GENE THERAPY PIPELINE
3Q 2021–2H 2025

Gene Therapies in the Pipeline Highlight a Variety of Conditions and Anticipated Treatments

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	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: 2021										
4Q	**Tecartus** (brexucabtagene autoleucel)	Gilead Sciences/Kite	Pending FDA approval	Supplemental Indication	Yes	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory B-precursor acute lymphoblastic leukemia in adults	Injection-IV, one-time	10/01/21	30,000 adult patients
	ciltacabtagene autoleucel	Janssen Pharmaceuticals/ Johnson & Johnson	Pending FDA approval	New Biologic	Yes	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma in adults	Injection-IV, one-time	11/29/21	9,000 adult patients

Two CAR T-cell therapy approvals — with Breakthrough Therapy designations — are anticipated in late 2021.

Projected Launch Year: 2022										
1Q	**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
	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
2Q	**Kymriah** (tisagenlecleucel)	Novartis	Phase III	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The second-line treatment of adults with relapsed or refractory diffuse large B-cell lymphoma	Injection-IV, one-time		90,000 adult patients
	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
	Yescarta (axicabtagene ciloleucel)	Gilead Sciences/Kite	Phase III	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		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 or later treatment of relapsed or refractory, aggressive, large B-cell lymphoma	Injection-IV, one-time		90,000 adult patients
	eladocagene exuparvovec (fka AAVhAADC)	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

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of July 13, 2021.

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Projected Launch Year: 2022 (cont.)										
3Q	**Zynteglo** (betibeglogene autotemcel)	Bluebird Bio	Phase III	New Biologic	Yes	Gene therapy, ex vivo	The treatment of beta-thalassemia major in transfusion-dependent patients aged 12 years and older and the treatment of transfusion-dependent beta-thalassemia in patients aged 12 years and older with a beta-0/beta-0 genotype	Injection-IV, one-time		1,450 adult and pediatric patients
4Q	**beremagene geperpavec**	Krystal Biotech	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of dystrophic epidermolysis bullosa (DEB)	Topical, multi-dose		900 adult and pediatric patients
	etranacogene dezaparvovec	Uniqure	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		1,800 adult patients
	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		5,300 adult patients

Five gene therapies with Breakthrough Therapy designations are projected to be approved in 2022.

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Projected Launch Year: 2023										
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		1,700 adult patients
	obecabtagene autoleucel (fka AUTO1)	Autolus Therapeutics	Phase I/II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of acute lymphoblastic leukemia in adults	Injection-IV, one-time		30,000 adult patients
	RPL201	Rocket Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1 (LAD-1)	Injection-IV, one-time		300 pediatric patients worldwide
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
2Q	**debcoemagene autoficel**	Castle Creek Pharma	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa (RDEB) in patients aged 7 years and older	Injection-Intradermal, multi-dose		400 adult and pediatric patients
	EB101	Abeona Therapeutics	Phase III	New Biologic	Yes	Gene therapy, ex vivo	The treatment of RDEB in patients aged 6 years and older	Topical, one-time		400 adult and pediatric patients

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Projected Launch Year: 2023 (cont.)										
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
	olenasufligene relduparvovec (fka LYSSAF302)	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
	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
	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
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	**atidarsagene autotemcel** (fka OTL200)	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
	JNJ64400141	Janssen Pharmaceuticals/ Johnson & Johnson	Phase II	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
	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
4Q	**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
	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		5,000 adult patients

Gene therapies used to treat a variety of conditions, such as diabetic peripheral neuropathy, may be approved in 2023.

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	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: 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
2Q	**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
2Q	**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
2Q	**RPL102**	Rocket Pharmaceuticals	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia in pediatrics	Injection-IV, one-time		<1,000 pediatric patients
2Q	**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
2H	**Zolgensma** (onasemnogene abeparvovec-xioi)	AveXis/ Novartis	Phase I	New Formulation	No	Gene therapy, in vivo	The treatment of spinal muscular atrophy Type 2 and Type 3 in pediatrics	Injection-Intrathecal, one-time		8,000 pediatric patients
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
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 pediatrics	Injection-IV, one-time		7,500 pediatric patients

Several gene therapies may be approved in 2024, including one for the improvement of exercise tolerance in patients with refractory angina due to myocardial ischemia.

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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		1,700 adult patients
2Q	**Invossa** (tonogenchoncel-L)	Kolon Group	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of knee osteoarthritis (OA)	Injection-Intra-articular, one-time		13 million adult patients
2Q	**nadofaragene firadenovec**	Trizell	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of relapsed or refractory 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 (AMD)	Injection-Intraocular, one-time		2 million adult patients

Treatments for knee osteoarthritis and age-related macular degeneration are two of the treatments that may be approved in 2025.

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