SIIA
SELF-INSURANCE INSTITUTE OF AMERICA

2025 Cell & Gene Therapy

Overview & Resources

June 2025

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Introduction

How Cell & Gene Therapy Started

On September 15, 1990, a four-year-old girl received the first gene therapy for adenosine deaminase (ADA) deficiency, a rare immune disorder. A retroviral vector was used to insert functional copies of the ADA gene into her white blood cells, helping her immune system work better. Although this treatment wasn't permanent and required follow-up therapies, it was a major breakthrough in medicine. It showed that gene therapy could address genetic disorders at their source, bringing hope to patients and inspiring further research. This milestone paved the way for future advancements in gene therapy and genetic medicine.

Introduction to Cell and Gene Therapy

Cell and gene therapy represent groundbreaking advancements in modern medicine, offering transformative approaches to treating and potentially curing a wide range of diseases. These innovative therapies leverage the power of cellular and genetic engineering to address the root causes of
illnesses, rather than merely managing symptoms.

Why is Cell and Gene Therapy Important?

These therapies offer hope for patients with conditions that were previously considered untreatable or incurable. By targeting the underlying mechanisms of disease, cell and gene therapy have the potential to deliver long-lasting, personalized solutions that improve quality of life and reduce healthcare costs over time.

Applications and Advancements

Cancer Treatment: Chimeric Antigen Receptor (CAR)-T cell therapy has revolutionized the treatment of certain blood cancers.
Rare Genetic Disorders: Gene therapy is providing new options for conditions like spinal muscular atrophy and hemophilia.
Regenerative Medicine: Stem cell therapy is being explored for repairing heart tissue, treating neurodegenerative diseases, and more.

As research and technology continue to evolve, cell and gene therapy are poised to redefine the future of healthcare, offering hope and healing to millions worldwide.

Glossary

Term	Definition	Examples
Adenovirus / Adenovirus Vector	Adenoviruses can be genetically modified and used in gene therapy to treat cancer, in vaccines and may potentially be used to treat other diseases. It is one of the viruses that is the basis for viral vector-based gene therapy.	
Adeno-Associated Virus (AAV) / Adeno-Associated Viral Vector	Adeno-associated virus is a non-enveloped virus that can be engineered to deliver DNA to target cells. It is one of the viruses that is the basis for viral vector-based gene therapy.	Luxturna, Zolgensma, Glybera, Roctavian, Hemgenix, Upstaza, Kebilidi
Allogeneic Transplantation	The transfer of tissue or cells from one person to a distinct member of the same species. For example, a sister may donate bone marrow to a brother of the same family (bone marrow transplant). Another example is a car crash victim may donate his kidney which is surgically transferred to an unrelated person.	
Antibodies	Proteins that help fight infections and toxins and are found in the blood. They are made by B lymphocytes. Each antibody binds to a specific part of a protein or antigen. Recently, many biological drugs are comprised of huge quantities of exact copies of one antibody which binds to a specific disease target.	
Antigens	A part of a protein or other molecule that causes an immune response. Antigens are found in toxins, bacteria, molds, and viruses. Antigens can induce the production of antibodies (immunoglobulins) and/or induce T lymphocytes to kill cells or suppress the immune response.	
Apheresis	A process where specific components (e.g., stem cells) of a patient's blood are separated and removed. It is a critical step in the process used in making an ex-vivo gene therapy.	
Autologous Transplantation	Removal of tissue or cells from one person and then returning the cells back to the same person.	
Average Wholesale Price (AWP)	The average price that wholesalers sell prescription drugs to pharmacies and other providers.	
Biologics	A classification of products derived from living sources, such as humans, animals, bacteria and viruses. Vaccines, immune globulin, antibodies with a specific target (such as anti-TNF), gene therapy vectors, cell therapy, stem cell therapy, and anti-toxins are biologics.	
Cell Therapy	One of the three most commonly used gene therapy approaches is cell-modified gene therapy. In this approach the patient's cells are removed (via leukapheresis) from the body, the cells are treated (a working copy of the gene is added, or the affected gene is edited), the modified cells are returned to the patient with the goal of improving a disease.	Kymriah, Yescarta, Tecartus, Breyanzi, Carvykti, Omisirge, Amtagvi, Tecelra, Aucatzyl

