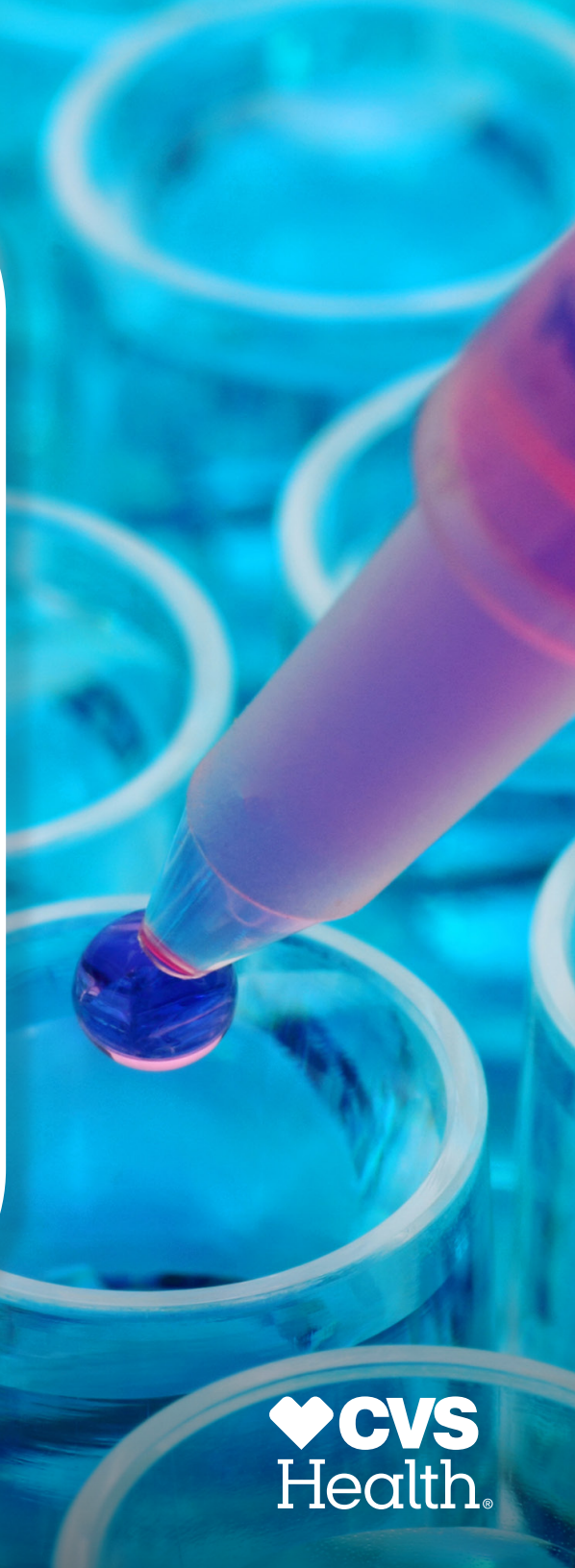
Gene Therapy Report

Q1 2023-Q1 2027

Projected Treatments and Approval Timelines



CVS Health

2023 PROJECTED LAUNCH YEAR

Several gene therapies expecting approval this year target blood disorders.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**Roctavian** (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Pending FDA approval 3/31/2023	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time	8,000 adult patients
2Q	**delandistrogene moxeparvovec**	Sarepta Therapeutics	Pending FDA approval 5/29/2023	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	**Vyjuvek** (beremagene geperpavec)	Krystal Biotech	Pending FDA approval 5/19/2023	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
2H	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Phase III	Supplemental Indication	Yes	Chimeric antigen receptor (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	78,000 adult patients
3Q	**Adstiladrin** (nadofaragene firadenovec) (fka Instiladrin)	FKD Therapies/ Ferring Pharmaceuticals	Approved	New Biologic	Yes	Gene therapy, in vivo	The treatment of adults with high-risk bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer with carcinoma in situ with or without papillary tumors.	Injection-Intravesical, multi-dose	60,000 adult patients
4Q	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of metachromatic leukodystrophy in patients aged 6 years or younger with late infantile form without clinical manifestations, and in patients aged 6 years or younger 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
4Q	**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

