Approved Cell and Gene Therapies

Cell and Gene Therapies offer a new treatment option 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 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 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

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.

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.

Cell Therapies

Kymriah®

(tisagenlecleucel)
Condition: Acute lymphoblastic leukemia
Company: Novartis
Approved: August 2017
Current list price: $475,000
More: kymriah.com

Treats patients up to age 25 with r/r acute lymphoblastic leukemia

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.

Kymriah®

(tisagenlecleucel)
Condition: Large B-cell lymphoma & DLBCL
Company: Novartis
Approved: May 2018
Current list price: $373,000
More: kymriah.com

Treats adult patients with r/r large B-cell lymphoma including diffuse large B-cell lymphoma (DLBCL)

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, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma.

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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.
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.
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.
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.
Yescarta® (axicabtagene ciloleucel) Condition: Large B-cell lymphoma & DLBCL, and follicular lymphoma Company: Kite Pharma Approved: October 2017 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. Yescarta is indicated for the treatment of adult patients with relapsed or refractory (r/r) large B-cell lymphoma after failing two or more lines of systemic therapy. Indications also include diffuse large B-cell lymphoma (DLBCL) not otherwise specified; primary mediastinal large B-cell lymphoma; high grade B-cell lymphoma; and diffuse large B-cell lymphoma 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.

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