Glossary

Term	Definition	Examples
Chimeric Antigen Receptor T Cell Therapy (CAR-T)	A form of cell-modified gene therapy used to treat blood cancers such as diffuse, acute lymphoblastic leukemia (ALL), large B-cell lymphoma (DLBCL), follicular lymphoma (FL), mantle cell lymphoma (MCL), chronic lymphocytic leukemia (CLL), and multiple myeloma (MM). The patient's T cells are removed from the patient, modified in a manufacturing process where a gene for a chimeric antigen receptor is inserted into the T cells. These CAR-T cells are then multiplied and later returned to the patient by infusion so that they will recognize cancer cells, bind to them and kill them.	
Chromosomes	Long strings of genetic material made up of DNA and accessory proteins. The DNA contains approximately 30,000 to 100,000 genes that make up the human genome. Human cells contain 23 pairs of chromosomes (46 total), with the mother and the father each contributing one chromosome to each pair in their children.	Luxturna, Zolgensma, Glybera, Roctavian, Hemgenix, Upstaza, Kebilidi
Clinical Trial	A research study in human volunteers and is designed to answer specific questions about a disease, new therapies, or new ways of using known treatments. Clinical trials are used to determine whether new drugs or treatments are both safe and effective. Trials are in four phases: Phase I tests a new drug or treatment in a small group to evaluate safety and toxicity. Phase II expands the study and begins to assess efficacy. Phase III expands the study to an even larger group of people and often compares the agent to a standard of care treatment. Phase IV takes place after the drug or treatment has been licensed and marketed.	
Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR)/Cas9	A gene editing technique that uses a specially designed RNA molecule to guide a Cas9 enzyme to a specific sequence of DNA so it can change or edit that site sequence.	
Differentiation	A multi-step process of maturation whereby a stem cell can generate daughter cells which then become the specialized cells within our body. The differentiated cell expresses some different genes and can perform specialized functions, such as contraction of muscle cells, or secretion of insulin from islet cells located in the pancreas.	
DNA (deoxyribonucleic acid)	A very long molecule that carries a cell's genetic information. DNA is made up of two antiparallel strands that are held together by weak chemical bonds between base pairs of nucleotides. DNA is comprised of the four nucleotides based on these four bases: adenine (A), guanine (G), cytosine (C), and thymine (T).	
Ex Vivo Gene Therapy	Patient cells are harvested, cultivated in the laboratory, and incubated with vectors carrying a corrective or therapeutic gene. Cells with the new genetic information are then transplanted back into the patient from whom they were derived.	
US Food & Drug Administration (FDA)	An agency in the US federal government whose mission is to protect public health by making sure that drugs, medical devices, and other equipment are safe and effective.	

Glossary

Term	Definition	Examples
Gammaretrovirus/ Gammaretroviral Vector	Gammaretrovirus is a simple retrovirus. The vector requires active cell division for successful infection. Although this vector is not as frequently used as other viral vectors such as lentivirus and AAV, it is commonly used to generate CAR-T cells by efficiently delivering the CAR gene into T cells, enabling them to target and destroy cancer cells.	Yescarta
Gene	A segment of DNA found on a chromosome that codes for a particular protein. Humans have tens of thousands of genes that act as a blueprint for making specific enzymes or other proteins for virtually every biomedical reaction and structure in the body.	
Gene Addition	One of three most commonly used gene therapy approaches, where a functioning genetic material (e.g., a working gene) is added to do the work of a faulty or missing gene.	Luxturna, Zolgensma, Zynteglo, Skysona, Hemgenix, Elvidys, Roctavian, Lyfgenia, Lenmeldy, Kebilidi
Gene Editing	The creation of targeted double-stranded breaks in DNA, with or without repair instructions, to disrupt or correct the function of a gene.	Casgevy
Gene Inactivation	An approach in gene therapy that turns off or reduces the function of a gene to have a therapeutic effect.	
Gene Therapy	The introduction, removal, or change in the content of a person's genetic code with the goal of treating or curing a disease. The three most common approaches used today are gene addition, gene editing, and cell-modified gene therapy	
Genetic Information	The hereditary information coded in a person's DNA or RNA.	
Hematopoetic Cells	The various cell types that comprise blood. The blood contains red blood cells, platelets, macrophages, basophils, eosinophils, neutrophils, B lymphocytes, T lymphocytes.	
Hemopoetic Stem Cells	A specialized type of stem cell that can replenish itself and produce cells that develop into a variety of mature blood cells, including red blood cells, macrophages, basophils, eosinophils, neutrophils, lymphocytes, and platelets. The greatest number of these cells are found in the bone marrow, but can also be found in the peripheral blood, umbilical cord blood, and the fetal liver.	
Immunodeficiency	One or more defects in the immune system that leads to an increased risk of infections. Some immunodeficiency's are due to genetic mutations. Certain viruses, including HIV, can also cause immune deficiencies.	
In Vivo	The administration of a vector carrying the therapeutic genetic material to a live animal. The vector can be delivered by a variety of methods, including direct injection into the blood (intravenous injection) or various organs by other physical means of administration (hypodermic injection, aerosol, intrathecal, etc.).	

