Cell and Gene Therapies

With U.S. Food and Drug Administration (FDA) Approval

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Cell therapy is the transfer of live cells into a patient to lessen or cure a disease using cells from the patient or a donor. **Gene therapy** is used to treat or cure a disease by replacing a missing or mutated gene in the targeted cell to "correct" the missing function. Below is a brief introduction to cell and gene therapies currently approved by the FDA and available in the United States. For complete indications, safety, and packaging information, visit the manufacturer's website. List pricing is based on current known therapy cost from publicly available information and does not include administration or treatment costs. Learn more at 877.445.4822.

Gene Therapies

Adstiladrin® (nadofaragene firadenovec-vncg) Condition: Bladder cancer Company: Ferring Pharmaceuticals Approved: December 2022 Current WAC*: $60,000 per instillation More: ferring.com	**Treats bladder cancer in adults** Adstiladrin is a novel adenovirus vector-based *in-vivo* gene therapy from Ferring Pharmaceuticals for the treatment of adult patients with high-risk Bacillus Calmette Guerin (BCG)-unresponsive non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors. This is the first gene therapy approved to treat bladder cancer.
Beqvez™ (fidanacogene elaparvovec-dzkt) Condition: Hemophilia B Company: Pfizer Inc. Approved: April 2024 Current WAC: $3,500,000 More: pfizer.com	**Treats moderate to severe hemophilia B in adults** Beqvez is an adeno-associated virus vector-based gene therapy for adults with moderate to severe hemophilia B who currently use factor IX (FIX) prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes and do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test. Beqvez is designed to enable people living with hemophilia B to produce FIX themselves rather than the current standard of care.
Casgevy™ (exagamglogene autotemcel) Conditions: Sickle cell disease, Beta-thalassemia Company: CRISPR Therapeutics and Vertex Pharmaceuticals Approved: December-23, January-24 Current WAC: $2,200,000 (SCD), $2,200,000 (TDT) More: vrtx.com	**Treats sickle cell disease and transfusion-dependent beta-thalassemia in patients aged 12 years and older** Casgevy is an autologous genome edited hematopoietic stem cell-based gene therapy indicated for the treatment of patients aged 12 years and older with sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs) or with transfusion-dependent beta-thalassemia (TDT) who need regular blood transfusions. It was the first-ever approved therapy using CRISPR/Cas9 gene-editing technology.
Elevidys® (delandistrogene moxeparvovec-rokl) Condition: Duchenne muscular dystrophy Company: Sarepta Therapeutics Approved: June 2023, June 2024 Current WAC: $3,200,000 More: sarepta.com	**Treats Duchenne muscular dystrophy patients at least 4 years old** Elevidys is an adeno-associated virus-based gene therapy that was originally approved in June 2023 for the treatment of ambulatory DMD pediatric patients aged 4 through 5 years with a confirmed mutation in the *DMD* gene. This indication was approved under accelerated approval based on expression of Elevidys micro-dystrophin observed in patients treated with Elevidys with continued approval contingent upon confirmatory trial(s). Elevidys is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the DMD gene. In June 2024, the FDA approved an expansion to the label indication for Elevidys to include individuals with DMD with a confirmed mutation in the *DMD* gene who are at least 4 years of age. Confirming the functional benefits, the FDA granted traditional approval for ambulatory patients. The FDA granted accelerated approval for non-ambulatory patients. Continued approval for non-ambulatory DMD patients may be contingent upon verification of clinical benefit in a confirmatory trial. Elevidys remains contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.
Hemgenix® (etranacogene dezaparvovec-drlb) Condition: Hemophilia B Company: CSL Behring Approved: November 2022 Current WAC: $3,500,000 More: cslbehring.com	**Treats hemophilia B in adults** Hemgenix, an adeno-associated virus vector-based gene therapy for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who currently use Factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes is the first in-vivo gene therapy approved by the United States (US) Food and Drug Administration (FDA) for treating hemophilia B in adults and uses an Adeno-Associated Virus Type 5 (AAV5) vector.

