

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 chemotherapy, targeted therapy, immunotherapy, allogeneic hematopoietic stem cell transplantation (HSCT), chimeric antigen receptor (CAR) T-cell therapy	Approved 8/30/2017 \$605,404
	Diffuse large B-cell lymphoma Non-Hodgkin lymphoma		Approved 5/1/2018 \$523,152
	Follicular lymphoma	Chemoimmunotherapy, targeted therapy, radiation, HSCT, CAR-T therapy	Approved 5/27/2022
Yescarta® (axicabtagene ciloleucel; axi-cel) Kite Pharma	Diffuse large B-cell lymphoma Non-Hodgkin lymphoma		Approved 10/18/2017 \$548,900
	Follicular lymphoma		Approved 3/5/2021
	Large B-cell lymphoma in the second-line setting	Chemoimmunotherapy, targeted therapy, radiation, HSCT, CAR-T therapy	Approved 4/1/2022
	Central nervous system (CNS) lymphoma		Approved 2/6/2026
Tecartus® (brexucabtagene autoleucel; brexu-cel) Kite Pharma	Mantle cell lymphoma Non-Hodgkin lymphoma	Chemoimmunotherapy, targeted therapy, radiation, autologous HSCT, CAR-T therapy	Approved 7/24/2020 \$503,580
	Acute lymphoblastic leukemia	Multidrug chemotherapy, targeted therapy, immunotherapy, allogeneic HSCT, CAR-T therapy	Approved 10/1/2021
Breyanzi® (lisocabtagene maraleucel) Bristol Myers Squibb	Diffuse large B-cell lymphoma Non-Hodgkin lymphoma	Chemoimmunotherapy, targeted therapy, radiation, HSCT, CAR-T therapy	Approved 2/5/2021 \$557,918
	Follicular lymphoma		
	Large B-cell lymphoma in the second-line setting	Chemotherapy, immunotherapy, radiation, autologous HSCT, CAR-T therapy	Approved 6/24/2022
	Chronic lymphocytic leukemia/Small lymphocytic lymphoma	Targeted therapy, chemotherapy, HSCT, CAR-T therapy	Approved 3/14/2024
	Follicular lymphoma	Chemoimmunotherapy, targeted therapy, radiation, HSCT, CAR-T therapy	Approved 5/16/2024
	Mantle cell lymphoma	Chemoimmunotherapy, targeted therapy, radiation, autologous HSCT, CAR-T therapy	Approved 5/30/2024
	Marginal zone lymphoma	Radiation, surgery, immunotherapy, targeted therapy, chemotherapy, and HSCT	Approved 12/4/2025
Rethymic® (Allogeneic Processed Thymus Tissue-agdc) Enzyvant Therapeutics	Congenital athymia	Strict isolation, prophylactic infection control, immunoglobulins, and in some situations, immunosuppression	Approved 10/8/2021 \$2,923,840
Carvykti™ (ciltacabtagene autoleucel; cilta-cel) Janssen/Legend Biotech	Multiple myeloma	Multidrug regimen (chemotherapy, immunomodulators, proteasome inhibitors, monoclonal antibodies), autologous HSCT, radiation, CAR-T therapy	Approved 2/28/2022 \$571,970
	Multiple myeloma in the second-line setting		Approved 4/5/2024

