

Gene Therapy Report

Q4 2024-Q3 2027

Projected Treatments and Launch Timelines



2024 PROJECTED LAUNCHES

A new therapy for leukemia is expected to launch by the end of 2024.



QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
4Q	Aucatzyl (obecabtagene autoleucel)	Autolus Therapeutics	Approved 11/8/2024	New biologic	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory adult B-cell acute lymphoblastic leukemia	Injection-IV, one-time split-dose	21,600 adult patients
4Q	Kebilidi (eladocagene exuparvovec-tneq; fka Upstaza)	PTC Therapeutics	Approved 11/13/2024	New biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in adult and pediatric patients	Injection-Intracerebral, one-time	330 pediatric patients

2025 PROJECTED LAUNCHES

The 2025 pipeline includes a new treatment for hemophilia A.



QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
2Q	prademagene zamikeracel (fka EB101)	Abeona Therapeutics	Pending FDA approval 4/29/2025	New biologic	Yes	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients ages 6 years and older	Surgical graft, one-time	450 adult and pediatric patients
2Q	Kresladi (marnetegrage autotemcel)	Rocket Pharmaceuticals	Pending FDA approval	New biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1 in patients ages 3 months and older	Injection-IV, one-time	150 pediatric patients
3Q	giroctocogene fitelparvovec	Pfizer/Sangamo BioSciences	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of hemophilia A without inhibitors in adults	Injection-IV, one-time	3,000–3,300 adult patients
3Q	PRGN2012	Precigen	Phase III	New biologic	Yes	Gene therapy, in vivo	The treatment of recurrent respiratory papillomatosis in adults	Injection-subcutaneous, multi-dose	6,400–11,600 adult patients

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
3Q	RPL102	Rocket Pharmaceuticals	Phase II	New biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia in patients ages 1–17 years	Injection-IV, one-time	<1,000 pediatric patients
3Q	UX111	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	1,500–4,000 adult and pediatric patients
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 ages 3 years and older	Injection-Intraocular, one-time per eye	5,500–13,000 adult and pediatric patients
4Q	resamirigene bilparvovec	Astellas Pharma	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of X-linked myotubular myopathy in males younger than 5 years	Injection-IV, one-time	40 male newborns per year
4Q	RGX121	RegenxBio	Phase III	New biologic	No	Gene therapy, in vivo	The treatment for mucopolysaccharidosis type II, also known as Hunter syndrome, in patients ages 5 years and younger	Injection-Intracerebral, one-time	<25 pediatric patients
4Q	sonpiretigene isteparvovec	Nanoscope Therapeutics	Phase II	New biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa in adults	Injection-Intraocular, one-time per eye	63,000–72,000 adult patients
4Q	vusolimogene oderparepvec	Replimune Group Inc.	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of cutaneous melanoma after progression on anti-PD1 therapy, in combination with Opdivo (nivolumab)	Injection-Intratumoral, multi-dose	24,700 adult patients

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	isarlalgagene civaparvovec	Sangamo BioSciences	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of Fabry disease in adults	Injection-IV, one-time	3,200 male adult patients
1Q	anitocabtagene autoleucl (fka CARTddBCMA)	Arcellx, Inc./ Gilead Sciences/ Kite	Phase II	New biologic	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma after at least 3 prior systemic therapies in adults	Injection-IV, one-time	47,800 adult patients
1Q	avalotcagene ontaparvovec	Ultragenyx Pharmaceutical	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of ornithine transcarbamylase deficiency in patients ages 12 years and older	Injection-IV, one-time	3,600–5,700 adult and pediatric patients
1Q	pariglasgene breccaparvovec	Ultragenyx Pharmaceutical	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of glycogen storage disease type 1a in patients ages 8 years and older	Injection-IV, one-time	3,000 adult and pediatric patients
1Q	ProstAtak (aglatimagene besadenovec)	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	73,800 adult patients
1Q	Zolgensma (onasemnogene abeparvovec-xioi)	AveXis/Novartis	Phase III	New formulation	No	Gene therapy, in vivo	The treatment of spinal muscular atrophy type 2 in patients ages 2–17 years	Injection-Intrathecal, one-time	3,900 pediatric patients
2Q	cretostimogene grenadenorepvec	Cold Genesys	Phase III	New biologic	Yes	Gene therapy, in vivo	The treatment of high-risk, non-muscle invasive, Bacillus Calmette-Guérin (BCG)-refractory bladder cancer with carcinoma in-situ (CIS) with or without Ta or T1 papillary tumors	Injection-Intravesical, multi-dose	38,800 adult patients

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
3Q	DBOTO	Regeneron Pharmaceuticals	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of congenital hearing loss due to mutations of the otoferlin gene, in patients ages 17 years and younger	Injection-Intracochlear, one-time per ear	810–6,500 pediatric patients
3Q	OCU400	Ocugen	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa in patients ages 8 years and older	Injection-Intraocular, one-time per eye	5,800–28,900 adult and pediatric patients
4Q	AAVAQP1	MeiraGTx	Phase II	New biologic	No	Gene therapy, in vivo	The treatment of radiation-induced late xerostomia in adults	Injection-Intraparotid, one-time	129,000 adult patients
4Q	laruparetigene zosaparvovec	Beacon Therapeutics	Phase II/III	New biologic	No	Gene therapy, in vivo	The treatment of X-linked retinitis pigmentosa in males ages 8–50 years with a mutation in the RPGR gene	Injection-Intraocular, one-time per eye	3,100–7,100 adult and pediatric patients
4Q	RPA501	Rocket Pharmaceuticals	Phase II	New biologic	No	Gene therapy, in vivo	The treatment of Danon disease in males ages 8 years and older	Injection-IV, one-time	7,500–15,000 adult and pediatric patients

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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 per eye	2 million adult patients
1Q	detalimogene voraplasmid	enGene Holdings	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of high-risk non-muscle invasive bladder cancer with carcinoma in situ with or without papillary tumors that is unresponsive to Bacillus Calmette-Guérin therapy	Injection-Intravesical, multi-dose	38,800 adult patients
1Q	NTLA2002	Intellia Therapeutics	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of hereditary angioedema in adults	Injection-IV, one-time	5,000 adult patients
1Q	UX701	Ultragenyx Pharmaceutical	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of hepatolenticular degeneration (Wilson's Disease) in adults	Injection-IV, one-time	6,300–8,400 adult patients
3Q	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 ages 2 to 21 years	Injection-IV, one-time	2,800 pediatric and adult patients



While the probability of any single individual needing a gene therapy today is low, the product pipeline is robust. It is also starting to shift from targeting ultra-rare diseases to addressing more common conditions. Read our Insights article, [“The future potential of gene therapy,”](#) to learn more.

This document contains references to brand-name prescription drugs that are trademarks or registered trademarks of pharmaceutical manufacturers not affiliated with CVS Health. The information contained herein is compiled from independent clinical sources and is provided for informational purposes only. Due to circumstances beyond CVS Health's control, prospective drug launch dates are subject to change without notice. This information should not be solely relied upon for decision-making purposes. This email includes products that may fall under a general specialty drug benefit. All products contained herein may not be provided by CVS Specialty. Dates included in this email are reflective of likely FDA approval date (otherwise known as PDUFA date). Actual approval date may occur before or after the date shown. Some drugs may not gain FDA approval at all. Dates do not reflect a projection for actual market availability. Drug launch may in some cases occur several months after FDA approval. Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 25, 2024.