

GENE THERAPY PIPELINE 4Q 2020–2022

A Thriving Pipeline Expected to Continue Expanding Treatment Options



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: 2020										
4Q	Breyanzi (lisocabtagene maraleucel)	Bristol-Myers Squibb	Pending FDA approval	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory B-cell non-Hodgkin's lymphoma, including diffuse large B-cell lymphoma, primary mediastinal B-cell lymphoma, and follicular lymphoma, in adults who have received at least 2 prior therapies	Injection-IV	11/16/20	7,500 adult patients per year



Multiple CAR T-cell therapies for various cancers projected to be approved in 2021, including two for multiple myeloma.

Projected Launch Year: 2021										
1Q	idecabtagene vicleucel	Bluebird Bio/ Bristol-Myers Squibb/ Celgene	Pending FDA approval	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory multiple myeloma in adults who have received at least 3 prior therapies, including an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody	Injection-IV	03/27/21	9,000 adult patients
	Yescarta (axicabtagene ciloleucel)	Gilead Sciences/ Kite	Pending FDA approval	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of adults with relapsed or refractory indolent non-Hodgkin's lymphoma including follicular lymphoma and marginal zone lymphoma after 2 or more prior lines of systemic therapy	Injection-IV	03/04/21	94,000–117,000 adult patients
2Q	Instiladrin (nadofaragene firadenovec)	FKD Therapies/ Ferring Pharmaceuticals	Pending FDA approval	New Biologic	Yes	Gene therapy	The treatment of high-grade, non-muscle invasive, bacillus Calmette-Guérin (BCG)-refractory bladder cancer in adults	Injection-Intravesical		56,000 adult patients per year
3Q	ciltacabtagene autoleucel (fka JNJ68284528)	Janssen Pharmaceuticals/ Johnson & Johnson	Phase III	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory multiple myeloma in adults	Injection-IV		9,000 adult patients
	Kymriah (tisagenlecleucel)	Novartis	Phase II	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory follicular lymphoma in adults	Injection-IV		86,000–100,000 adult patients
	Tecartus (brexucabtagene autoleucel)	Gilead Sciences/ Kite	Phase I/II	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory B-precursor acute lymphoblastic leukemia in adults	Injection-IV		30,000 adult patients
4Q	allogeneic mesenchymal stem cells	Abeona Therapeutics	Phase III	New Biologic	Yes	Gene therapy	The treatment of recessive dystrophic epidermolysis bullosa in patients aged 6 years and older	Topical		400 adult and pediatric patients
	Engensis (donaperminogene seltoplasmid)	Helixmith	Phase III	New Biologic	No	Gene therapy	The treatment of diabetic foot ulcers in adults with peripheral artery disease	Injection-IM		4.5 million adult patients

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4Q	Kymriah (tisagenlecleucel)	Novartis	Phase III	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of adults with relapsed or refractory diffuse large B-cell lymphoma after first-line therapy	Injection-IV		90,000 adult patients
	OTL101	Orchard Therapeutics	Phase I/II	New Biologic	Yes	Gene therapy	The treatment of adenosine deaminase severe combined immunodeficiency in pediatrics aged 1 month and older	Injection-IV		80–160 newborns per year worldwide



Three hemophilia gene therapies expected to launch in 2022.

Projected Launch Year: 2022										
1H	AAVhAADC	Agilis Biotherapeutics/ PTC Therapeutics	Phase II	New Biologic	No	Gene therapy	The treatment of aromatic L-amino acid decarboxylase deficiency in pediatrics	Injection-Intracerebral		100 pediatric patients worldwide
	etranacogene dezaparvovec	Uniqure	Phase III	New Biologic	Yes	Gene therapy	The treatment of hemophilia B in adults	Injection-IV		1,800 adult patients
1Q	LentiD (elivaldogene autotemcel)	Bluebird Bio	Phase III	New Biologic	Yes	Gene therapy	The treatment of cerebral adrenoleukodystrophy in males younger than 18 years	Injection-IV		700 pediatric patients
	OTL103	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy	The treatment of Wiskott Aldrich syndrome in pediatrics	Injection-IV		500 pediatric male patients
	OTL200	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy	The treatment of late infantile or early juvenile metachromatic leukodystrophy	Injection-IV		400–1,700 pediatric patients worldwide
	resamirigene bilparvovec	Audentes Therapeutics	Phase I/II	New Biologic	No	Gene therapy	The treatment of X-linked myotubular myopathy in pediatrics	Injection-IV		40 male newborns per year
	Tavo (tavokinogene telsaplasmid)	Merck/ OncoSec	Phase II	New Biologic	No	Gene therapy	The treatment of advanced or metastatic malignant melanoma in adults whose cancer has progressed on a checkpoint inhibitor, in combination with Keytruda (pembrolizumab)	Injection-Intratumoral		Subset of the ~1.2 million living with melanoma
	Yescarta (axicabtagene ciloleucel)	Gilead Sciences/ Kite	Phase III	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The second-line treatment of relapsed or refractory diffuse large B-cell lymphoma in adults	Injection-IV		90,000 adult patients
Zynteglo (betibeglogene autotemcel)	Bluebird Bio	Phase III	New Biologic	Yes	Gene therapy	The treatment of transfusion-dependent beta-thalassemia in patients aged 12 years and older	Injection-IV		1,500 adult and pediatric patients	

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2Q	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	Phase III	New Biologic	Yes	Gene therapy	The treatment of hemophilia B in adults	Injection-IV	1,700 adult patients
	timrepigene emparvovec	Biogen/ Nightstar Therapeutics	Phase III	New Biologic	No	Gene therapy	The treatment of choroideremia in males aged 18 years and older	Injection-Intraocular	2,500–5,000 adult patients
2H	Lumevoq	GenSight Biologics	Phase III	New Biologic	No	Gene therapy	The treatment of Leber's hereditary optic neuropathy in adults with the ND4 mutation	Injection-Intraocular	4,500–7,500 adult patients
3Q	BIIB112	Biogen/ Nightstar Therapeutics	Phase II/III	New Biologic	No	Gene therapy	The treatment of X-linked retinitis pigmentosa in males aged 10 years and older	Injection-Intraocular	7,000–19,000 adult and pediatric patients
	Roctavian (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Phase III	New Biologic	Yes	Gene therapy	The treatment of severe hemophilia A in adults	Injection-IV	5,300 adult patients
4Q	AUTO1	Autolus Therapeutics	Phase I/II	New Biologic	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory acute lymphoblastic leukemia in adults	Injection-IV	30,000 adult patients
	beremagene geperpavec	Krystal Biotech	Phase III	New Biologic	No	Gene therapy	The treatment of dystrophic epidermolysis bullosa	Topical	900 adult and pediatric patients

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Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 5, 2020.

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