Cell Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Abecma® (idecabtagene vicleucel; ide-cel) Bristol Myers Squibb	Multiple myeloma	Multidrug regimen (chemotherapy, immunomodulators, proteasome inhibitors, monoclonal antibodies), autologous HSCT, radiation, CAR-T therapy	Approved 3/26/2021 \$544,162
	Multiple myeloma in third line setting		Approved 4/4/2024
Omisirge® (omidubicel-only) Gamida Cell	Hematological malignancies	Varies by cancer type, chemotherapy, targeted therapy, immunotherapy, radiation, HSCT, CAR-T	Approved 4/17/2023 \$693,900
	Severe aplastic anemia	Allogeneic HSCT, immunosuppressive therapy, blood transfusions	Approved 12/05/2025
Amtagvi™ (lifileucel) Iovance Biotherapeutics	Metastatic melanoma	Surgery, chemotherapy, radiation, checkpoint inhibitor immunotherapy, targeted therapy drugs, tumor-infiltrating lymphocyte (TIL) therapy	Approved 2/16/2024 \$612,580
Ryoncil™ (remestemcel-L-rknd) Mesoblast Limited	Acute graft-versus- host disease	Corticosteroids, ruxolitinib, and other immunosuppressants	Approved 12/18/2024 \$1,550,000 (\$194,000 per intravenous infusion; estimate is for the initial treatment course. Additional dosing may be indicated depending on response.)
Tecelra® (afamitresgene autoleucel; afami-cel) US WorldMeds	Synovial sarcoma	Surgery, chemotherapy, radiation therapy, T-cell therapy	Approved 8/1/2024 \$781,525 Expanded indication approved 6/17/2026
Aucatzyl® (obecabtagene autoleucel; obe-cel) Autolus Therapeutics	Acute lymphoblastic leukemia	Multidrug chemotherapy, targeted therapy, immunotherapy, allogeneic HSCT, CAR-T therapy	Approved 11/08/2024 \$572,250
Tregzi™ Orca Bio	Hematologic malignancies	Varies by cancer type, chemotherapy, targeted therapy, immunotherapy, radiation, HSCT, CAR-T	Approved 6/30/2026 \$428,000
Deramiciocel™ (CAP-1002) Capricor Therapeutics	Duchenne muscular dystrophy	Corticosteroids, <i>in vivo</i> gene therapy, heart medications, implantable cardiac devices, supportive and symptomatic treatments	PDUFA 11/22/2026
Anito-cel (anitocabtagene autoleucel) Arcellx	Multiple myeloma	Multidrug regimen (chemotherapy, immunomodulators, proteasome inhibitors, monoclonal antibodies), autologous HSCT, radiation, CAR-T therapy	PDUFA 12/23/2026
Mivocabtagene autoleucel (miv-cel, KYV-101) Kyverna Therapeutics	Stiff person syndrome	Medications, physical and occupational therapies, immunotherapies	H1 2027
Letetresgene autoleucel (lete-cel) US WorldMeds	Myxoid/round cell liposarcoma	Surgery, chemotherapy, radiation therapy	H2 2027
