GENE THERAPY PIPELINE
1Q 2022–2H 2025

Breakthrough Therapies and New Biologics Feature in the Gene Therapy Pipeline

CVS Health®

	Therapy Name	Manufacturer	Phase of Development	Type	Breakthrough Therapy Designation	Drug Class	Indication	Route of Administration	FDA Decision Date	Estimated Potential U.S. Candidates
Projected Launch Year: 2022										
1Q	**ciltacabtagene autoleucel**	Janssen Pharmaceuticals/ Johnson & Johnson	Pending FDA approval	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma in adults	Injection-IV, one-time	02/28/22	9,000 adult patients
2Q	**Zynteglo** (betibeglogene autotemcel)	Bluebird Bio	Pending FDA approval	New Biologic	Yes	Gene therapy, ex vivo	The treatment of transfusion-dependent beta-thalassemia in pediatrics and adults	Injection-IV, one-time	05/21/22	1,000 adult and pediatric patients
2Q	**Instiladrin** (nadofaragene firadenovec)	FKD Therapies/ Ferring Pharmaceuticals	Pending FDA approval	New Biologic	Yes	Gene therapy, in vivo	The treatment of high-grade, non-muscle invasive, bacillus Calmette-Guérin (BCG)-refractory bladder cancer in adults	Injection-Intravesical, multi-dose		56,000 adult patients per year
2Q	**Kymriah** (tisagenlecleucel)	Novartis	Phase II	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory follicular lymphoma in adults	Injection-IV, one-time		86,000–100,000 adult patients
3Q	**LentiD** (elivaldogene autotemcel)	Bluebird Bio	Phase III	New Biologic	Yes	Gene therapy, ex vivo	The treatment of cerebral adrenoleukodystrophy in males aged less than 18 years	Injection-IV, one-time		700 pediatric patients
3Q	**Yescarta** (axicabtagene ciloleucel)	Gilead Sciences/Kite	Pending FDA approval	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The second-line treatment of relapsed or refractory diffuse large B-cell lymphoma in adults	Injection-IV, one-time	07/30/22	90,000 adult patients
3Q	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Phase III	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The second-line treatment of relapsed or refractory, aggressive, large B-cell lymphoma, in adults who are eligible for stem cell transplant	Injection-IV, one-time		90,000 adult patients
4Q	**Roctavian** (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time		8,000 adult patients
4Q	**eladocagene exuparvovec**	Agilis Biotherapeutics/ PTC Therapeutics	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in pediatrics	Injection-Intracerebral, one-time		100 pediatric patients worldwide
4Q	**etranacogene dezaparvovec**	Uniqure	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		2,600 adult patients

Nine gene therapies are projected to be approved in 2022 including breakthrough therapies for both severe hemophilia A and hemophilia B.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 22, 2021.

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1H	**fidanacogene elaparvovec**	Pfizer/ Spark Therapeutics	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		2,600 adult patients
1H	**RPL201**	Rocket Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1	Injection-IV, one-time		300 pediatric patients worldwide
1H	**obecabtagene autoleucel**	Autolus Therapeutics	Phase I/II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of acute lymphoblastic leukemia	Injection-IV, one-time		30,000 adult patients
1Q	**beremagene geperpavec**	Krystal Biotech	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of dystrophic epidermolysis bullosa	Topical, multi-dose		900 adult and pediatric patients
2Q	**olenasufligene relduparvovec**	Lysogene/Sarepta Therapeutics	Phase II/III	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA, also called Sanfilippo Type A	Injection-Intracerebral, one-time		240–1,840 patients
2Q	**Lumevoq** (lenadogene nolparvovec)	GenSight Biologics	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of Leber's hereditary optic neuropathy, in adults with the ND4 mutation	Injection-Intraocular, one-time		4,500–7,500 adult patients
2Q	**debcoemagene autoficel**	Castle Creek Pharma	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients aged 7 years and older	Injection-Intradermal, multi-dose		400 adult and pediatric patients
2Q	**EB101**	Abeona Therapeutics	Phase III	New Biologic	Yes	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients aged 6 years and older	Topical, one-time		400 adult and pediatric patients
2H	**Engensis** (donaperminogene seltoplasmid)	Helixmith	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of diabetic peripheral neuropathy	Injection-IM, multi-dose		7.1–13.5 million adult patients
2H	**OTL103**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Wiskott Aldrich syndrome in pediatrics	Injection-IV, one-time		500 pediatric male patients
3Q	**LentiGlobin** (beta-globin gene therapy)	Bluebird Bio	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in adults and pediatrics	Injection-IV, one-time		58,000 adult and pediatric patients
3Q	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of pre-symptomatic, early-onset metachromatic leukodystrophy	Injection-IV, one-time		400–1,700 pediatric patients worldwide
3Q	**PBCMA101**	Poseida Therapeutics	Phase II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma	Injection-IV, one-time		9,000 adult patients

