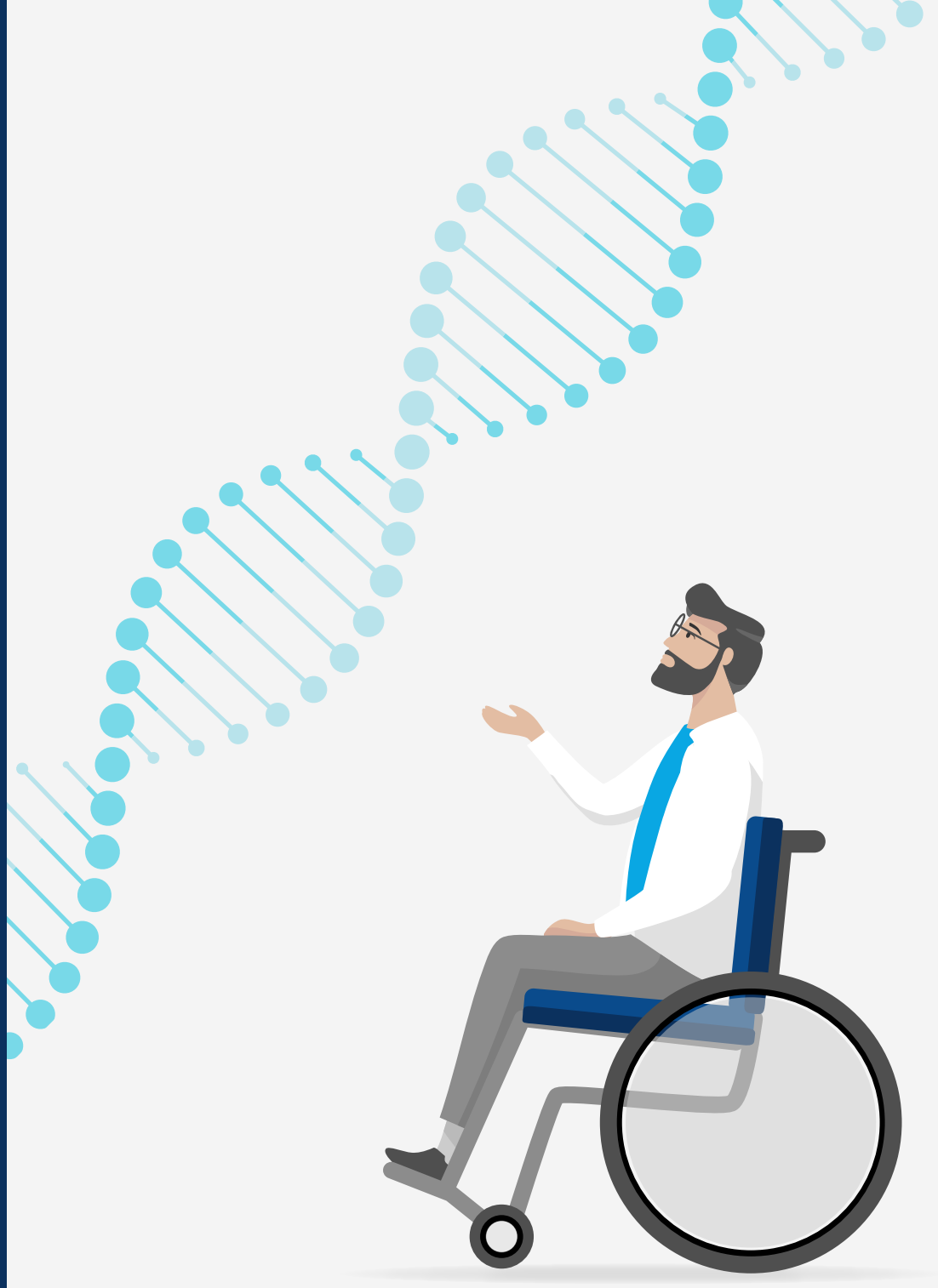
GENE THERAPY PIPELINE
2Q 2022–4Q 2025

Latest Look at Projected Gene Therapies and Approval Timelines



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	Therapy Name	Manufacturer	Phase of Development	Type	Breakthrough Therapy Designation	Drug Class	Indication	Route of Administration & Frequency	FDA Decision Date	Estimated Potential U.S. Candidates
Projected Launch Year: 2022										
2Q	**Kymriah** (tisagenlecleucel)	Novartis	Pending FDA approval	Supplemental Indication	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory follicular lymphoma in adults	Injection-IV, one-time	04/27/22	86,000–100,000 adult patients
	Breyanzi (lisocabtagene maraleucel)	Bristol-Myers Squibb	Pending FDA approval	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	06/24/22	90,000 adult patients
3Q	**Zynteglo** (betibeglogene autotemcel)	Bluebird Bio	Pending FDA approval	New Biologic	Yes	Gene therapy, ex vivo	The treatment of transfusion-dependent beta-thalassemia in adults and pediatrics	Injection-IV, one-time	08/19/22	1,450 adult and pediatric patients
	LentiD (elivaldogene autotemcel)	Bluebird Bio	Pending FDA approval	New Biologic	Yes	Gene therapy, ex vivo	The treatment of early cerebral adrenoleukodystrophy in males aged 17 years and younger who do not have a HLA-matched sibling hematopoietic stem cell donor	Injection-IV, one-time	09/16/22	700 pediatric patients
4Q	**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		60,000 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		7,500 adult patients

Treatments for thalassemia and hemophilia A could be approved in 2022.

