Cell and Gene Therapies
With US Food and Drug Administration (FDA) Approval

Cell and Gene Therapies offer new treatment options for patients and providers.

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 list price: 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.
Hemgenix® (etranacogene dezaparvovec-drlb) Condition: Hemophilia B Company: CSL Behring Approved: November 2022 Current list price: $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.
Luxturna® (voretigene neparvovec-rzyl) Condition: Biallelic *RPE65* mutation Company: Spark Therapeutics Approved: December 2017 Current list price: $425,000/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.
Skysona® (elivaldogene autotemcel) Condition: Cerebral adrenoleukodystrophy Company: bluebird bio, Inc. Approved: September 2022 Current list price: $3,000,000 More: bluebirdbio.com	**Treats cerebral adrenoleukodystrophy (CALD) in boys 4 to 17 years of age** 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.
Zolgensma® (onasemnogene abeparvovec-xioi) Condition: Spinal muscular atrophy Company: Novartis Approved: May 2019 Current list price: $2,125,000 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 list price: $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.

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Cell Therapies

Abecma® (idecabtagene vicleucel) Condition: Multiple myeloma Company: Bristol Myers Squibb Approved: March 2021 Current List Price: $457,255 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. Abecma is approved for adult patients with r/r multiple myeloma after four or more prior lines of therapy including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody.
Breyanzi® (lisocabtagene maraleucel) Condition: Large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Bristol Myers Squibb Approved: February 2021, June 2022 Current List Price: $447,227 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** Breyanzi, 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. Expanded indication is for 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. It is not indicated for the patients with primary central nervous system lymphoma.
Carvykti™ (ciltacabtagene autoleucel) Condition: Multiple myeloma Company: Janssen Pharmaceutical/Legend Biotech Approved: February 2022 Current List Price: $465,000 More: carvykti.com	**Treats adult patients with r/r multiple myeloma** Carvykti is a B-cell maturation antigen (BCMA)-directed CAR T-cell therapy. Carvykti is approved for adult patients with r/r multiple myeloma after four or more prior lines of therapy including an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody.
Kymriah® (tisagenlecleucel) Condition: Acute lymphoblastic leukemia, large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Novartis Approved: August 2017, May 2018, May 2022 Current list price: $508,250 (ALL), $399,110 (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.
Tecartus® (brexucabtagene autoleucel) Condition: Acute lymphoblastic leukemia Company: Kite, a Gilead Company Approved: July 2020 (MCL), October 2021 (ALL) Current List Price: $399,000 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.
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 List Price: $399,000 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.