

2023 CONTINUED

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


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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 breccaparvovec	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 sparaparvovec	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	avalotcogene 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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