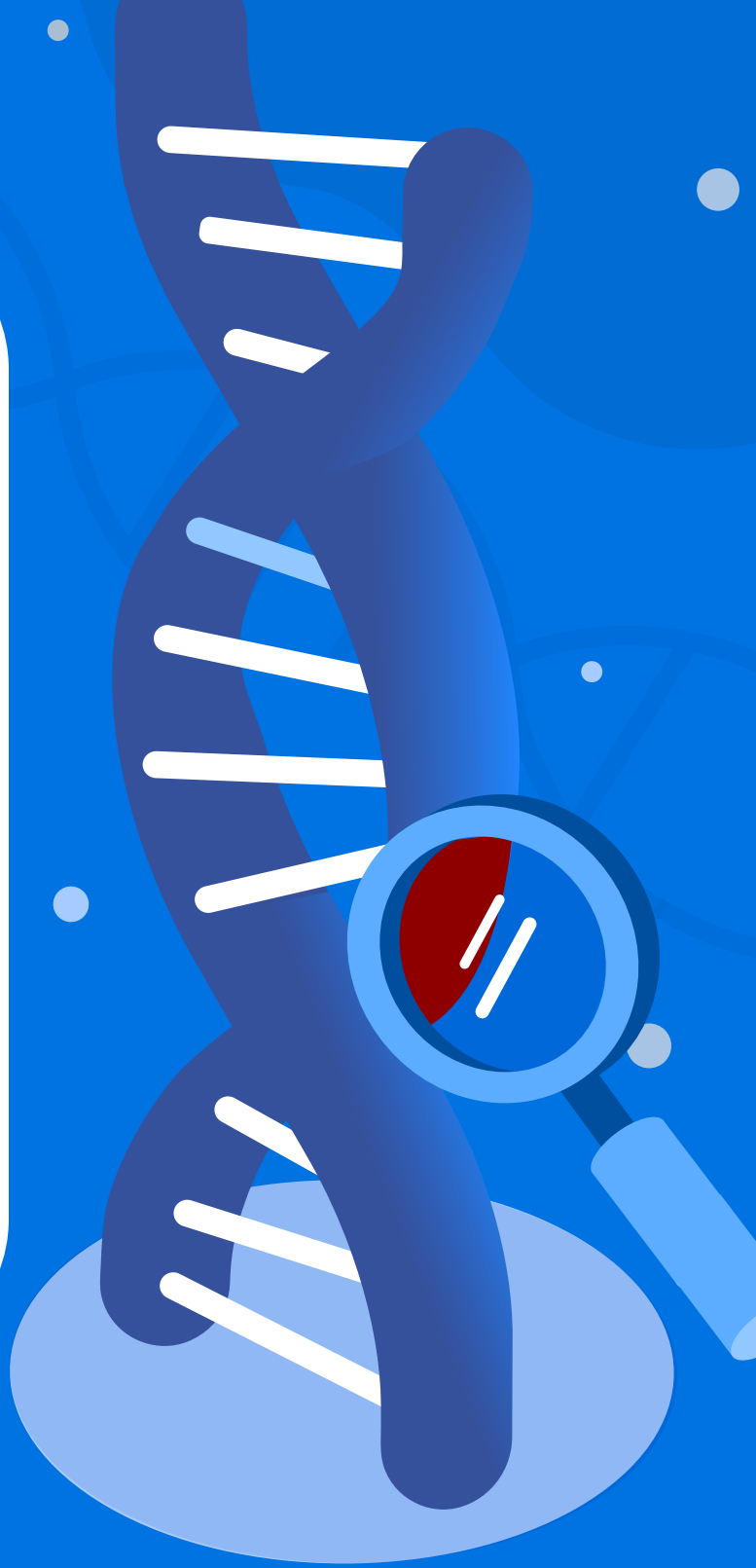
Gene Therapy Report

Q1 2025-Q4 2027

Projected Treatments and Launch Timelines



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2025 PROJECTED LAUNCHES

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 04/29/2025	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 per wound	450 adult and pediatric patients
3Q	**Kresladi** (marnetegragene 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	**rebisufligene etisparvovec** (fka UX111)	Ultragenyx Pharmaceutical	Pending FDA approval 08/18/2025	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 to 4,000 adult and pediatric patients
3Q	**vusolimogene oderparepvec**	Replimune Group Inc.	Pending FDA approval 7/22/2025	New biologic	No	Gene therapy, in vivo	The treatment of unresectable or metastatic cutaneous melanoma after progression on anti-PD1 therapy, in combination with nivolumab (e.g., Opdivo, Opdivo Qvantig)	Injection-Intratumoral, multi-dose	24,700 adult patients
3Q	**zopapogene imadenovec** (fka PRGN2012)	Precigen	Pending FDA approval 8/27/2025	New biologic	Yes	Gene therapy, in vivo	The treatment of recurrent respiratory papillomatosis in adults	Injection-SC, multi-dose	6,400–11,600 adult 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	**clemidsogene lanparvovec** (fka RGX121)	RegenxBio	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type II, also known as Hunter syndrome, in patients ages 5 years and younger	Injection-Intracerebral, one-time	<25 pediatric patients
4Q	**mozafancogene autotemcel** (fka 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

2025 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	**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	**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 to 17 years	Injection-Intrathecal, one-time	3,900 pediatric patients

2026 PROJECTED LAUNCHES

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**isaralgagene 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 adult male patients
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	47,800 adult patients
1Q	**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
2Q	**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 marginal zone lymphoma	Injection-IV, one time	11,000 adult 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 Ta or T1 papillary tumors	Injection-Intravesical, multi-dose	38,800 adult patients

2026 CONTINUED

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
2H	**Elevidys** (delandistrogene moxeparvovec-rokl)	Sarepta Therapeutics	Phase III	Supplemental indication	No	Gene therapy, in vivo	The treatment of patients ages 3 years and younger with Duchenne muscular dystrophy with a confirmed mutation in the DMD gene	Injection-IV, one-time	650 pediatric patients
2H	**RGX202**	RegenxBio	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystrophy in ambulatory patients ages 1 year and older	Injection-IV, one-time	1,100 pediatric patients
3Q	**AMT130**	Uniqure	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of early Huntington's disease in patients ages 25–60 years	Injection-Intracerebral, one-time	7,000 adult 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 per gland	129,000 adult patients
4Q	**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
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 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	5,800–8,900 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

2027 PROJECTED LAUNCHES

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**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
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 Bacillus Calmette-Guérin-refractory bladder cancer with carcinoma in-situ with or without Ta or T1 papillary tumors	Injection-Intravesical, multi-dose	38,800 adult patients
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	6,300–8,400 adult patients
2Q	**NTLA2002**	Intellia Therapeutics	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of hereditary angioedema in adults	Injection-IV, one-time	5,000 adult patients
3Q	**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	105,000 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 2–21 years	Injection-IV, one-time	2,800 pediatric and adult patients
4Q	**cemacabtagene ansegedleucel**	Allogene Therapeutics	Phase II	New biologic	No	CAR T-cell therapy, in vivo	Consolidation therapy in adults with minimal residual disease after response to first-line treatment of large B-cell lymphoma	Injection-IV, one-time	10,200 adult patients

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FDA (U.S. Food and Drug Administration), SC (subcutaneous), IM (intramuscular), IV (intravenous).

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Source: RxPipeline, CVS Health Clinical Affairs. Information current as of March 17, 2025.

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