Letetresgene autoleucel (lete-cel) US WorldMeds	Synovial sarcoma	Surgery, chemotherapy, radiation therapy	H2 2027
Ebstrocel™ (CORDStrom™) INmune Bio	Recessive dystrophic epidermolysis bullosa	Wound care, topical gene therapy and gene-modified cell sheets	H2 2027

Cell Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Soficabtagene geuleucel (sofi-cel; WU-CART-007) Allotera Therapeutics	T cell acute lymphoblastic leukemia / lymphoblastic lymphoma	Multidrug chemotherapy, targeted therapy, immunotherapy, allogeneic HSCT	H2 2027
Lifileucel Iovance Biotherapeutics	Non-small cell lung cancer	Surgery, chemotherapy, radiation therapy, targeted therapy, and immunotherapy	H2 2027
Rondecabtagene autoleucel (ronde- cel) Lyell Immunopharma	Large B-cell lymphoma	Chemoimmunotherapy, targeted therapy, radiation, HSCT, CAR-T therapy	H2 2027
Anzutresgene autoleucel (anzu-cel, IMA203) Immatics Biotechnologies	Metastatic melanoma	Surgery, chemotherapy, radiation, checkpoint inhibitor immunotherapy, targeted therapy drugs, tumor-infiltrating lymphocyte (TIL) therapy	H2 2027
Zimislecel (VX-880) Vertex Pharmaceuticals	Diabetes type 1	Insulin, and/or other injected medicines	2027
Revascor® (rexlemestrocel-L) Mesoblast Limited	End-stage ischemic heart failure with LVAD	Medications, device therapies, LVAD, heart transplant	H1 2028
NXC-201 Immix Biopharma (Nexcella Inc)	Light chain amyloidosis (AL)	Autologous HSCT; chemotherapy, immunomodulators, monoclonal antibodies	H1 2028
Resecabtagene autoleucel (rese-cel; CABA-201) Cabaletta Bio	Idiopathic inflammatory myopathy (myositis)	High-dose steroids, immunosuppressants, IV immunoglobulin	H1 2028
Rexlemestrocel-L (Revascor) Mesoblast Limited	Chronic low back pain	Physical therapy, pain medications, steroid injections, and surgery	H1 2028
Laromestrocel (lomecel-B™) Longeveron	Hypoplastic left heart syndrome	Open-heart surgery, heart transplantation	H1 2028
Descartes-08 Cartesian Therapeutics	Myasthenia gravis	AChE inhibitor, immunosuppressants, IVIG, plasma exchange, biologics, thymus removal	H1 2028
Tabelecleucel (tab-cel, Ebvallo™) Atara Biotherapeutics/Pierre Fabre	Epstein-Barr virus-associated post-transplant lymphoproliferative disease	Immunotherapy, rituximab, radiation and antiviral therapy, immunochemotherapy	Delayed
NurOwn® (debamestrocel) BrainStorm Cell Therapeutics	Amyotrophic lateral sclerosis	Supportive therapies	Delayed