Glossary

Term	Definition	Examples
Invoice or List Price	The price a manufacturer or distributor charges a retailer for a drug; the net amount a provider pays for the drug or biological after discounts, rebates, refunds and/or additional adjustments. List price is also known as the WAC price.	
Lentivirus/Lentiviral Vector	Lentiviruses represent a class of animal and human viruses. It is a complex retrovirus and is one of the viruses that is the basis for viral vector-based gene therapy, including CAR-T therapies. Modifications of these viruses for vectors involve removal of the viral genes that cause disease and replacing the viral genes with therapeutic genetic material. In this way the lentivirus is engineered to insert the new DNA into the genome of the target cells which can then be used to treat disease.	Kymriah, Breyanzi, Abecma, Carvykti, Zynteglo, Skysona, Lyfgenia, Tecelra, Aucatzyl
Leukapheresis	A specific type of apheresis where white blood cells (leukocytes) are removed from a patient's blood. It is the first step in the process of making a CAR-T product.	
Market Access	The process of ensuring that pharmaceutical products are available to patients who need them, at an affordable price and in a timely manner	
Messenger RNA (mRNA)	A single molecule of RNA that works as a chemical map for a protein product	Comirnaty, Spikevax
Mutation	A change in the sequence of DNA which alters gene function. Sometimes the mutation changes the gene so that the protein encoded by the gene is abnormal. In other cases, the protein may be normal but the mutation causes the cell to make too little or too much of the protein.	
Pipeline	A list of drugs that a pharmaceutical company is developing; the process of developing new drugs from discovery to market availability.	
Prior Authorization	A process that healthcare payers use to review and approve medical care before it's provided.	
Qualified Treatment Centers (QTC)	A clinical facility that administers gene therapy that has been approved by the FDA. QTCs are selected by manufacturers based on their expertise in areas like genetic therapy, transplantation, and rare diseases. QTCs are also called authorized treatment centers (ATCs) by some manufacturers.	
Retrovirus/Retroviral Vector	Retroviruses insert a DNA copy of its RNA genome into the DNA of a host cell that it invades, changing the genome of the infected cell. The most notable retrovirus is HIV, which causes AIDS.	Tecartus
RNA (Ribonucleic Acid)	A molecule which is chemically similar to DNA and carries a matching code. RNA is the intermediary molecule that cells use to translate the information found in genes into the corresponding protein the genes encode.	
Stem Cells	Cells in the body that have the unique potential to develop into all the different cell types with specific functions, such as blood cells, brain cells, muscle cells, or bone cells.	

Glossary

Term	Definition	Examples
Viral Vector	A way to deliver genetic material to a cell using the blueprint of a virus as a guide; it may be used to carry genes and change mutated cells to healthy ones. Gene therapy delivery vehicles, or carriers, encapsulate therapeutic genes for delivery to cells. They include both genetically disabled viruses such as adenovirus or AAV, and non-viral vectors such as liposomes.	
Warranty Program	Provides health care payers, including Medicaid and Medicare, reimbursement should an advanced therapy treatment fail to deliver the expected medical outcomes on a given patient.	Kymriah, Breyanzi, Abecma, Carvykti, Zynteglo, Skysona, Lyfgenia, Tecelra, Aucatzyl
Wholesale Acquisition Cost (WAC)	The price that represents the manufacturer's published catalog or list price to wholesalers and is reported to First Databank (FDB) by manufacturers	

