

Column1	Column2	Column3	Column4	Column6	Column5	Column7	Column8
<a href="https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products">https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products</a> <a href="https://www.whatisbiotechnology.org/index.php/science/summary/gene-therapy/">https://www.whatisbiotechnology.org/index.php/science/summary/gene-therapy/</a>	Date of approval	Disease	Gene Therapy Agent	# Individuals Affected	Gene Therapy Strategy	Citation	Side Effects
Retinal Degeneration	12/19/2017	Retinal dystrophy (bi-allelic mutation of RPE65)	LUXTURN A (voretigene neparovec-rzyl)	2-3 per 100,000 Individuals	Subretinal injection adeno-associated virus vector-based gene therapy (AAV2)	<a href="https://www.fda.gov/media/109906/download?attachment">https://www.fda.gov/media/109906/download?attachment</a> <a href="https://www.cell.com/molecular-therapy-family/molecular-therapy/pdf/S1525-0016(20)30655-9">https://www.cell.com/molecular-therapy-family/molecular-therapy/pdf/S1525-0016(20)30655-9</a> <a href="https://www.fda.gov/oc/foia/turna-side-effects.html">https://www.fda.gov/oc/foia/turna-side-effects.html</a>	Conjunctival hyperemia, cataract, increased intraocular pressure, retinal tear, dellen (thinning of the corneal stroma), macular hole, subretinal deposits, eye inflammation, eye irritation, eye pain, and maculopathy (wrinkling on the surface of the macula)
Spinal Muscular Atrophy	5/24/2019	Spinal muscular atrophy (pediatric patients < 2 years old with bi-allelic mutations in SMN1)	ZOLGENSMA (onasemnogene abeparovex-xioi)	1/10,000 live births	In-vivo adeno-associated virus vector-based gene therapy (AAV9)	<a href="https://www.fda.gov/media/126103/download?attachment">https://www.fda.gov/media/126103/download?attachment</a> <a href="https://www.cdc.gov/ncceb/dls/rsm/bb_sma.html#:~:text=Spina%20muscularis%20atrophy%20(SMA)%20is%20an%20inhereditary%20genetic%20protein.">https://www.cdc.gov/ncceb/dls/rsm/bb_sma.html#:~:text=Spina%20muscularis%20atrophy%20(SMA)%20is%20an%20inhereditary%20genetic%20protein.</a> <a href="https://journals.sagepub.com/proc/cc.uk.edu/doi/full/10.1177/1066028020314274">https://journals.sagepub.com/proc/cc.uk.edu/doi/full/10.1177/1066028020314274</a> <a href="https://www.fda.gov/oc/foia/zolgensma-side-effects.html">https://www.fda.gov/oc/foia/zolgensma-side-effects.html</a>	Thrombocytopenia, Respiratory insufficiency, thrombotic microangiopathy, Elevated Liver Enzymes, vomiting, elevated troponin-I levels, fevers
Beta-Thalassemia	8/17/2022	Transfusion dependent B-thalassemia	ZYNTEGLO (betibeglogene autotemcel)	Rare in USA (exact number is unknown, estimated that 1,300 people in U.S. are affected)	Ex-vivo Lenti-D lentiviral vector gene therapy (LVV)	<a href="https://www.fda.gov/media/160291/download?attachment">https://www.fda.gov/media/160291/download?attachment</a> <a href="https://www.bluebirdbio.com/our-focus/beta-thalassemia#:~:text=As%20a%20rare%20disease%20in%20Asian%20Americans%20and%20Middle%20Eastern%20populations.">https://www.bluebirdbio.com/our-focus/beta-thalassemia#:~:text=As%20a%20rare%20disease%20in%20Asian%20Americans%20and%20Middle%20Eastern%20populations.</a> <a href="https://www.fda.gov/oc/foia/zynteglo.html">https://www.fda.gov/oc/foia/zynteglo.html</a>	Tachycardia, pain in arm or legs, Alopecia, rash, mucositis, vomiting, abdominal pain, diarrhea, nausea, constipation, leukopenia, thrombopenia
X-linked Adrenoleukodystrophy	9/16/2022	Early Cerebral Adrenoleukodystrophy	SKYSONA (elivaldogene autotemcel)	1/10,000 - 1/17,000	Ex-vivo Lenti-D lentiviral vector gene therapy (LVV)	<a href="https://www.fda.gov/media/161649/download?attachment">https://www.fda.gov/media/161649/download?attachment</a> <a href="https://rare-diseases.org/rare-diseases/adrenoleukodystrophy/">https://rare-diseases.org/rare-diseases/adrenoleukodystrophy/</a> <a href="https://www.fda.gov/oc/foia/skysona.html">https://www.fda.gov/oc/foia/skysona.html</a>	Tachycardia, Alopecia, pruritus, rash, skin hyperpigmentation, fever, febrile neutropenia, hypertension, transfusion reaction, decreased appetite, anxiety, headache, vision blurred, cough, epistaxis, oropharyngeal pain