Gene Therapies

Therapy & Manufacturer	Condition	Current Treatment*	Actual / Anticipated Approval Date
Luxturna® (voretigene neparvovec-rzyl) <i>in vivo</i> Spark Therapeutics / Roche	Biallelic <i>RPE65</i> mutation	<i>in vivo</i> gene therapy	Approved 12/2017 \$456,875 per eye
Zolgensma® (onasemnogene abeparvovec-xioi) <i>in vivo</i> ; intravenous (IV) Novartis Pharmaceuticals	Spinal muscular atrophy	Spinraza® (nusinersen), Evrysdi® (risdiplam), Zolgensma	Approved 05/2019 \$2,715,961
Zynteglo® (betibeglogene autotemcel; beti-cel) <i>ex vivo</i> Genetix Biotherapeutics	Transfusion-dependent beta-thalassemia	Chronic blood transfusions, HSCT, chelation therapy	Approved 8/17/2022 \$2,800,000
Skysona™ (elivaldogene autotemcel; eli-cel) <i>ex vivo</i> Genetix Biotherapeutics	Cerebral adreno-leukodystrophy	Allogeneic HSCT	Approved 9/16/2022 \$3,000,000
Hemgenix® (etranacogne dezaparvovec) <i>in vivo</i> uniQure / CSL Behring	Hemophilia B	Factor replacement (prophylaxis) therapy	Approved 11/22/2022 \$3,500,000
Adstiladrin® (nadofarogene firadenovec-vncg) <i>in vivo</i> ; intravesical instillation Ferring Pharmaceuticals	BCG-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ	Transurethral resection of bladder tumor (TURBT), intravesical immunotherapy, radiation/chemotherapy, gene therapy	Approved 12/16/2022 \$61,814 per instillation
Vyjuvek™ (beremagene geperpavec) <i>topical</i> Krystal Biotech	Dystrophic epidermolysis bullosa	Wound care, birch bark extract, gene-modified cell sheets (see Zevaskyn, for recessive dystrophic epidermolysis bullosa only)	Approved 5/19/2023 \$655,980 (Expected avg annual cost/patient after induction & based on cost of \$26,000/vial for 26 weeks)
Elevidys® (delandistrogene moxeparvovec-rokl) <i>in vivo</i> Sarepta Therapeutics	Duchenne muscular dystrophy	Corticosteroids, exon-skipping medications, histone modulator, supportive and symptomatic treatments	Approved 6/22/2023 \$3,200,000 Expanded indication approved 6/20/2024
Casgevy™ (exagamglogene autotemcel; exa-cel) <i>ex vivo</i> CRISPR/Vertex	Sickle cell disease	Allogeneic HSCT, disease-modifying agents,	Approved 12/8/2023 \$2,365,000
	Transfusion-dependent beta-thalassemia	chronic blood transfusions, allogeneic HSCT, chelation therapy	Approved 1/16/2024 \$2,365,000 Expanded indication approved 7/1/2026
Lyfgenia™ (lovotibeglogene autotemcel; lovo-cel) <i>ex vivo</i> Genetix Biotherapeutics	Sickle cell disease	Allogeneic HSCT, disease-modifying agents	Approved 12/8/2023 \$3,100,000
Lenmeldy® (atidarsagene autotemcel) <i>ex vivo</i> Orchard Therapeutics	Metachromatic leukodystrophy	Allogeneic HSCT	Approved 3/18/2024 \$4,250,000
Kebilidi™ (elandocagene exuparvovec) <i>in vivo</i> PTC Therapeutics	Aromatic L-amino acid decarboxylase	Limited to none (treating symptoms)	Approved 11/13/2024 \$3,950,000
Encelto™ (revakinagene taroretcel-lwey) <i>allogeneic encapsulated cell-based</i> Neurotech Pharmaceuticals	Macular telangiectasia type 2	Anti-VEGF, steroid injections, and laser therapy	Approved 03/5/2025 \$250,000 per eye
Zevaskyn™ (prademagene zamikeracel) <i>gene-modified cell sheets</i> Abeona Therapeutics	Recessive dystrophic epidermolysis bullosa	Wound care, birch bark extract, topical gene therapy	Approved 4/29/2025 \$3,147,000
Papzimeos™ (zopapogene imadenovec-drab) <i>immunotherapy</i> Precigen Inc.	Recurrent respiratory papillomatosis	Surgery, antivirals, immunotherapy, monoclonal antibodies	Approved 08/2025 \$115,000 per single-use vial
Itvisma® (onasemnogene abeparvovec-brve) <i>in vivo</i> ; intrathecal injection Novartis Pharmaceuticals	Spinal muscular atrophy	Spinraza® (nusinersen), Evrysdi® (risdiplam), Zolgensma	Approved 11/24/2025 \$2,664,229
Waskyra® (etuvetidigene autotemcel) <i>ex vivo</i> Fondazione Telethon	Wiskott-Aldrich syndrome	Allogeneic HSCT, supportive care	Approved 12/9/2025 \$3,250,000
Kresladi™ (marnetegragene autotemcel) <i>ex vivo</i> Rocket Pharmaceuticals	Severe leukocyte adhesion deficiency type I	Allogeneic HSCT, supportive care	Approved 3/26/2026 \$ TBD