Gene Therapies | Approved

Brand	Generic	Condition Treated	ICD-10	Product Code	Manufacturer	Information Link
Adstiladrin®	nadofaragene firadenovec-vncg	Non-muscular Invasive Bladder Cancer resistant to BCG therapy	C67 C67.0 C67.1 C67.2 C67.3 C67.4 C67.5 C67.6 C67.7 C67.8 C67.9	J9029	Ferring Pharmaceuticals	www.adstiladrinhcp.com
Beqvez™	fidanacogene elaparvovec-dzkt	Hemophilia B	D67 D68	J3590	Pfizer, Inc	www.beqvez.com www.beqvezpfizerpro.com
Casgevy®	exagamglogene autoemcel	Sickle Cell Disease	Sickle Cell D57.0 D57.00 D57.01 D57.02 D57.03 D57.04 D57.09 D57.1 D57.2 D57.20 D57.211 D57.212 D57.213 D57.214 D57.218 D57.219	J3392	Vertex, CRISPR Therapeutics	www.casgevy.com

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Brand	Generic	Condition Treated	ICD-10	Product Code	Manufacturer	Information Link
Casgevy® (cont.)		Beta Thalassemia	Beta Thalassemia D56.1 D56.2 D56.3 D56.4 D56.5 D56.8 D56.9		Vertex, CRISPR Therapeutics	www.casgevy.com
Elevidys®	delandistrogene moxeparvovec- rokl	Duchenne Muscular Dystrophy	G71.01	J1413	Sarepta Therapeutics	www.elevidys.com
Encelto™	revakinagene taroretcel-lwey, NT501, allogenic encapsulated cell based	Macular Telangiectasia Type 2	H35.07	J3590	Abeona Therapeutics	www.encelto.com
Hemgenix®	etranacogene dezaparvovec-drlb	Hemophilia B	D67 D68	J1411	CSL Behring	www.hemgenix.com
Kebilidi™	elandocagene exuparvovec -tneq	Aromatic l-Amino Acid Decarboxylase Deficiency	E70.81	J3590	PTC Therapeutics	www.kebilidi.com
Lenmeldy™	atidarsagene autotemcil	Metachromatic Leukodystrophy	E75.25	J3590	Orchard Therapeutics	www.lenmeldy.com
Luxturna®	voretigene neparvovec-rzyl	Inherited Retinal Disease (due to mutations in both copies of the RPE65 gene)	H35.50 H35.52 H35.54	J3398	Spark Therapeutics	www.luxturna.com

Gene Therapies | Approved

Brand	Generic	Condition Treated	ICD-10	Product Code	Manufacturer	Information Link
Lyfgenia™	lovotibeglogene autotemcel	Sickle Cell Disease	D57.0 D57.00 D57.01 D57.02 D57.03 D57.04 D57.09 D57.1 D57.2 D57.20 D57.211 D57.212 D57.213 D57.214 D57.218 D57.219	J3394	bluebird bio, Inc.	www.lyfgenia.com
Rethymic®	allogeneic processed thymus tissue-agdc	Congenital Athymia	Q89.7	J3590	Sumitomo Pharma	www.rethymic.com
Roctavian®	valoctocogene roxaparvovec-rvox	Hemophilia A	D66 D68	J1412	Biomarin Pharmaceutical, Inc	www.roctavian.com
Skysona™	elivaldpgene autotemcel	Childhood Cerebral X-Linked Adrenoleukodystrophy	E71.52 E71.520 E71.521 E71.522 E71.528 E71.529	J3590	bluebird bio, Inc.	www.skysona.com
Vyjuvek®	beremagene geperpavec-svdt	Dominant Dystrophic Epidermolysis Bullosa	Q81.1	J3590	Krystal Biotech	www.vyjuvek.com
Zevaskyn™	prademagene zamikeracel, pz-cel	Recessive Dystrophic Epidermolysis Bullosa	Q81.2	J3590	Abeona Therapeutics	www.zevaskyn.com
Zolgensma®	onasemnogene abeparvovec-xioi	Spinal Muscular Atrophy Type 1	G12.0 G12.1 12.25 12.8 12.9	J3399	Novartis Gene Therapies, Inc	www.zolgensma.com

Gene Therapies | Approved

Brand	Generic	Condition Treated	ICD-10	Product Code	Manufacturer	Information Link
Zynteglo™	betibeglogene autotemce	Beta Thalassemia	D56.1 D56.2 D56.3 D56.4 D56.5 D56.8 D56.9	J3393	bluebird bio, Inc.	www.zynteglo.com

