

GENE THERAPY PIPELINE 2020–2022

**Near-term Pipeline
Includes Cancer and
Hemophilia Treatments**



Therapy Name	Manufacturer	Phase of Development	Type	Breakthrough Therapy Designation	Drug Class	Indication	Route of Administration	FDA Decision Date	
Projected Launch Year: 2020									
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	05/25/20
2H	Tavo (tavokinogene tetsaplasmid)	Merck/OncoSec	Phase II/III	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	
3Q	KTEX19	Gilead Sciences/ Kite	Pending FDA approval	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory mantle cell lymphoma in adults	Injection-IV	08/10/20
	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	08/17/20
	valoctocogene roxaparovec	BioMarin Pharmaceutical	Pending FDA approval	New Biologic	Yes	Gene therapy	The treatment of severe hemophilia A in patients aged 18 years and older	Injection-IV	08/21/20
4Q	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	11/30/20



5 of 6 treatments expected to be approved this year are to treat a range of cancers.

Projected Launch Year: 2021

2Q	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	
	AT132	Audentes Therapeutics	Phase I/II	New Biologic	No	Gene therapy	The treatment of X-linked myotubular myopathy in pediatrics	Injection-IV	
	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	

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2Q	Yescarta (axicabtagene ciloleucel)	Gilead Sciences/ Kite	Phase II	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory indolent non-Hodgkin's lymphoma including follicular lymphoma and marginal zone lymphoma in adults	Injection-IV	
	etranacogene dezaparvovec	Uniqure	Phase III	New Biologic	Yes	Gene therapy	The treatment of hemophilia B in adults	Injection-IV	
2H	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	
	OTL200	Orchard Therapeutics	Phase I/II	New Biologic	No	Gene therapy	The treatment of late infantile or early juvenile metachromatic leukodystrophy	Injection-IV	
3Q	KTEX19	Gilead Sciences/ Kite	Phase II	New Biologic	No	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory B-precursor acute lymphoblastic leukemia in adults	Injection-IV	
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	
	BIIB112	Biogen/ Nightstar Therapeutics	Phase I/II	New Biologic	No	Gene therapy	The treatment of X-linked retinitis pigmentosa in males aged 10 years and older	Injection-Intraocular	
TBD	JNJ68284528	Janssen Pharmaceuticals/ Johnson & Johnson	Phase II	New Biologic	Yes	Chimeric antigen receptor (CAR) T-cell therapy	The treatment of relapsed or refractory multiple myeloma in adults	Injection-IV	
	Zolgensma (onasemnogene abeparvovec-xioi)	AveXis/Novartis	Phase I	New Biologic	No	Gene therapy	The treatment of spinal muscular atrophy Type 2 and Type 3 in pediatrics	Injection-Intrathecal	



The 2021 treatment pipeline has several treatments for **pediatric patients**.

Projected Launch Year: 2022

1H	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	
1Q	LentiD	Bluebird Bio	Phase II/III	New Biologic	Yes	Gene therapy	The treatment of cerebral adrenoleukodystrophy in males younger than 18 years	Injection-IV	

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1Q	ProstAtak (aglatimagene besadenovec)	Advantagene	Phase III	New Biologic	No	Gene therapy	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
	timrepigene emparvovec	Biogen/ Nightstar Therapeutics	Phase III	New Biologic	No	Gene therapy	The treatment of choroideremia, an inherited retinal dystrophy, in males aged 18 years and older	Injection-Intraocular
	Zynteglo (betibeglogene autotemcel)	Bluebird Bio	Phase III	New Biologic	Yes	Gene therapy	The treatment of beta-thalassemia major in transfusion-dependent patients aged 12 years and older	Injection-IV
	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 with a beta-0/beta-0 genotype	Injection-IV
2Q	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
2H	LentiGlobin (beta-globin gene therapy)	Bluebird Bio	Phase III	New Biologic	No	Gene therapy	The treatment of sickle cell disease in adult and pediatric patients	Injection-IV
4Q	fidanacogene elaparvovec	Pfizer/Spark Therapeutics	Phase III	New Biologic	Yes	Gene therapy	The treatment of hemophilia B in adults	Injection-IV
TBD	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
	OTL103	Orchard Therapeutics	Phase I/II	New Biologic	No	Gene therapy	The treatment of Wiskott Aldrich syndrome in pediatrics	Injection-IV
	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



Notable among the pipeline for 2022 are treatments for **hematological conditions**.

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