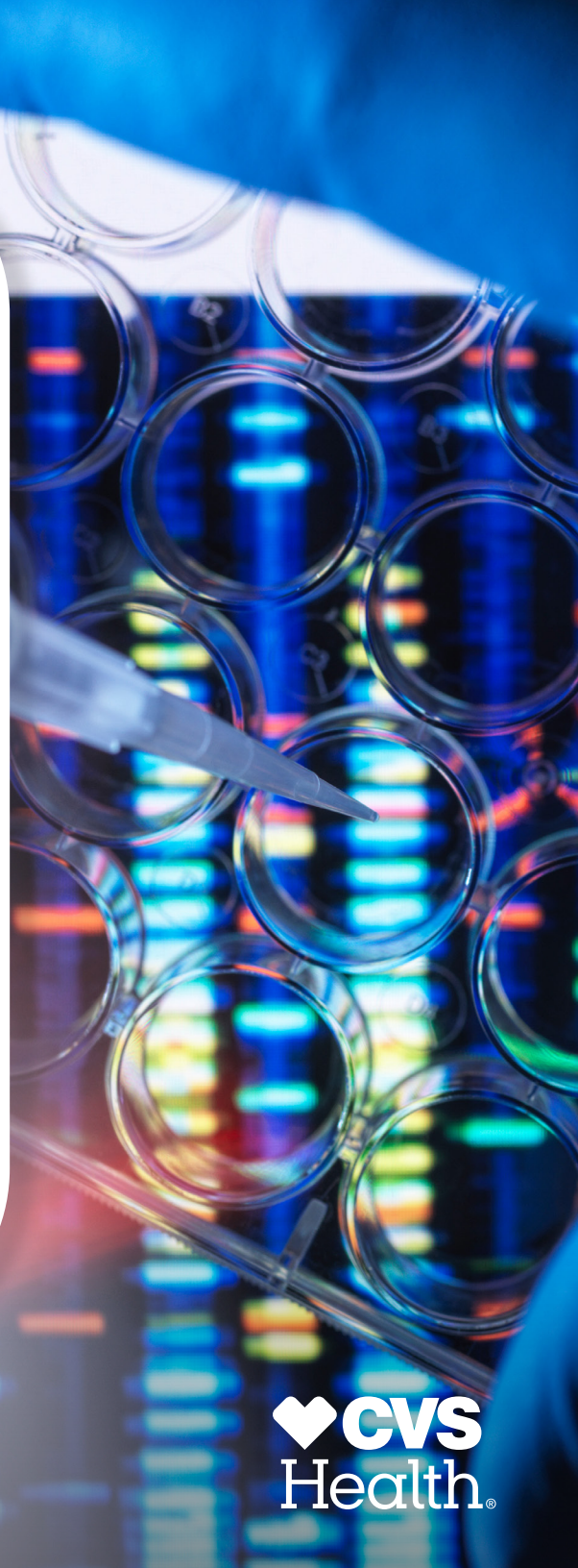
Gene Therapy Report

Q2 2023-Q3 2027

Projected Treatments and Approval Timelines



CVS Health

2023 PROJECTED LAUNCH YEAR

Gene therapies for Duchenne muscular dystrophy, hemophilia A, and sickle cell disease could soon be approved.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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
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	**Roctavian** (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Pending FDA approval 6/30/2023	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time	8,000 adult patients
3Q	**Adstiladrin** (nadofaragene firadenovec-vncg)	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	**exagamglogene autotemcel**	CRISPR Therapeutics/ Vertex Pharmaceuticals	Pending FDA approval 12/03/2023	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	Pending FDA approval 12/03/2023	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	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Pending FDA approval 12/16/2023	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

2024 PROJECTED LAUNCH YEAR

The 2024 pipeline includes treatment for diabetic peripheral neuropathy and certain types of leukemia.

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1Q	**lovotibeglogene autotemcel**	Bluebird Bio	Pending FDA approval 12/24/2023	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in patients aged 12 years and older who have a history of vaso-occlusive events	Injection-IV, one-time	42,500 adult and pediatric patients
1Q	**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
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	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of metachromatic leukodystrophy in patients aged 6 years and younger with late infantile form without clinical manifestations, and in patients aged 6 years and 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
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
2Q	**Upstaza** (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
2Q	**UX111**	Abeona Therapeutics/ Ultragenyx Pharmaceutical	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo syndrome type A)	Injection-IV, one-time	200 pediatric patients
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	5.5–11.2 million adult patients

2024 CONTINUED

New CAR T-cell therapies are in the pipeline for 2024.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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	**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
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	**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
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
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
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	**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
4Q	**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

2025 PROJECTED LAUNCH YEAR

A gene therapy for diabetic retinopathy could be approved in 2025.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
2Q	**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	**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	**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
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
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
3Q	**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
3Q	**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
4Q	**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
4Q	**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
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 potential new gene therapy for knee osteoarthritis.

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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 and the treatment of Leber congenital amaurosis associated with CEP290 mutations in patients aged 6 years and older	Injection-Intraocular, one-time	3,600–5,700 adult and pediatric patients
1Q	**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
1Q	**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
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

2027 PROJECTED LAUNCH YEAR

The first gene therapy for Wilson's disease could be approved in 2027.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**UX701**	Ultragenyx Pharmaceutical	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of Wilson's disease	Injection-IV, one-time	8,500 adult patients
3Q	**Carvykti** (ciltacabtagene autoleucel)	Janssen Pharmaceuticals/ Johnson & Johnson	Phase III	Supplemental Indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma in patients who have received 1 to 3 prior lines of therapy	Injection-IV, one-time	145,000 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 May 18, 2023.

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