2023 CONTINUED

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4Q	**exagamglogene autotemcel**	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	42,500 adult and pediatric patients
4Q	**exagamglogene autotemcel**	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	1,500 adult and pediatric patients
4Q	**JNJ64400141**	Janssen Pharmaceuticals/ Johnson & Johnson	Phase III	New Biologic	Yes	Gene therapy, in vivo	The prevention of respiratory syncytial virus-mediated lower respiratory tract disease in adults aged 60 years or older	Injection-IM, multi-dose	48 million adult patients
4Q	**lovotibeglogene autotemcel** (beta-globin gene therapy)	Bluebird Bio	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease	Injection-IV, one-time	42,500 adult and pediatric patients

2024 PROJECTED LAUNCH YEAR

The 2024 pipeline includes gene therapies for chronic lymphocytic leukemia and diabetic peripheral neuropathy.

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1H	**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
1H	**zevorcabtagene autoleucel**	CARsgen Therapeutics	Phase I/II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma in adults	Injection-IV, one-time	43,000 adult 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

2024 CONTINUED

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1Q	**RPL201**	Rocket Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1 in patients aged 3 months and older	Injection-IV, one-time	300 pediatric patients worldwide
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	62,000 adult patients
1Q	**UX111**	Abeona Therapeutics/ Ultragenyx Pharmaceutical	Phase III	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 pediatric patients
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
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
2Q	**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	50,000 adult patients
2H	**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	140,000 adult patients

2024 CONTINUED

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2H	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Phase II	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory follicular lymphoma or marginal zone lymphoma	Injection-IV, one-time	34,000–49,000 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 8–50 years with a mutation in the RPGR gene	Injection-Intraocular, one-time	6,000 adult and pediatric patients
3Q	**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	5.5–11.2 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	73,000 adult patients
3Q	**RPL102**	Rocket Pharmaceuticals	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia in patients aged 1–17 years	Injection-IV, one-time	<1,000 pediatric patients
4Q	**pariglasgene brecaparvovec**	Ultragenyx Pharmaceutical	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of glycogen storage disease type 1a in patients aged 8 years and older	Injection-IV, one-time	6,000 adult and pediatric 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 2–21 years	Injection-IV, one-time	300 pediatric patients

2025 PROJECTED LAUNCH YEAR — Knee osteoarthritis, diabetic retinopathy, and age-related macular degeneration treatments are anticipated to be approved in 2025.

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1H	**RGX121**	RegenxBio	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment for mucopolysaccharidosis type II, also known as Hunter syndrome, in pediatrics aged 5 years and less	Injection-Intracerebral, one-time	< 25 pediatric patients
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	1.3 million adult patients
1Q	**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	6,000 adult and pediatric patients
2Q	**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	8,000 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
2Q	**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
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	16 million adult patients
4Q	**RGX314**	AbbVie/ RegenxBio	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of diabetic retinopathy	Injection-Intraocular, one-time	4.2 million adult patients

2026 PROJECTED LAUNCH YEAR

The 2026 pipeline includes a new formulation of a biologic treating spinal muscular atrophy Type 2 in pediatric patients.

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1H	**OCU400**	Ocugen	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa associated with NR2E3 and RHO mutations	Injection-Intraocular, one-time	3,400–4,600 adult and pediatric patients
1Q	**avalotcagene ontaparvovec**	Ultragenyx Pharmaceutical	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of ornithine transcarbamylase deficiency in patients aged 12 years and older	Injection-IV, one-time	4,300 adult and pediatric patients
1Q	**Zolgensma** (onasemnogene abeparvovec-xioi)	AveXis/Novartis	Phase I	New Formulation	No	Gene therapy, in vivo	The treatment of spinal muscular atrophy type 2 in patients aged 2–18 years	Injection-Intrathecal, one-time	4,000 pediatric patients

2027 PROJECTED LAUNCH YEAR

A gene therapy expected to be approved this year could expand the drug market for Wilson disease patients.

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1Q	**UX701**	Ultragenyx Pharmaceutical	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of Wilson's disease in adults	Injection-IV, one-time	8,500 adult patients

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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Source: RxPipeline, CVS Health Clinical Affairs. Information current as of January 19, 2023.

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