

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 <i>RPE65</i> mutation Company: Spark Therapeutics Approved: December 2017 Current list price: \$425,000 per eye More: luxturna.com	Treats biallelic <i>RPE65</i> mutation associated retinal dystrophy Luxturna is an adeno-associated virus vector-based gene therapy. It is the first <i>in-vivo</i> gene therapy approved by the U.S. Food and Drug Administration (FDA). It was approved in 2017 for patients with confirmed biallelic <i>RPE65</i> 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 <i>SMN1</i> gene Zolgensma is an adeno-associated virus vector-based <i>in-vivo</i> 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 (<i>SMN1</i>) 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 <i>ex-vivo</i> lenti-viral vector gene therapy approved in the United States (US) for treating beta-thalassemia.
	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 from 4 to 17 years of age SKYSONA (elivaldogene autotemcel; eli-cel) 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 <i>ex-vivo</i> lenti-viral vector gene therapy approved in the US for treating CALD.
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 relapsed or refractory (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.

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Cell Therapies	<p>Breyanzi[®] (lisocabtagene maraleucel) Condition: Large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Bristol Myers Squibb Approved: February 2021, June 2022 Current List Price: \$410,300 More: breyanzi.com</p>	<p>Treats adult patients with r/r large B-cell lymphoma (LBCL) including diffuse large B-cell lymphoma (DLBCL) & r/r follicular lymphoma</p> <p>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. 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.</p>
	<p>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</p>	<p>Treats adult patients with r/r multiple myeloma</p> <p>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.</p>
	<p>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</p>	<p>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 follicular lymphoma</p> <p>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.</p>
	<p>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</p>	<p>Treats adult patients with r/r B-cell precursor acute lymphoblastic leukemia (ALL) and adult patients with r/r mantle cell lymphoma</p> <p>Tecartus is a CAR-T therapy indicated for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia. Tecartus is also 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.</p>
	<p>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</p>	<p>Treats adult patients with r/r large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL) and r/r follicular lymphoma</p> <p>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.</p>

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