Gene Therapies | Pipeline

Brand	Generic	Condition Treated	ICD-10	Anticipated	Manufacturer	Information Link
TBD	RP-L102 ex vivo	Fanconi Anemia	D61.03	1/1/26	Rocket Pharmaceuticals	https://ir.rocketpharma.com/news-releases/news-re- lease-details/rocket-pharmaceuti-cals-receives-fda-regener- ative-medicine
Kresladi™	marnetegragene autotemcel; RP-L201	Leukocyte Adhesion Deficiency Type I	D72.0	6/30/25	Rocket Pharmaceuticals	www.biopharmadive.com/news/rocket-phar-ma-fda-reject-gene-therapy-kresladi-manu-facturing/720145
TBD	MCO-010 in vivo (sonpiretigene isteparvovec)	Retinitis Pigmentosa	H35 H35.32 H35.5	9/1/25	Nanoscope Therapeutics Inc.	www.nanostherapeutics.com/pipeline
TBD	UX111 in vivo (fka ABO-102)	Mucopolysaccharidosis Type II	E76.1	8/18/25	Ultragenyx Pharmaceutical	www.ultragenyx.com/our-research/pipeline
TBD	RGX-121 in vivo	Mucopolysaccharidosis Type II	E76.1	11/1/25	REGENXBIO	www.regenxbio.com/therapeutic-programs/rgx-121
TBD	DTX301 in vivo (avalotcagene ontaparvovec)	Ornithine Transcarbamylase Deficiency	E72.4	2025-2026	Ultragenyx Pharmaceutical	www.ultragenyx.com/our-research/pipeline
TBD	DTX401 in vivo (pariglasgene brecaparvovec)	Glycogen Storage Disease Type Ia	E74.00 E74.01 E74.09	12/1/25	Ultragenyx Pharmaceutical	www.ultragenyx.com/our-research/pipeline
TBD	ABBV-RGX-314 in vivo, subretinal	Wet Age-Related Macular Degeneration	H35.30 H35.32 H35.3210 H35.3220 H35.3290 H35.3293	H2 2026 Delayed?	REGENXBIO	www.regenxbio.com/therapeutic-programs
TBD	NTLA-2002 in vivo	Hereditary Angioedema	D84.1	2026	Intellia Therapeutics, Inc.	www.intelliatx.com/pipeline
TBD	giroctocogene fitelparvovec in-vivo	Hemophilia A	D66 D68	2026	Sangamo Therapeutics	www.sangamo.com/programs

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Brand	Generic	Condition Treated	ICD-10	Anticipated	Manufacturer	Information Link
TBD	ST-920 in vivo (isaralgagene civaparvovec)	Fabry Disease	E75.21	2026 Delayed?	Sangamo Therapeutics	www.sangamo.com/programs
TBD	cretostimogene grenadenorepvec in vivo	Bladder Cancer	C67 C67.0 C67.1 C67.2 C67.3 C67.4 C67.5 C67.6 C67.7 C67.8 C67.9	2026	CG Oncology	www.cgoncology.com/pipeline
TBD	detalimogene voraplasmid	BCG-Unresponsive NMIBC with CIS	C67 C67.0 C67.1 C67.2 C67.3 C67.4 C67.5 C67.6 C67.7 C67.8 C67.9	2026	enGene	www.engene.com/clinical-development-pipeline
TBD	RGX-202 in vivo	Duchenne Muscular Dystrophy	G71.01	2026	REGENXBIO	www.regenxbio.com/therapeutic-programs
TBD	AAV-RPGR in vivo (botaretigene sparoparvovec)	X-Linked Retinitis Pigmentosa	Z83.5 Z83.518 H35.52	12/1/25	MeiraGTx/Janssen Pharmaceuticals, Inc.	https://investors.meiragtx.com/news-releases/news-release-details/meiragtx-announces-50-million-milestone-janssen-pharmaceuticals