**WAC: Wholesale Acquisition Cost based on current data available from sources deemed to be reliable.*

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Kebilidi™ (elandocagene exuparvovec; PTC-AADC) Condition: Aromatic l-amino acid decarboxylase deficiency Company: PTC Therapeutics Approved: November 2024 Current WAC: N/A More: ptcbio.com	**Treats aromatic L–amino acid decarboxylase (AADC) deficiency in children and adults** Kebilidi is a gene therapy for the treatment of children and adult patients with aromatic L–amino acid decarboxylase (AADC) deficiency, including the full spectrum of disease severity. Kebilidi is the first-ever gene therapy approved in the United States that is directly administered to the brain. Kebilidi was approved by the FDA using its accelerated approval pathway with continued approval possibly contingent upon verification and description of clinical benefit in a confirmatory clinical trial, which is ongoing to verify Kebilidi's clinical benefit.
Lenmeldy™ (atidarsagene autotemcel) Condition: Metachromatic leukodystrophy Company: Orchard Therapeutics Approved: March 2024 Current WAC: $4,250,000 More: orchard-tx.com	**Treats metachromatic leukodystrophy in children** Lenmeldy is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD).
Luxturna® (voretigene neparvovec-rzyl) Condition: Biallelic RPE65 mutation Company: Spark Therapeutics Approved: December 2017 Current WAC: $456,875/eye More: luxturna.com	**Treats biallelic *RPE65* mutation associated retinal dystrophy** Luxturna is an adeno-associated virus vector-based gene therapy. It is the first *in-vivo* gene therapy approved by the FDA. It was approved in 2017 for patients with confirmed biallelic *RPE65* gene mutations. Luxturna is approved for patients over the age of 12 months. The indication also requires that patients must have some level of vision, which is determined through evidence of viable retinal cells.
Lyfgenia™ (lovotibeglogene autotemcel) Condition: Sickle cell disease Company: bluebird bio, Inc. Approved: December 2023 Current WAC: $3,100,000 More: bluebirdbio.com	**Treats sickle cell disease in patients aged 12 years and older** Lyfgenia is a one-time ex-vivo lentiviral vector gene therapy approved for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events (VOEs). Lyfgenia works by adding a functional β-globin gene to patients' own hematopoietic (blood) stem cells (HSCs).
Roctavian™ (valoctocogene roxaparvovec-rvox) Condition: Hemophilia A Company: BioMarin Pharmaceutical, Inc. Approved: June 2023 Current WAC: $2,900,000 More: biomarin.com	**Treats severe hemophilia A in adults** Roctavian is a gene therapy for the treatment of adults with severe hemophilia A (congenital factor VIII (FVIII) deficiency with FVIII activity < 1 IU/dL) without antibodies to adeno-associated virus serotype 5 (AAV5) detected by an FDA-approved test. The company's biologics license application (BLA) is supported by data from the phase III GENEr8-1 trial (NCT03370913). This is the first gene therapy approved to treat hemophilia A.0
Skysona® (elivaldogene autotemcel) Condition: Cerebral adrenoleukodystrophy Company: bluebird bio, Inc. Approved: September 2022 Current WAC: $3,000,000 More: bluebirdbio.com	**Treats cerebral adrenoleukodystrophy (CALD) in boys aged 4 to 17** Skysona is a gene therapy from bluebird bio, Inc. for the treatment of boys aged 4 to 17 with early, active cerebral adrenoleukodystrophy (CALD). Skysona is the first ex-vivo lentiviral vector gene therapy approved in the US for treating CALD.

