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 and limited introduction to the cell and gene therapies currently approved by the FDA and available in the United States. For complete indications, safety, and packaging information, please visit the manufacturer's website. List pricing is based on the current known therapy cost from publicly available information and does not include administration or treatment costs.

Contact Emerging Therapy Solutions® (ETS) to learn more about these therapies: 877.445.4822

Gene Therapies	**Luxturna®** (voretigene neparvovec-rzyl) Condition: Biallelic *RPE65* mutation Company: Spark Therapeutics Approved: December 2017 Current list price: $425,000 per eye More: luxturna.com	**Treats biallelic *RPE65* mutation associated retinal dystrophy** Luxturna is an adeno-associated virus vector-based gene therapy. It was approved by the U.S. Food and Drug Administration (FDA) 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.
	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 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.
Cell Therapies	**Abecma®** (idecabtagene vicleucel) Condition: Multiple myeloma Company: Bristol Myers Squibb /bluebird bio Approved: March 2021 Current List Price: $419,500 More: abecma.com	**Treats adult patients with r/r multiple myeloma** Abecma, previously called ide-cel, is a B-cell maturation antigen (BCMA)-directed chimeric antigen receptor (CAR) T-cell therapy. Abecma is approved for adult patients with relapsed or refractory (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 Current List Price: $410,300 More: breyanzi.com	**Treats adult patients with r/r (relapsed or refractory) large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL) & r/r follicular lymphoma** Breyanzi is approved for adult patients with relapsed or refractory (r/r) large B-cell lymphoma, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified (and including DLBCL arising from indolent lymphoma); high-grade B-cell lymphoma; primary mediastinal large B-cell lymphoma; and follicular lymphoma grade 3B after two or more lines of systemic therapy.
	Carvykti™ (ciltacabtagene autoleucel) Condition: Multiple myeloma Company: Janssen Pharmaceutical/Legend Biotech Approved: February 2022 Current List Price: $465,000 More: fda.gov/media/156560/download	**Treats adult patients with r/r multiple myeloma** Carvykti, previously called cilta-cel, is a B-cell maturation antigen (BCMA)-directed chimeric antigen receptor (CAR) T-cell therapy. Carvykti is approved for adult patients with relapsed or refractory (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.

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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 acute lymphoblastic leukemia and adult patients with r/r large B-cell lymphoma including DLBCL and r/r relapsed or refractory follicular lymphoma** Kymriah is a chimeric antigen receptor (CAR) T-cell therapy. Kymriah is approved for patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse. In 2018, Kymriah was approved for an expanded indication to include adult patients with relapsed or refractory (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: October 2021 Current List Price: $399,000 More: tecartus.com	**Treats adult patients with r/r B-cell precursor acute lymphoblastic leukemia (ALL)** Tecartus is a CAR-T therapy indicated for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia.
Tecartus® (brexucabtagene autoleucel) Condition: Mantle cell lymphoma Company: Kite, a Gilead Company Approved: July 2020 Current List Price: $399,000 More: tecartus.com	**Treats adult patients with r/r mantle cell lymphoma** Tecartus is a CAR-T therapy indicated for the treatment of adult patients with relapsed or refractory 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 relapsed or refractory (r/r) large B-cell lymphoma after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (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.

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