Gene Therapies | Pipeline

Brand	Generic	Condition Treated	ICD-10	Anticipated	Manufacturer	Information Link
TBD	rAAV2tYF-GRK1-RPGR in vivo (laruparetigene zosaparvovec)	X-Linked Retinitis Pigmentosa	Z83.5 Z83.518 H35.52	12/1/25	Beacon Therapeutics	www.beacontx.com/pipeline
TBD	Bidridisrogene xeboparvovec (SRP-9003)	Limg-Girdle Muscular Dystrophy, Type 2E/R4	G71.03	Hi 2026	Sarepta Therapeutics	www.Sarepta.com
TBD	AMT-130	Huntington's Disease	G10	H2 2026	uniQure	https://www.uniqure.com/programs-pipeline/huntingtons-disease

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Brand Name	Generic Name	Condition Treated	ICD-10	Approved/Cost	Manufacturer	Link
Kymriah®	tisagenlecleucel; tisa-cel	Acute Lymphoblastic Leukemia Diffuse Large B-cell Lymphoma Follicular Lymphoma	C91.0 C83.30 C82.90	8/30/2017 5/1/2018 $581,895 5/27/2022 $456,941	Novartis Pharmaceuticals	https://us.kymriah.com
Yescarta®	axicabtagene ciloleucel; axi-cel	Diffuse Large B-cell Lymphoma Non-Hodgkin Lymphoma Follicular Lymphoma	C83.30 C85.90 C82.90	10/18/2017 4/1/2022 $503,580	Kite Pharma	www.yescartahcp.com
Tecartus®	brexucabtageneautoleucel; brexu-cel	Mantle Cell Lymphoma Non-Hodgkin Lymphoma Acute Lymphoblastic Leukemia	C83.1 C85.90 C91.0	7/24/2020 10/1/2021 $462,000	Kite Pharma	www.tecartushcp.com
Breyanzi®	lisocabtagene maraleucel; liso-cel	Diffuse Large B-cell Lymphoma Follicular Lymphoma Non-Hodgkin Lymphoma Chronic lymphocytic Leukemia Small lymphocytic Lymphoma Mantle Cell Lymphoma	C83.30 C82.90 C85.90 C91.1 C83.0 C83.1	2/5/2021 6/24/2022 3/14/2024 5/30/2024 $531,350	Bristol Myers Squibb	www.breyanzi.com
Abecma®	idecabtagene vicleucel; ide-cel	Multiple Myeloma	C90.0	3/26/2021 4/4/2024 $528,312	Kita Pharma	www.abecma.com
Carvykti®	ciltacabtagene autoleucel; cilta-cel	Multiple Myeloma	C90.0	2/28/2022 4/4/2024 $522,055	Janssen/Legend Biotech	www.carvykti.com

Cell Therapies | Approved

Brand Name	Generic Name	Condition Treated	ICD-10	Approved/Cost	Manufacturer	Link
Omisirge®	omidubicel-onlv	Hematologic Malignancies	C95.90	4/17/2023 $512,070	Gamida Cell	www.omisirge.com
Lantidra®	donislecel-jujn	Diabetes Type 1	E10.9	6/28/2023 $300,000	CellTrans, Inc.	www.lantidra.com
Amtagvi™	lifileucel	Metastatic Melanoma	C43.9	2/16/2024 $515,000	Iovance Biotherapeutics	www.amtagvi.com
Tecelra®	afamitresgene autoleucel; afami-cel	Synovial Sarcoma	C49.2	8/1/2024 $727,000	Adaptimmune Therapeutics	www.tecelra.com
Aucatzyl®	obecabtagene autoleucel; obe-cel	Acute Lymphoblastic Leukemia	C91.0	22/8/2024 $525,000	Autolus Therapeutics	www.aucatzyl.com
Rynocil™	remestemcel-L-rknd	Acute Graft vs. Host Disease	D89.810	12/18/2024 $1,550,000	Mesoblast Limited	www.ryoncil.com