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Vyjuvek™ (beremagene geperpavec-svdt) Condition: Dystrophic epidermolysis bullosa Company: Krystal Biotech Approved: May 2023 Current WAC: $24,735 per vial More: krystalbio.com	**Treats wounds in patients six months of age and older with dystrophic epidermolysis bullosa** Vyjuvek is a herpes-simplex virus type 1 (HSV-1) vector-based gene therapy from Krystal Biotech for the treatment of wounds in patients six months of age and older with dystrophic epidermolysis bullosa (DEB) with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene. Vyjuvek is the first gene therapy in the US for treating DEB. The redosable, topical gel is applied directly to skin wounds.
Zolgensma® (onasemnogene abeparvovec-xioi) Condition: Spinal muscular atrophy Company: Novartis Pharmaceuticals Approved: May 2019 Current WAC: $2,322,044 More: zolgensma.com	**Treats spinal muscular atrophy (SMA) in children under age two with biallelic mutations in the SMN1 gene** Zolgensma is an adeno-associated virus vector-based in-vivo gene therapy indicated for pediatric patients less than two years of age with spinal muscular atrophy with biallelic mutations in the survival motor neuron 1 (SMN1) gene.
Zynteglo® (betibeglogene autotemcel) Condition: Beta-thalassemia Company: bluebird bio, Inc. Approved: August 2022 Current WAC: $2,800,000 More: zynteglo.com	**Treats transfusion-dependent beta-thalassemia (TDT) in adult and pediatric patients who require regular red blood cell transfusions** Zynteglo is a vector-based gene therapy approved by the FDA for the treatment of adult and pediatric patients with transfusion-dependent beta-thalassemia who require regular red blood cell transfusions. Zynteglo is the first ex-vivo lentiviral vector gene therapy approved in the United States (US) for treating beta-thalassemia.

Cell Therapies

Abecma® (idecabtagene vicleucel) Condition: Multiple myeloma Company: Bristol Myers Squibb / 2seventybio Approved: March 2021, April 2024 Current WAC: $498,408 ($528,312 - 1/1/25) More: abecma.com	**Treats adult patients with relapsed or refractory (r/r) multiple myeloma** Abecma is a B-cell maturation antigen (BCMA)-directed chimeric antigen receptor (CAR) T-cell therapy. In April 2024, an expanded indication for Abecma received FDA approval for adults with r/r multiple myeloma after two or more prior lines of therapy including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody. Abecma was initially approved by the FDA in March 2021 for the treatment of adult patients with r/r multiple myeloma after four or more prior lines of therapy.
Amtagvi™ (lifileucel) Condition: Metastatic melanoma Company: Iovance Biotherapeutics Approved: February 2024 Current WAC: $515,000 More: amtagvi.com	**Treats adult patients with unresectable or metastatic melanoma** Amtagvi™ is a tumor-derived autologous T-cell therapy indicated for the treatment of adult patients with unresectable or metastatic melanoma previously treated with a PD-1 blocking antibody, and if BRAF V600 mutation positive, a BRAF inhibitor with or without a MEK inhibitor. Amtagvi is a tumor-infiltrating lymphocyte (TIL) cell therapy and is the first and only one-time, individualized T-cell therapy approved for solid tumor cancer. This indication is approved under an accelerated approval based on overall response rate (ORR) and duration of response. Iovance is also conducting TILVANCE-301, a phase III clinical trial to confirm clinical benefit.