Hemophilia B	11/22/2022	Hemophilia B	HEMGENIX (etranacogene dezaparvovec-dtri)	1/25,000 Male Births	In-vivo adeno-associated virus vector-based gene therapy (AAV5)	<a href="https://www.fda.gov/vaccines-blood-biologics/vaccines/hemgenix">https://www.fda.gov/vaccines-blood-biologics/vaccines/hemgenix</a> <a href="https://rare-diseases.org/rare-diseases/hemophilia-b/#affected">https://rare-diseases.org/rare-diseases/hemophilia-b/#affected</a> <a href="https://www.fda.gov/oc/foia/hemgenix-side-effects.html">https://www.fda.gov/oc/foia/hemgenix-side-effects.html</a>	Increased Liver Enzymes, headache, hypersensitivity, flu-like symptoms, fatigue, nausea, malaise
Bladder Cancer (without invasion of the muscle wall)	12/16/2022	Non-muscle invasive bladder cancer (NMIBC) (unresponsive to BCG treatment)	ADSTILADRIN (nadofaragene firadenovec-vgic)	NMIBC incidence ~75% of 81,190 estimated newly diagnosed bladder cancer cases in USA. This is about 1/5,000 - 1/6,000.	In-vivo Intravesical Instillation adeno-associated virus vector-based gene therapy (AAV5)	<a href="https://www.fda.gov/media/164029/download?attachment">https://www.fda.gov/media/164029/download?attachment</a> <a href="https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6615913/">https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6615913/</a> <a href="https://www.fda.gov/oc/foia/adstladrin-side-effects.html">https://www.fda.gov/oc/foia/adstladrin-side-effects.html</a>	Glucose increased, instillation site discharge, triglycerides increased, fatigue, bladder spasm, micturition (urination urgency), creatinine increased, hematuria (blood in urine), phosphate decreased, chills, pyrexia (fever), and dysuria (painful urination)
Hemophilia A	6/30/2023	Hemophilia A	ROCTAVIAN (valoctocogene roxaparvovec-rvov)	1/5,000 Male Births	In-vivo adeno-associated virus vector-based gene therapy (AAV5)	<a href="https://www.fda.gov/vaccines-blood-biologics/vaccines/roctavian">https://www.fda.gov/vaccines-blood-biologics/vaccines/roctavian</a> <a href="https://rare-diseases.org/rare-diseases/hemophilia-a/">https://rare-diseases.org/rare-diseases/hemophilia-a/</a> <a href="https://www.fda.gov/oc/foia/roctavian.html">https://www.fda.gov/oc/foia/roctavian.html</a>	Increased Liver Enzymes, Nausea, fatigue, headache, infusion-related reactions, vomiting, abdominal pain, hepatotoxicity, thromboembolic events.

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<a href="https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products">https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products</a> <a href="https://www.whatisbiotechnology.org/index.php/science/summary/gene-therapy/">https://www.whatisbiotechnology.org/index.php/science/summary/gene-therapy/</a>	Date of approval	Disease	Gene Therapy Agent	# Individuals Affected	Gene Therapy Strategy	Citation	Side Effects
Acute Lymphoblastic Leukemia (and two types of non-Hodgkin lymphomas (FL and DLBCL))	08/30/2017 (ALL)	ALL/DLBCL/FL (in patients that are refractory or in second or later relapse)	KYMRIAH (tisagenlecleucel)	34 per 1 million (pediatric ALL)	Ex-vivo CD19-directed CAR-T cell therapy	<a href="https://www.fda.gov/media/107296/download?attachment">https://www.fda.gov/media/107296/download?attachment</a> <a href="https://www.cdc.gov/mmwr/wwmm63/sa3.html#text=Acute%20lymphoblastic%20leukemia%20(ALL)%20vs%20the%20most%20prevalent%20cancer%20among%20cases%20each%20year%20(11)">https://www.cdc.gov/mmwr/wwmm63/sa3.html#text=Acute%20lymphoblastic%20leukemia%20(ALL)%20vs%20the%20most%20prevalent%20cancer%20among%20cases%20each%20year%20(11)</a> <a href="https://www.drugs.com/sfx/kymriah-side-effects.html">https://www.drugs.com/sfx/kymriah-side-effects.html</a> <a href="https://clinicaltrials.gov/study/NCT05432310">https://clinicaltrials.gov/study/NCT05432310</a> <a href="https://primaryimmune.org/understanding-primary-immunodeficiency-treatment/gene-therapy">https://primaryimmune.org/understanding-primary-immunodeficiency-treatment/gene-therapy</a>	Cytokine release syndrome, infections-pathogen unspecified, hypogammaglobulinemia, fever, decreased appetite, viral infectious disorders, headache, febrile neutropenia, hemorrhage, musculoskeletal pain, vomiting, encephalopathy, diarrhea, hypotension, cough, nausea, bacterial infectious disorders, pain, hypoxia, tachycardia, edema, fatigue, and acute kidney injury
SCID	Clinical Trials	ADA and X-linked are major ones of note					
Sickle cell disease	Clinical Trials						