Cell Therapies | Pipeline

Brand Name	Generic Name	Condition Treated	ICD-10	Anticipated	Manufacturuer	Information Link
TBD	CAP-1002 (deramiocel)	Duchenne Muscular Dystrophy Cardiomyopathy	G71.01 I42.9	PDUFA 8/31/2025	Capricor Therapeutics	www.capricor.com/our-science
TBD	lete-cel (letetresgene auto-leucel)	Myxoid/Round Cell Liposarcoma	C49.9	2026	Adaptimmune Therapeutics	www.adaptimmune.com/pipeline/lete-cel
TBD	lete-cel (letetresgene auto-leucel)	Synovial Sarcoma	C49.20	2026	Adaptimmune Therapeutics	www.adaptimmune.com/pipeline/lete-cel
CORDStrom™	N/A	Recessive Dystrophic Epidermolysis bullosa	Q81.2	2026	INmune Bio	www.inmunebio.com/index.php/newsroom/2025- news/muneion-nounceslantoubmitiologicsicen-sep202502111 04706
TBD	zimislecel (VX-880)	Diabetes Type 1	E10.0	2026	Vertex Pharmaceuticals	https://investors.vrtx.com/news-releases/news-release-details/vertex-announces-program-updates-type-1-diabetes-portfolio
Ebvallo™	tabelecleucel; ATA129/EBV-CTL/Tab-cel	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative disease	D47.Z1	PDUFA Delayed	Atara Biotherapeutics	www.ebvallo-ebv.com
NurOwn®	N/A	Amyotrophic Lateral Sclerosis	G12	Delayed	BrainStorm Cell Therapeutics	https://ir.brainstorm-cell.com/2025-05-19-BrainStorm-Receives-FDA-Clearance-to-Initiate-Phase-3b-Trial-of-NurOwn-R-for-ALS
TBD	KYV-101	Stiff Person Syndrome	G25.82	H2 2026	Kyverna Therapeutics	www.kyvernatx.com/platform-pipeline

Gene Therapy | Uptake

Therapy	Condition	FDA Approval Date	Wholesale Acquisition Price (WAC)*	Average Wholesale Price (AWP)	Estimated Target Population	Incidence	Prevalence
Adstiladrin®	Non-muscular Invasive Bladder Cancer resistant to BCG Therapy	December 2022	$60,000 per installation, may be given as often as every 3 months	$72,000 per installation, may be given as often as every 3 months	1.4 per million	18 per million	67 per million
Beqvez™	Hemophilia B	April 2024	$3,500,000	$4,200,000	2,600 adults	1 per million	3.7 per 100,000 males
Casgevy®	Sickle Cell Disease Beta Thalassemia	December 2023 January 2024	$2,200,000 $2,200,000	$2,640,000	9,000 patients 12 years old and older 1,300	4 per million 10 per million	100,000 200 per million
Elevidys®	Duchenne Muscular Dystrophy	June 2023 June 2024 expanded indications	$3,200,000	$3,840,000	1,360 boys	25 per million	1 in 3,500 live male births
Encelto™	Revakinagene Taroretcel-NT501	March 2025	$250,000 per eye	$300,000	Anticipate that most patients will receive the therapy	?	0.005-0.1% in individuals over 40 years old
Hemgenix®	Hemophilia B	November 2022	$3,500,000	$4,200,000	2,600 adults	1 per million	3.7 per 100,000 males
Kebilidi™	Aromatic L-Amino Acid Decarboxylase Deficiency	November 2024	$3,950,000	$4,740,000	50 pediatric patients	1 per million	Unknown
Lenmeldy™	Metachromatic Leukodystrophy	March 2024	$4,250,000	$5,100,000	400-1,700 pediatric patients worldwide	4 per million	1 in 40,000 to 1 in 100,000
Luxturna®	Inherited Retinal Disease (due to mutations in both copies of the RPE65 gene)	December 2017	$456,875 per eye	$548,250 per eye	1,000-2,500	3 per million	1:330,000 to 130,000

* Product only, does not include administration cost.

Information contained in this document is current as of June 2025.

Gene Therapy | Uptake

Therapy	Condition	FDA Approval Date	Wholesale Acquisition Price (WAC)*	Average Wholesale Price (AWP)	Estimated Target Population	Incidence	Prevalence
Lyfgenia™	Sickle Cell Disease	December 2023	$3,100,000	$3,720,000	9,000 patients 12 years od age and older	4 per million	100,000
Rethymic®	Congenital Athymia	October 2021	$2,811,385	$3,373,622	1 per million	1 per million	1 per million
Roctavian®	Hemophilia A	June 2023	$2,900,000	$3,480,000	8,000 adult patients	1 per million	12 per 100,000 males
Skysona™	Childhood Cerebral X-Linked Adrenoleukodystrophy	September 2022	$3,00,000	$3,600,000	700 pediatric patients	1 per million	800 males 2 per million
Vyjuvek®	Dominant Dystrophic Epidermolysis Bullosa	May 2023	$25,230 per vial	$30,276 per vial	As of Feb 2025-510 starts	4 per million	3 per million
Zevaskyn™	Recessive Dystrophic Epidermolysis Bullosa	April 2025	$3,147,000	TBD	TBD	1 per million	2 per million
Zolgensma®	Spinal Muscular Atrophy Type 1	May 2019	$2,391,000	$2,870,047	500 pediatric patients annually	2 per million	9.1 and 10 per 100,000
Zynteglo™	Beta Thalassemia	August 2022	$2,800,000	$3,360,000	1,000-1,300 adult and pediatric patients	10 per million	200 per million