Gene Therapies

THERAPY & MANUFACTURER	CONDITION	CURRENT TREATMENT*	ACTUAL / ANTICIPATED APPROVAL DATE
Otarmeni™ (lunsotogene parvec-cwba) <i>in vivo</i> Regeneron Pharmaceuticals	<i>Otoferlin (OTOF)</i> gene-related hearing loss	Cochlear implantations	Approved 4/23/2026 No cost for the therapy to clinically eligible patients in US
Genglycos™ (pariglasgene breca-parvovec) <i>in vivo</i> Ultragenyx Pharmaceuticals	Glycogen storage disease type Ia	Glucose replacement therapy, dietary modifications, and medical management of secondary chronic diseases	Approved 8/19/2026 \$2,700,000
UX111 (ABO-102) <i>in vivo</i> Ultragenyx Pharmaceuticals	Mucopolysaccharidosis type IIIa	Limited to none (treating symptoms)	PDUFA 9/19/2026
INO-3107 <i>immunotherapy</i> Inovio Pharmaceuticals	Recurrent respiratory papillomatosis	Surgery, antivirals, immunotherapy, monoclonal antibodies, Papzimeos	PDUFA 10/30/2026
Lonvoguran ziclumeran (lonvo-z; NTLA-2002) <i>in vivo</i> Intellia Therapeutics	Hereditary angioedema	Long- and short-term prophylaxis; life-long therapies, which may require chronic intravenous or subcutaneous administration	PDUFA 3/10/2027
Isaralgagene civaparvovec (ST-920) <i>in vivo</i> PTC Therapeutics	Fabry disease	Enzyme replacement therapy	H1 2027
Sonpiretigene Isteparvovec (MCO-010) <i>in vivo</i> Nanoscope Therapeutics	Retinitis pigmentosa	Limited to none (treating symptoms)	H1 2027
OCU400 <i>in vivo</i> Ocugen			H2 2027
SGT-003 <i>In vivo</i> Solid Biosciences	Duchenne muscular dystrophy	Corticosteroids, exon-skipping medications, histone modulator, <i>in vivo</i> gene therapy, supportive and symptomatic treatments	H1 2027
AMT-130 <i>in vivo</i> uniQure	Huntington's disease	Symptom management and supportive care	H1 2027
ProstAtak® (aglatimagene besadenovec, CAN-2409) <i>Immunotherapy</i> Candel Therapeutics	Prostate cancer	surgery, radiation, focal therapy, hormone therapy, chemotherapy, targeted therapy, immunotherapy, radiopharmaceuticals	H1 2027
Cretostimogene grenadenorepvec (CG0070) <i>in vivo</i> ; intravesical instillation CG Oncology	BCG-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ	Transurethral resection of bladder tumor (TURBT), intravesical immunotherapy, radiation/chemotherapy, gene therapy	H1 2027
Detalimogene voraplasmid (EG-70) <i>in vivo</i> intravesical instillation enGene			H2 2027
TSHA-102 <i>In vivo</i> Taysha	Rett syndrome	Medications, supportive care, and rehabilitative therapy	H2 2027
Ristoglogene autogetemcel (risto-cel; BEAM-101) <i>Ex vivo</i> Beam Therapeutics	Sickle cell disease	Allogeneic HSCT, disease-modifying agents	H2 2027
KB803 (Vyjuvek, beremagene geperpavec) <i>Topical</i> Krystal Biotech	Dystrophic epidermolysis bullosa ocular complications	Supportive care and surgery	H2 2027
Surabgene lomparvovec (sura-vec; ABBV-RGX-314) <i>in vivo</i> ; subretinal REGENXBIO	Wet age-related macular degeneration	Intravitreal injection of vascular endothelial growth factor (VEGF), photodynamic therapy, vitamin/mineral formula	2027

Gene Therapies

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Botaretigene sparaparvovec (bota-vec) <i>in vivo</i> MeiraGTx	X-linked retinitis pigmentosa	Limited to none (treating symptoms)	2027
Laruparetigene zosaparvovec (laru-zova) <i>in vivo</i> Beacon Therapeutics			2027
RGX-202 <i>in vivo</i> REGENXBIO	Duchenne muscular dystrophy	Corticosteroids, exon-skipping medications, histone modulator, supportive and symptomatic treatments	H2 2027
OCU410ST <i>in vivo</i> Ocugen, Inc.	Stargardt disease	Vision rehabilitation, lifestyle changes, and low vision services	Q4 2027
AAV2-hAQP1 <i>in vivo</i> MeiraGTx	Radiation-induced xerostomia	Supportive care and symptom management	Q4 2027
Bidridistrogene xeboparvovec (SRP-9003) <i>in vivo</i> Sarepta Therapeutics	Limb-girdle muscular dystrophy, type 2E/R4	Rehabilitative therapy, multidisciplinary treatment	H1 2028
Avalotcagene ontaparvovec (DTX301) <i>in vivo</i> UltraGenyx Pharmaceutical	Ornithine transcarbamylase deficiency	Dietary restrictions, liver transplantation	H1 2028
Clemidsogene lanparvovec (RGX-121) <i>in vivo</i> REGENXBIO	Mucopolysaccharidosis type II	Limited to none (treating symptoms)	H1 2028
PM359 <i>Ex vivo</i> Prime Medicine	Chronic granulomatous disease	Immunomodulators, corticosteroids, allogeneic HSCT	H1 2028
LX2006 <i>In vivo</i> Lexeo Therapeutics	Friedreich's ataxia cardiomyopathy	Supportive care and symptom management	H2 2028
Elevidys® (delandistrogene moxeparvovec-rokl; SRP-9001) <i>in vivo</i> Sarepta Therapeutics	Duchenne muscular dystrophy (expand to non-ambulatory)	Corticosteroids, exon-skipping medications, histone modulator, supportive and symptomatic treatments	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. 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.