapeutics	Biallelic *RPE65* mutation	*In-vivo* gene therapy	Approved 12/2017 $456,875 per eye
Zolgensma® *in-vivo* (onasemnogene abeparvovec) Novartis Pharmaceuticals	Spinal muscular atrophy	Spinraza® (nusinersen), Evrysdi® (risdiplam), *In-vivo* gene therapy	Approved 05/2019 $2,322,044
Zynteglo® *ex-vivo* (betibeglogene autotemcel; beti-cel) bluebird bio	Transfusion-dependent beta-thalassemia	Chronic blood transfusions, HSCT, chelation therapy, *Ex-vivo* gene therapy	Approved 8/17/2022 $2,800,000
Skysona™ *ex-vivo* (elivaldogene autotemcel; eli-cel) bluebird bio	Cerebral adrenoleukodystrophy	HSCT, *Ex-vivo* gene therapy	Approved 9/16/2022 $3,000,000
Hemgenix® *in-vivo* (etranacogne dezaparvovec) uniQure / CSL Behring	Hemophilia B	Factor replacement (prophylaxis) therapy, *In-vivo* gene therapy	Approved 11/22/2022 $3,500,000
Adstiladrin® *in-vivo* (nadofaragene firadenovec-vncg) Ferring Pharmaceuticals	Bladder cancer	Transurethral resection of bladder tumor (TURBT), intravesical immunotherapy, radiation/chemotherapy, gene therapy	Approved 12/16/2022 $60,000 per instillation
Vyjuvek™ *Topical* (beremagene geperpavec; B-VEC; KB103) Krystal Biotech	Dominant and recessive dystrophic epidermolysis bullosa	Wound care. †Expected avg annual cost/patient after induction & based on cost of $24,735/vial for 26 weeks; $900,000 commercial member cap with terms.	Approved 5/19/2023 $643,110†
Elevidys® *in-vivo* (delandistrogene moxeparvovec-rokl) Sarepta Therapeutics	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, *In-vivo* gene therapy, otherwise treatment is symptomatic	Approved 6/22/2023 $3,200,000
Roctavian™ *in-vivo* (valoctocogene roxaparvovec-rvox) BioMarin Pharmaceutical Inc.	Hemophilia A	Factor replacement (prophylaxis) therapy, *In-vivo* gene therapy	Approved 6/29/2023 $2,900,000
Casgevy™ *ex-vivo* (exagamglogene autotemcel; exa-cel) CRISPR/Vertex	Sickle cell disease	HSCT, disease-modifying agents, *Ex-vivo* gene therapy	Approved 12/8/2023 $2,200,000
Lyfgenia™ *ex-vivo* (lovotibeglogene autotemcel; lovo-cel) bluebird bio	Sickle cell disease	HSCT, disease-modifying agents, *Ex-vivo* gene therapy	Approved 12/8/2023 $3,100,000
Casgevy™ *ex-vivo* (exagamglogene autotemcel; exa-cel) CRISPR/Vertex	Transfusion-dependent beta-thalassemia	Chronic blood transfusions, HSCT, chelation therapy, *Ex-vivo* gene therapy	Approved 1/16/2024 $2,200,000
Lenmeldy® *ex-vivo* (atidarsagene autotemcel; OTL-200) Orchard Therapeutics	Metachromatic leukodystrophy	HSCT, *Ex-vivo* gene therapy	Approved 3/18/2024 $4,250,000
Beqvez™ *in-vivo* (fidanacogene elaparvovec-dzkt) Pfizer, Inc.	Hemophilia B	Factor replacement (prophylaxis) therapy, *In-vivo* gene therapy	Approved 4/26/2024 $3,500,000
Elevidys® *in-vivo* (delandistrogene moxeparvovec-rokl) Sarepta Therapeutics (Expanded Indication)	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, *In-vivo* gene therapy, otherwise treatment is symptomatic	Approved 6/20/2024 $3,200,000
Upstaza™ *in-vivo* (elandocagene exuparvovec: PTC-AADC) PTC Therapeutics	Aromatic l-amino acid decarboxylase	Limited to none (Treating symptoms)	PDUFA 11/13/2024
RP-L102 *ex-vivo* Rocket Pharmaceuticals	Fanconi anemia	HSCT, oxymetholone	2024 - 2025
RGX-121 *in-vivo* REGENEXBIO	Mucopolysaccharidosis type II	Limited to none (Treating symptoms)	2025
botaretigene sparoparvovec *in-vivo* (AAV-RPGR) MeiraGTx/Janssen Pharmaceuticals, Inc. laruparetigene zosaparvovec *in vivo* (rAAV2tYF-GRK1-RPGR), Beacon Therapeutics	X-linked retinitis pigmentosa	Limited to none (Treating symptoms)	2025
Lumevoq® *in-vivo* (lenadogene nolparvovec; GS010) GenSight Biologics	Leber hereditary optic neuropathy	Limited to none (Treating symptoms)	2025
giroctocogene fitelparvovec *in-vivo* Pfizer and Sangamo Therapeutics	Hemophilia A	Factor replacement (prophylaxis) therapy, *In-vivo* gene therapy	2025
fordadistrogene movaparvovec *in-vivo* (PF-06939926) Pfizer, Inc.	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, I*n-vivo* gene therapy; otherwise, medical management	2025
DTX401 *in-vivo* (pariglasgene brecaparvovec) Ultragenyx Pharmaceuticals	Glycogen storage disease type Ia	Glucose replacement therapy, strict dietary modifications, and medical management of secondary chronic diseases	2025
DTX301 *in-vivo* (avalotcagene ontaparvovec) Ultragenyx Pharmaceutical	Ornithine transcarbamylase deficiency	Dietary restrictions, liver transplantation	2025
sonpiretigene isteparvovec *in-vivo* (MCO-010) Nanoscope Therapeutics Inc.	Retinitis pigmentosa	Limited to none (Treating symptoms)	2025
RGX-314 *in-vivo* REGENEXBIO	Wet age-related macular degeneration	Intravitreal injection of vascular endothelial growth factor (VEGF), photodynamic therapy, vitamin/mineral formula	2025 - 2026
Zolgensma® *in-vivo* (onasemnogene abeparvovec-xioi) Novartis Pharmaceuticals	Spinal muscular atrophy (expanded indications)	Spinraza® (nusinersen), Evrysdi® (risdiplam)	2025 - 2026
olenasufligene relduparvovec *in-vivo* (LYS-SAF302) Lysogene UX111 *in vivo* (fka ABO-102) Ultragenyx Pharmaceuticals	Mucopolysaccharidosis type IIIa	Limited to none (Treating symptoms)	2025 - 2026
Kresladi™ *ex-vivo* (marnetegragene autotemcel; RP-L201) Rocket Pharmaceuticals	Leukocyte adhesion deficiency type I	HSCT, antibiotic therapy	Delayed
Pz-cel *keratinocyte sheets* (prademagene zamikeracel; EB-101) Abeona Therapeutics	Recessive dystrophic epidermolysis bullosa	Wound care, Vyjuvek *topical* gene therapy	Delayed

*Current Treatment here is only a general categorization of generally published and known alternatives and does not reflect that every individual situation will vary. **FDA Advisory Panel scheduled.