* Product only, does not include administration cost.

Advocacy Groups

Name	Website	Contact	Comments
AADC Family Network	www.aadcfamilynetwork.org	Bruce Heger bruceheger@aadcfamilynetwork.org Kelly Heger kellyheger@aadcfamilynetwork.org	
American Bladder Cancer Society	https://bladdercancersupport.org	888.413.2344	
American Cancer Society	https://www.cancer.org/support-programs-and-services/online-communities.html	800.227.2345	
American Foundation for the Blind	https://www.afb.org	212.502.7600 connectcenter@aph.org	
Bladder Cancer Advocacy Network	https://bcan.org	888.901.BCAN info@bcan.org	
CancerCare	https://www.cancercare.org/support_groups	800.813.HOPE	
Clinical Trials	https://clinicaltrials.gov	https://clinicaltrials.gov/about-site/about-ctg	
Duchenne Muscular Dystrophy	https://www.duchenne.com	888.727.782 www.duchenne.com/contact	
Foundation Fighting Blindness	https://www.fightingblindness.org	800.683.5555 info@fightingblindess.org	
Hemophilia Alliance	https://hemoalliance.org	215.279.8679 info@hemoalliance.org https://hemoalliance.org/contact	
Hemophilia Federation of America (HFA)	https://www.hemophiliafed.org	202.675.6984 www.hemophiliafed.org/contact-hfa	
International Myeloma Foundation	https://www.myeloma.org/support-groups	800.452.CURE	
Leukemia & Lymphoma Society	https://www.lls.org/support-resources	800.955.4572	

Advocacy Groups

Name	Website	Contact	Comments
Macular society	www.macularsociety.org/macular-disease/macular-conditions/mac-tel	Helpline - 0300 3030 111 Lines are open 9am – 5pm Monday to Friday	Located in England
MLD Foundation	https://mld.foundation	www.mld.foundation/contactus	
Multiple Myeloma Research Foundation	https://themmrf.org/support	203.229.0464	
National Bleeding Disorder Foundation	https://www.bleeding.org	888.463.6643 info@bleeding.org	Formerly the National Hemophilia Foundation
National Institutes of Health (NIH)	https://www.nih.gov	www.nih.gov/about-nih/contact-us	
National Organization for Rare Disorders	https://rarediseases.org	617.249.7300 (MA) 203.744.0100 (CT) 202.588.5700 (DC)	
National Sickle Cell Advocacy Network	https://www.sicklecelldisease.org/advocacy/national-sickle-cell-advocacy-network	800.421.8453 info@sicklecelldisease.org	
Parent Project Muscular Dystrophy	https://www.parentprojectmd.org/	800.714.5437 info@parentprojectmd.org	
Retina International	https://retina-international.org	+3 53 85 867 7328 info@retina-International.org	
United Leukodystrophy Foundation	https://ulf.org	800.728.5483 office@ulf.org	
World Bladder Cancer Patient Coalition	https://worldbladdercancer.org	+32 (0) 2300 33 info@worldbladdercancer.org	Located in Belgium
World Federation of Hemophilia	https://wfh.org	514.875.7944 wfh@wfh.org	Located on Canada

References

References
Emerging Therapy Solutions (ETS) Approved and Investigational Cell and Gene Therapies Pipeline, Updated March 2025
ICD-10 codes, online 2025, www.icd10data.com/ICD10CM/Codes
J codes and procedure codes, online 2025 www.hcpcsdata.com/Codes/J
American Society of Cell and Gene Therapy, https://patienteducation.asgct.org/gene-therapy-101/glossary
Milliman DNA Gene and Cell Therapy Forecasting: v3.6.0, March 2025