Projected Launch Year: 2023										
1H	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Phase I/II	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma in adults	Injection-IV, one-time		135,000 adult patients
1Q	**EtranaDez** (etranacogene dezaparvovec)	CSL Behring/ 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
	Vyjuvek (beremagene geperpavec)	Krystal Biotech	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of dystrophic epidermolysis bullosa in patients aged 6 months and older	Topical, multi-dose		900 adult and pediatric patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of April 29, 2022.

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Projected Launch Year: 2023 (cont.)										
2Q	**dabocemagene 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
	eladocagene exuparvovec	PTC Therapeutics	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in patients aged 17 years and younger	Injection-Intracerebral, one-time		100 pediatric patients worldwide
	olenasufligene relduparvovec	Lysogene	Phase II/III	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo type A) in pediatrics aged 6 months and older	Injection-Intracerebral, one-time		200–1,800 pediatric patients
3Q	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of metachromatic leukodystrophy in children with late infantile form without clinical manifestations and in children with early juvenile form without clinical manifestations or with early clinical manifestations of the disease	Injection-IV, one-time		400–1,700 pediatric patients worldwide
	CTX001	CRISPR Therapeutics/Vertex Pharmaceuticals	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in patients aged 12 years and older	Injection-IV, one time		58,000 adult and pediatric patients
	CTX001	CRISPR Therapeutics/Vertex Pharmaceuticals	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of transfusion-dependent beta thalassemia in patients aged 12 years and older	Injection-IV, one time		1,000 adult and pediatric patients
	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
	RPL201	Rocket Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1 in pediatrics aged 3 months and older	Injection-IV, one-time		300 pediatric patients worldwide
4Q	**botaretigene sparoparvovec**	Johnson & Johnson/ MeiraGTx	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of X-linked retinitis pigmentosa due to RPGR mutations in patients aged 3 years and older	Injection-Intraocular, one-time		54,600–62,400 adult and pediatric patients
	lovotibeglogene autotemcel (formerly known as LentiGlobin)	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
	RPL102	Rocket Pharmaceuticals	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia in patients aged 1 to 17 years	Injection-IV, one-time		<1,000 pediatric patients

Gene therapies looking for approval in 2023 include agents targeting sickle cell disease and hemophilia B.

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1H	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Phase III	Supplemental Indication	Yes	CAR T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory multiple myeloma after 2 or 3 prior lines of therapy	Injection-IV, one time		9,000 adult patients
1H	**obecabtagene autoleucel**	Autolus Therapeutics	Phase I/II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory acute lymphoblastic leukemia in adults	Injection-IV, one-time		30,000 adult patients
1Q	**ABO102**	Abeona Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type 3A (also known as Sanfilippo syndrome type A)	Injection-IV, one time		200–1,800 pediatric patients
1Q	**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
1Q	**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 adult females
1Q	**resamirigene bilparvovec**	Astellas Pharma/ 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
1Q	**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
2Q	**Engensis** (donaperminogene seltoplasmid)	Helixmith	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of diabetic peripheral neuropathy in adults	Injection-IM, multi-dose		7.1–13.5 million 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
2Q	**GS030**	GenSight Biologics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa in adults	Injection-Intraocular, one-time		65,000 adult patients
2H	**laruparetigene zosaparvovec**	Applied Genetic Technologies Corp.	Phase II/III	New Biologic	No	Gene therapy, in vivo	The treatment of X-linked retinitis pigmentosa in males aged 8 to 50 years with a mutation in the RPGR gene	Injection-Intraocular, one-time		19,000–22,000 adult and pediatric patients
3Q	**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
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

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Projected Launch Year: 2024 (cont.)										
4Q	**delandistrogene moxeparvovec**	Sarepta Therapeutics	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystophy	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 hemophilia A in adults	Injection-IV, one-time		7,500 adult patients
	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

Thirteen new biologics may be approved in 2024 including for diabetic peripheral neuropathy and metastatic malignant melanoma.

Projected Launch Year: 2025										
1H	**RGX314**	AbbVie/ 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
	verbrinacogene setparvovec	Freeline Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		2,600 adult patients
2Q	**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
4Q	**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

The 2025 pipeline includes treatment of neovascular (wet) age-related macular degeneration.

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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