

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 \$503,580
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 \$531,350
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 \$528,312
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 \$503,580
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 \$531,350
Omisirge® (omidubicel-onyl) Gamida Cell	Hematologic malignancies (Blood cancers)	Umbilical cord blood transplantation (UCBT)	Approved 4/17/2023 \$512,070
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 \$531,350
Abecma – Third line Bristol Myers Squibb/BBB	Multiple myeloma	Chemotherapy, HSCT, surgery, radiation or combination of these options, CAR-T	Approved 4/4/2024 \$528,312
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 \$531,350
Breyanzi (lisocabtagene maraleucel) Bristol Myers Squibb	Mantle cell lymphoma	Chemotherapy, HSCT	Approved 5/30/2024 \$531,350
Tecelra® (afamitresgene autoleucel; afami-cel) Adaptimmune Therapeutics	Synovial sarcoma	Surgical excision, chemotherapy, radiation therapy, T-cell therapy	Approved 8/1/2024 \$727,000
Aucatzyl® (obecabtagene autoleucel; obe-cel) Autolus Therapeutics	Acute lymphoblastic leukemia	Multidrug regimen, CAR-T therapy	Approved 11/08/2024 \$525,000
Ryoncil™ (remestemcel-L-rknd) Mesoblast Limited	Acute graft-versus-host disease	Immunosuppressive prevention; corticosteroid; **\$194,000 per intravenous infusion, with estimate shown for full treatment course.	Approved 12/18/2024 \$1,550,000**

Cell Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
CAP-1002 (deramiocel™) Capricor Therapeutics	Duchenne muscular dystrophy cardiomyopathy	Medications include Amondys 45, Exondys 51, and Vyondys 53, In-vivo gene therapy; otherwise, medical management	PDUFA 8/31/2025
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
CORDStrom™ INmune Bio	Recessive dystrophic epidermolysis bullosa	Wound care	2026
Ebvallo™ (tabelecleucel; ATA129/EBV-CTL/Tab-cel) Atara Biotherapeutics	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	Immunotherapy, rituximab, radiation & antiviral therapy, immunochemotherapy	PDUFA Delayed
NurOwn® BrainStorm Cell Therapeutics	Amyotrophic lateral sclerosis	Supportive therapies	Delayed

Gene Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Luxturna® <i>in vivo</i> (voretigene neparvovec-rzyl) Spark Therapeutics	Biallelic <i>RPE65</i> mutation	<i>In-vivo</i> gene therapy	Approved 12/2017 \$456,875 per eye
Zolgensma® <i>in vivo</i> (onasemnogene abeparvovec) Novartis Pharmaceuticals	Spinal muscular atrophy	Spinraza® (nusinersen), Evrysdi® (risdiplam), <i>In-vivo</i> gene therapy	Approved 05/2019 \$2,391,706
Zynteglo® <i>ex vivo</i> (betibeglogene autotemcel; beti-cel) bluebird bio	Transfusion-dependent beta-thalassemia	Chronic blood transfusions, HSCT, chelation therapy, <i>Ex-vivo</i> gene therapy	Approved 8/17/2022 \$2,800,000
Skysona™ <i>ex vivo</i> (elivaldogene autotemcel; eli-cel) bluebird bio	Cerebral adrenoleukodystrophy	HSCT, <i>Ex-vivo</i> gene therapy	Approved 9/16/2022 \$3,000,000
Hemgenix® <i>in vivo</i> (etranacogene dezaparvovec) uniQure / CSL Behring	Hemophilia B	Factor replacement (prophylaxis) therapy, <i>In-vivo</i> gene therapy	Approved 11/22/2022 \$3,500,000
Adstiladrin® <i>in vivo</i> (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 \$25,230/vial for 26 weeks; \$900,000 commercial member cap with terms.	Approved 5/19/2023 \$655,980†
Elevidys® <i>in vivo</i> (delandistrogene moxeparvovec-rokl) Sarepta Therapeutics	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, <i>In-vivo</i> gene therapy, otherwise treatment is symptomatic	Approved 6/22/2023 \$3,200,000
Roctavian™ <i>in vivo</i> (valoctocogene roxaparvovec-rvox) BioMarin Pharmaceutical Inc.	Hemophilia A	Factor replacement (prophylaxis) therapy, <i>In-vivo</i> gene therapy	Approved 6/29/2023 \$2,900,000
Casgevy™ <i>ex vivo</i> (exagamglogene autotemcel; exa-cel) CRISPR/Vertex	Sickle cell disease	HSCT, disease-modifying agents, <i>Ex-vivo</i> gene therapy	Approved 12/8/2023 \$2,200,000
Lyfgenia™ <i>ex vivo</i> (lovotibeglogene autotemcel; lovo-cel) bluebird bio	Sickle cell disease	HSCT, disease-modifying agents, <i>Ex-vivo</i> gene therapy	Approved 12/8/2023 \$3,100,000
Casgevy™ <i>ex vivo</i> (exagamglogene autotemcel; exa-cel) CRISPR/Vertex	Transfusion-dependent beta-thalassemia	Chronic blood transfusions, HSCT, chelation therapy, <i>Ex-vivo</i> gene therapy	Approved 1/16/2024 \$2,200,000
Lenmeldy® <i>ex vivo</i> (atidarsagene autotemcel; OTL-200) Orchard Therapeutics	Metachromatic leukodystrophy	HSCT, <i>Ex-vivo</i> gene therapy	Approved 3/18/2024 \$4,250,000
Beqvez™ <i>in vivo</i> (fidanacogene elaparvovec-dzkt) Pfizer, Inc.	Hemophilia B	Factor replacement (prophylaxis) therapy, <i>In-vivo</i> gene therapy	Approved 4/26/2024 \$3,500,000 Discontinued 2/21/2025
Elevidys® <i>in vivo</i> (delandistrogene moxeparvovec-rokl) Sarepta Therapeutics (Expanded Indication)	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, <i>In-vivo</i> gene therapy, otherwise treatment is symptomatic	Approved 6/20/2024 \$3,200,000
Kebilidi™ <i>in vivo</i> (elandocagene exuparvovec; PTC-AADC) PTC Therapeutics	Aromatic L-amino acid decarboxylase	Limited to none (Treating symptoms), gene therapy	Approved 11/13/2024 \$3,950,000