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Projected Launch Year: 2023 (cont.)										
4Q	**giroctocogene fitelparvovec**	Pfizer/Sangamo BioSciences	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time		7,000 adult patients
	resamirigene bilparvovec	Audentes Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of X-linked myotubular myopathy in males aged younger than 5 years	Injection-IV, one-time		40 male newborns per year

The pipeline of potential gene therapy approvals in 2023 is robust, with several anticipated first-in-class agents, such as the first expected sickle cell disease gene therapy.

Projected Launch Year: 2024										
1Q	**ABO102**	Abeona Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA, also called Sanfilippo Type A	Injection-IV, one time		240–1,840 patients
	CTX001	CRISPR Therapeutics/Vertex Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in adults	Injection-IV, one time		Subset of the 100,000 patients with sickle cell disease
	CTX001	CRISPR Therapeutics/Vertex Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of transfusion-dependent beta thalassemia in adults	Injection-IV, one time		<1,000 adult patients
2Q	**fordadistrogene movaparvovec**	Pfizer	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of ambulatory patients with Duchenne muscular dystrophy	Injection-IV, one-time		4,000 pediatric males
	Generx (alferminogene tadenovec)	Gene Biotherapeutics/ Molecular Medicine BioServices	Phase III	New Biologic	No	Gene therapy, in vivo	The improvement of exercise tolerance in patients with refractory angina due to myocardial ischemia	Injection-Intracoronary, one-time		900,000–1.2 million adult patients
	Tavo (tavokinogene telsaplasmid)	Merck/OncoSec	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of advanced or metastatic malignant melanoma in adults whose cancer has progressed on a checkpoint inhibitor, in combination with Keytruda (pembrolizumab)	Injection, multi-dose		Eligible patient candidates unknown, subset of the ~1.2 million living with melanoma
	ofranergene obadenovec	VBL Therapeutics	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of recurrent platinum-resistant ovarian cancer, in combination with paclitaxel	Injection-IV, multi-dose		15,000 patients aged 15 and older
	RPL102	Rocket Pharmaceuticals	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia	Injection-IV, one-time		<1,000 pediatric patients

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3Q	**ProstAtak** (aglatimagene besadenovec)	Advantagene/ Candel Therapeutics	Phase III	New Biologic	No	Gene therapy, in vivo	The first-line treatment of adults with intermediate to high risk, localized, prostate cancer, in combination with external beam radiation therapy and valacyclovir	Injection-Intratumoral, multi-dose		125,000 adult patients
	JNJ64400141	Janssen Pharmaceuticals/ Johnson & Johnson	Phase III	New Biologic	Yes	Gene therapy, in vivo/Vaccine	The prevention of respiratory syncytial virus-mediated lower respiratory tract disease in adults aged 60 years or older	Injection-IM, multi-dose		34 million adult patients
4Q	**Tecartus** (brexucabtagene autoleucel)	Gilead Sciences/ Kite	Phase I/II	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory B-cell precursor acute lymphoblastic leukemia in patients aged 2 to 21 years	Injection-IV, one-time		7,500 pediatric patients

Treatments for prostate cancer and respiratory syncytial virus-mediated lower respiratory tract disease are two of the gene therapies anticipated to be approved in 2024.

Projected Launch Year: 2025										
1H	**verbrinacogene setparvovec**	Freeline Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of severe hemophilia B in adults	Injection-IV, one-time		2,600 adult patients
2Q	**Invossa** (tonogenchoncel-L)	Kolon Group	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of knee osteoarthritis	Injection-Intra-articular, one-time		13 million adult patients
	nadofaragene firadenovec	Trizell	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of malignant pleural mesothelioma	Injection-Intrapulmonary, one-time		2,400 adult patients per year
2H	**RGX314**	RegenxBio	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of neovascular (wet) age-related macular degeneration	Injection-Intraocular, one-time		2 million adult patients

Knee osteoarthritis and age-related macular degeneration treatments are anticipated to be approved in 2025.

Information compiled from external sources. Manufacturer drug launch dates are subject to change without notice. Some products may not be dispensed by CVS Specialty Pharmacy.

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