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Aucatzyl® (obecabtagene autoleucel) Condition: Acute lymphoblastic leukemia Company: Autolus Therapeutics Approved: November 2024 Current WAC: $525,000 More: aucatzylhcp.com	**Treats adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (r/r B-ALL)** AUCATZYL® (obecabtagene autoleucel), a chimeric antigen receptor (CAR) T-cell therapy for the treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (r/r B-ALL). Approval was based on results from the FELIX clinical trial of obe-cel in adults with r/r B-ALL. Aucatzyl is the first CAR-T therapy approved by the US Food and Drug Administration (FDA) with no requirement for a Risk Evaluation Mitigation Strategy (REMS) program.
Breyanzi® (lisocabtagene maraleucel) Condition: Large B-cell lymphoma, diffuse large B-cell lymphoma, follicular lymphoma, and chronic lymphocytic leukemia / small lymphocytic lymphoma, r/r mantle cell lymphoma Company: Bristol Myers Squibb Approved: February 2021, June 2022, March 2024, May 2024 Current WAC: $487,477 ($531,350 - 1/1/25) More: breyanzi.com	**Treats adult patients with r/r large B-cell lymphoma (LBCL) including diffuse large B-cell lymphoma (DLBCL), r/r follicular lymphoma, chronic lymphocytic leukemia (CLL) / small lymphocytic lymphoma (SLL), & r/r mantle cell lymphoma** Breyanzi, is a CD19-directed CAR-T therapy indicated for adult patients with r/r LBCL, including DLBCL not otherwise specified (including DLBCL arising from indolent lymphoma), high-grade B-cell lymphoma, primary mediastinal large B-cell lymphoma, and follicular lymphoma grade 3B. In June 2022, Breyanzi was approved for an expanded indication to include those who have refractory disease to first-line chemoimmunotherapy or relapse within 12 months of first-line chemoimmunotherapy; or refractory disease to first-line chemoimmunotherapy or relapse after first-line chemoimmunotherapy and are not eligible for hematopoietic stem cell transplantation (HSCT) due to comorbidities or age. In March 2024, Breyanzi was approved for another expanded indication to include adult patients with r/r CLL or SLL who have received at least two prior lines of therapy, including a Bruton tyrosine kinase (BTK) inhibitor and a B-cell lymphoma 2 (BCL-2) inhibitor. In May 2024, Breyanzi was approved for adult patients with r/r follicular lymphoma who have received two or more prior lines of systemic therapy. Both expanded indications received accelerated approval based on response rate and duration of response and may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). Breyanzi was also approved in May 2024 for adults with r/r mantle cell lymphoma (MCL) who have received at least two prior lines of systemic therapy, including a Bruton tyrosine kinase (BTK) inhibitor.
Carvykti™ (ciltacabtagene autoleucel) Condition: Multiple myeloma Company: Janssen Pharmaceutical/Legend Biotech Approved: February 2022, April 2024 Current WAC: $522,055 More: carvykti.com	**Treats adult patients with r/r multiple myeloma** Carvykti is a B-cell maturation antigen (BCMA)-directed CAR T-cell therapy. In April 2024, Carvykti was approved by the FDA for adults with r/r multiple myeloma after at least one prior line of therapy, including a proteasome inhibitor and an immunomodulatory agent, and are refractory to lenalidomide, making it the first and only B-cell maturation antigen (BCMA)-targeted therapy approved for the treatment of patients with multiple myeloma as early as first relapse. In February 2022, Carvykti was initially approved for adult patients with r/r multiple myeloma after four or more prior lines of therapy.
Kymriah® (tisagenlecleucel) Condition: Acute lymphoblastic leukemia, large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Novartis Pharmaceuticals Approved: August 2017, May 2018, May 2022 Current WAC: $581,895 (ALL), $456,941 (DLBCL, FL) More: kymriah.com	**Treats patients up to age 25 with r/r B-cell precursor acute lymphoblastic leukemia (ALL) and adult patients with r/r large B-cell lymphoma including DLBCL and r/r follicular lymphoma** Kymriah is a CAR T-cell therapy approved for patients up to 25 years of age with B-cell precursor ALL that is refractory or in second or later relapse. In 2018, Kymriah was approved for an expanded indication to include adult patients with r/r large B-cell lymphoma after two or more lines of systemic therapy. In May 2022, Kymriah was approved for another expanded indication for adult patients with r/r follicular lymphoma after two or more lines of systemic therapy. This expansion was approved under an accelerated approval; continued approval for this indication may be contingent upon clinical benefit in a confirmatory trial.

