Gene Therapy Report

Q3 2024-Q2 2027

Projected Treatments and Launch Timelines

CVS Health

2024 PROJECTED LAUNCHES

A new therapy for leukemia is expected to get approval 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	**Upstaza** (eladocagene exuparvovec)	PTC Therapeutics	Pending FDA approval 11/13/2024	New biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in patients ages 18 months and older	Injection-Intracerebral, one-time	330 pediatric patients
4Q	**obecabtagene autoleucel**	Autolus Therapeutics	Pending FDA approval 11/16/2024	New biologic	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory B-cell acute lymphoblastic leukemia in adults	Injection-IV, one-time split-dose	21,000 adult patients

2025 PROJECTED LAUNCHES

The 2025 pipeline includes two treatments for dystrophic epidermolysis bullosa.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**prademagene zamikeracel** (fka EB101)	Abeona Therapeutics	Pending FDA approval	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	**dabocemagene autoficel**	Castle Creek Pharma	Phase III	New biologic	No	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients ages 7 years and older	Injection-Intradermal, multi-dose	410 adult and pediatric patients
2Q	**Kresladi** (marnetegragene autotemcel, fka RPL201)	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
2Q	**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

2025 CONTINUED

New treatments for melanoma and hemophilia A could be on the way in 2025.

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2Q	**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
2Q	**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
2H	**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
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 adult 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	**pariglasgene brecaparvovec**	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
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

2026 PROJECTED LAUNCHES

The 2026 pipeline includes a new treatment for prostate cancer.

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1Q	**anitocabtagene autoleucel** (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	45,500 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	**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	73,800 adult patients
1Q	**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 after at least 3 prior systemic therapies	Injection-IV, one-time	45,500 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 -refractory bladder cancer with carcinoma in-situ with or without papillary tumors	Injection-Intravesical, multi-dose	38,800 adult patients
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

2026 CONTINUED

2 million adults could benefit from a treatment for wet macular degeneration in the 2026 pipeline.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
4Q	**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 (CIS) with or without papillary tumors that is unresponsive to Bacillus Calmette-Guerin therapy	Injection-Intravesical, multi-dose	38,800 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	**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 ages 6 years and older	Injection-Intraocular, one-time	6,500–9,700 adult and 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	2 million adult 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

2027 PROJECTED LAUNCHES

New therapies for angioedema, Wilson's disease, and a type of leukemia could get approval in 2027.

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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
2Q	**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 it's impossible to predict exactly how many claims your organization will incur for gene and genetically modified therapies, prevalence modeling can help estimate the potential based on the size of your membership. Read our Insights article, "**Estimating the impact of gene therapy claims,**" to learn more.