Gene Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Pz-cel <i>keratinocyte sheets</i> (prademagene zamikeracel; EB-101) Abeona Therapeutics	Recessive dystrophic epidermolysis bullosa	Wound care, Vyjuvek <i>topical gene therapy</i>	PDUFA 4/29/2025
UX111 <i>in vivo</i> (fka ABO-102) Ultragenyx Pharmaceuticals	Mucopolysaccharidosis type IIIa	Limited to none (Treating symptoms)	PDUFA 8/18/2025
RGX-121 <i>in vivo</i> REGENXBIO	Mucopolysaccharidosis type II	Limited to none (Treating symptoms)	Q3 2025
RP-L102 <i>ex vivo</i> Rocket Pharmaceuticals	Fanconi anemia	HSCT, oxymetholone	2025
Kresladi™ <i>ex vivo</i> (marnetegrane autotemcel; RP-L201) Rocket Pharmaceuticals	Leukocyte adhesion deficiency type I	HSCT, antibiotic therapy	2025
MCO-010 <i>in vivo</i> (sonpiretigene isteparvovec) Nanoscope Therapeutics Inc.	Retinitis pigmentosa	Limited to none (Treating symptoms)	2025
Zolgensma® <i>in vivo</i> (onasemnogene abeparvovec-xioi) Novartis Pharmaceuticals	Spinal muscular atrophy (expanded indications)	Spinraza® (nusinersen), Evrysdi® (risdiplam)	2025
DTX301 <i>in vivo</i> (avalotcagene ontaparvovec) Ultragenyx Pharmaceutical	Ornithine transcarbamylase deficiency	Dietary restrictions, liver transplantation	2025 - 2026
DTX401 <i>in vivo</i> (pariglasgene breccaparvovec) Ultragenyx Pharmaceuticals	Glycogen storage disease type Ia	Glucose replacement therapy, strict dietary modifications, and medical management of secondary chronic diseases	Q1 2026
ABBV-RGX-314 <i>in vivo, subretinal</i> REGENXBIO	Wet age-related macular degeneration	Intravitreal injection of vascular endothelial growth factor (VEGF), photodynamic therapy, vitamin/mineral formula	H1 2026
giroctocogene fitelparvovec <i>in-vivo</i> Sangamo Therapeutics	Hemophilia A	Factor replacement (prophylaxis) therapy, <i>In-vivo gene therapy</i>	2026
ST-920 <i>in vivo</i> (isaralgagene civaparvovec) Sangamo Therapeutics	Fabry disease	Enzyme replacement therapy	2026
cretostimogene grenadenorepvec <i>in vivo</i> CG Oncology	Bladder cancer	Transurethral resection of bladder tumor (TURBT), intravesical immunotherapy, radiation/chemotherapy, gene therapy	2026
detalimogene enGene	BCG-unresponsive nonmuscle invasive bladder cancer (NMIBC) with carcinoma in situ		
RGX-202 <i>in vivo</i> RegenexBio	Duchenne muscular dystrophy	Medications include Amondys 45, Exondys 51, and Vyondys 53, <i>In-vivo gene therapy</i> , otherwise treatment is symptomatic	2026
NTLA-2002 <i>in vivo</i> Intellia Therapeutics, Inc.	Hereditary angioedema	Long- and short-term prophylaxis; life-long therapies, which may require chronic intravenous or subcutaneous administration.	2026
AAV-RPGR <i>in vivo</i> (botaretigene sparaparvovec) MeiraGTx/Janssen Pharmaceuticals, Inc.	X-linked retinitis pigmentosa	Limited to none (Treating symptoms)	Delayed
rAAV2tyf-GRK1-RPGR <i>in vivo</i> (laruparetigene zosaparvovec) Beacon Therapeutics			

*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.

NOTE: This content is informational only and is intended for a US audience. This document has been prepared by Emerging Therapy Solutions (ETS) and provides information about prospective cell and gene therapy treatments as of the date of this document. The information provided has been obtained from third-party sources we believe to be reliable, however we may not be able to verify accuracy and make no guarantee, warranty, or representation about this information. Due to the rapidly evolving and changing nature of the content of this document, the information presented, including opinions and estimates, is subject to change without notice and may become quickly outdated. The information presented is not intended to be a recommendation as to medical care, or any form of legal or medical advice. Emerging Therapy Solutions is a trademark of Emerging Therapy Solutions, Inc. All other trademarks referenced herein are the property of their respective owners.