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Lantidra® (donislecel-jujn) Condition: Diabetes Type 1 Company: CellTrans Inc. Approved: June 2023 Current WAC: N/A More: celltransinc.com	**Treats adults with Diabetes Type 1** Lantidra is approved for the treatment of adults with type 1 diabetes who are unable to approach target glycated hemoglobin (average blood glucose levels) because of current repeated episodes of severe hypoglycemia (low blood sugar) despite intensive diabetes management and education. It is the first allogeneic (donor) pancreatic islet cellular therapy made from deceased donor pancreatic cells for the treatment of type 1 diabetes.
Omisirge® (omidubicel-onlv) Condition: Umbilical cord-blood transplant for blood cancers Company: Gamida Cell Approved: April 2023 Current WAC: $338,000 ($512,070 - 1/31/25) More: gamida-cell.com	**For patients requiring umbilical cord blood transplantation for blood cancer treatment** Omisirge is a substantially modified allogeneic (donor) cord blood-based cell therapy intended for use in adults and pediatric patients 12 years and older to quicken the recovery of neutrophils (a subset of white blood cells) in the body and reduce the risk of infection for individuals with blood cancers planned for umbilical cord blood transplantation following a myeloablative conditioning regimen (treatment such as radiation or chemotherapy). Related conditions include acute lymphoblastic leukemia, acute myeloid leukemia, myelodysplastic syndromes, and others.
Ryoncil® (remestemcel-L-rknd) Condition: Steroid-refractory acute graft-versus-host disease Company: Mesoblast Limited Approved: December 2024 Current WAC: N/A More: ryoncil.com	**For pediatric patients with steroid-refractory acute graft-versus-host disease (SR-aGvHD)** Ryoncil is an allogeneic bone marrow-derived mesenchymal stromal cell (MSC) therapy for pediatric patients aged two months and older with steroid-refractory acute graft-versus-host disease (SR-aGvHD). Ryoncil is the first FDA-approved MSC therapy.
Tecartus® (brexucabtagene autoleucel) Condition: Acute lymphoblastic leukemia Company: Kite, a Gilead Company Approved: July 2020 (MCL), October 2021 (ALL) Current WAC: $462,000 More: tecartus.com	**Treats adult patients with r/r B-cell precursor acute lymphoblastic leukemia (ALL) and adult patients with r/r mantle cell lymphoma** Tecartus is a CAR-T therapy indicated for the treatment of adult patients with r/r B-cell precursor ALL. Tecartus is also indicated for the treatment of adult patients with r/r mantle cell lymphoma. This was approved under an accelerated approval; continued approval for this indication may be contingent upon clinical benefit in a confirmatory trial.
Tecelra® (afamitresgene autoleucel) Condition: Synovial sarcoma Company: Adaptimmune Therapeutics Approved: August 2024 Current WAC: $727,000 More: tecelra.com	**Treats adult patients with advanced MAGE-A4+ synovial sarcoma** Tecelra is a T-cell receptor (TCR) therapy for the treatment of advanced MAGE-A4+ synovial sarcoma in adults with certain HLA types who have received prior chemotherapy. Tecelra is a one-time infusion and is the first approved engineered T-cell therapy for solid tumors. Tecelra is specifically indicated "for the treatment of adults with unresectable or metastatic synovial sarcoma who have received prior chemotherapy, are HLA-A*02:01P, -A*02:02P, -A*02:03P, or -A*02:06P positive and whose tumor expresses the MAGE-A4 antigen as determined by FDA-approved or cleared companion diagnostic devices."

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Yescarta® (axicabtagene ciloleucel) Condition: Large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Kite, a Gilead Company Approved: October 2017, April 2021, April 2022 Current WAC: $503,580 More: yescarta.com	**Treats adult patients with r/r large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL) and r/r follicular lymphoma** Yescarta is a CAR T-cell therapy that is indicated for the treatment of adult patients with r/r large B-cell lymphoma after two or more lines of systemic therapy, including DLBCL not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma. In 2021, Yescarta was approved for an expanded indication to include adults with r/r follicular lymphoma after two or more lines of systemic therapy. In April 2022, Yescarta was approved for another expanded indication for adult patients with large B-cell lymphoma that is refractory to first-line chemoimmunotherapy or that relapses within 12 months of first-line chemoimmunotherapy. It is not indicated for the treatment of patients with primary central nervous system lymphoma.

Allogeneic Processed Thymus Tissue

Rethymic® (allogeneic processed thymus tissue–agdc) Condition: Congenital athymia Company: Enzyvant Therapeutics Approved: October 2021 Current WAC: $2,811,385 More: rethymic.com	**Treats congenital athymia in children** Rethymic is an allogeneic processed thymus tissue product indicated for immune reconstitution in pediatric patients with congenital athymia. Rethymic is a one-time therapy that is surgically implanted into the quadriceps while the patient is under general anesthesia.