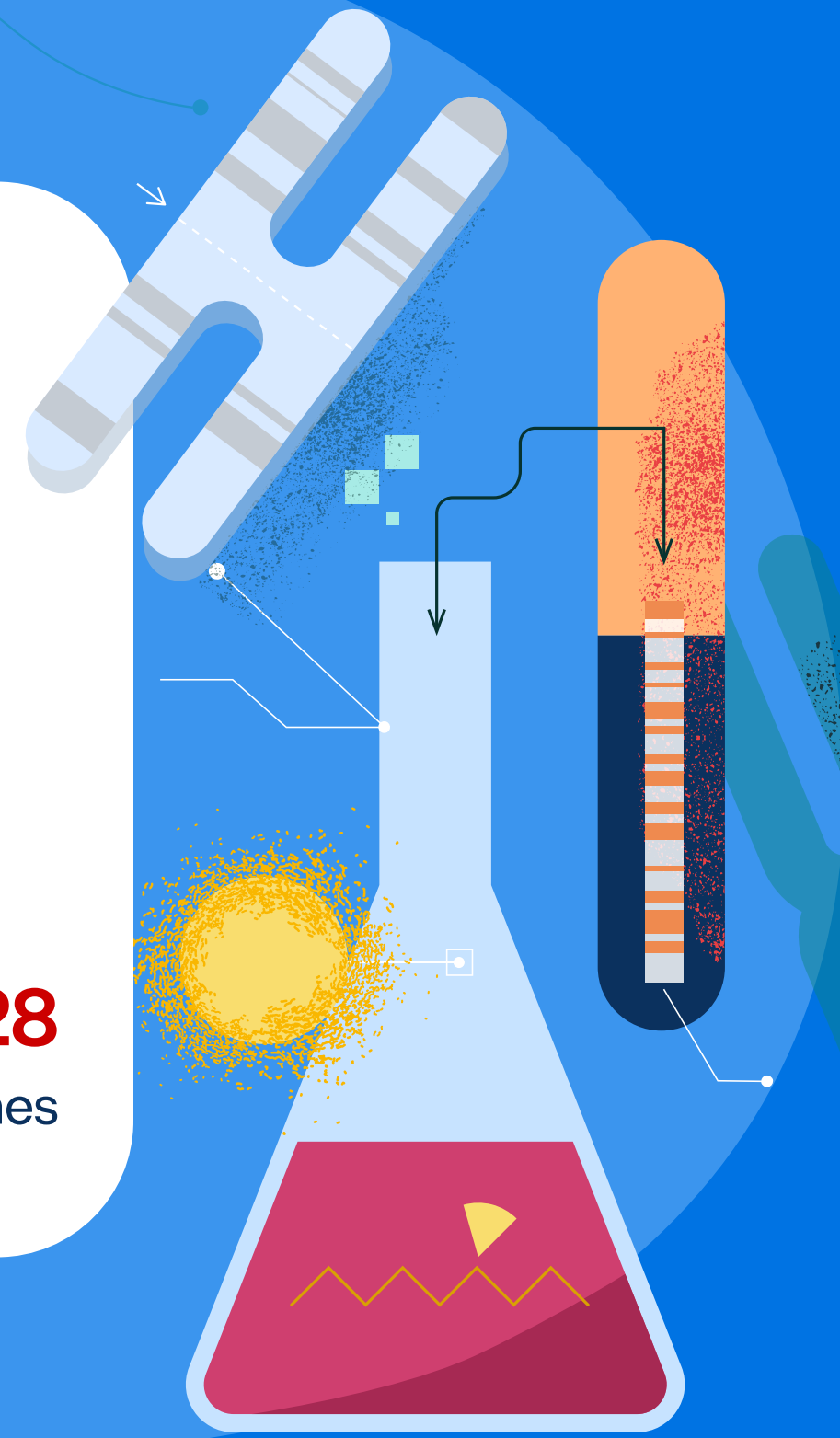
Gene Therapy Report

Q2 2025-Q1 2028

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
3Q	**vusolimogene oderparepvec**	Replimune Group Inc.	Pending FDA approval 7/22/2025	New biologic	Yes	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	78,800 adult patients
3Q	**rebisufligene etisparvovec**	Ultragenyx Pharmaceutical	Pending FDA approval 8/18/2025	New biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo syndrome type A)	Injection–IV, one-time	1,300–4,000 adult and pediatric patients
3Q	**zopapogene imadenovec**	Precigen	Pending FDA approval 8/27/2025	New biologic	Yes	Gene therapy, in vivo	The treatment of recurrent respiratory papillomatosis (RRP) in adults	Injection–SC, multi-dose	1,000–22,000 adult patients
4Q	**clemidsogene lanparvovec**	Nippon Shinyaku/ RegenxBio	Pending FDA approval 11/3/2025	New biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type II (Hunter syndrome) in patients ages 5 years and younger	Injection–intracerebral, one-time	< 25 pediatric patients
4Q	**etuvetidigene autotemcel**	Fondazione Telethon	Pending FDA approval 11/11/2025	New biologic	No	Gene therapy, ex vivo	The treatment of Wiskott-Aldrich syndrome (WAS)	Injection–IV, one-time	400 adult and pediatric patients
4Q	**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

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
1Q	**mozafancogene autotemcel**	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
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
1Q	**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
1Q	**Zolgensma (onasemnogene abeparvovec-xioi)**	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	**anitocabtagene autoleucel**	Arcellx, Inc./ Gilead Sciences/ Kite	Phase II	New biologic	No	Chimeric antigen receptor (CAR) T-cell therapy/ Gene therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma after at least 3 prior systemic therapies in adults	Injection–IV, one-time	51,300 adult patients
2Q	**bidridistrogene xeboparvove**	Sarepta Therapeutics	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of limb girdle muscular dystrophy (LGMD) type 2E/R4 in ambulatory and non-ambulatory patients ages 4 years and older	Injection–IV, one-time	< 1,000 adult and pediatric patients
2Q	**Breyanzi (lisocabtagene maraleucel)**	Bristol-Myers Squibb	Phase II	Supplemental indication	No	Chimeric antigen receptor (CAR) T-cell therapy/ Gene therapy, ex vivo	The treatment of adults with relapsed or refractory marginal zone lymphoma	Injection–IV, one-time	11,700 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
2Q	**cretostimogene grenadenorepvec**	CG Oncology	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	27,900–55,800 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	134,000 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	**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 patients
4Q	**ifezuntirgene inilparvovec (fka AMT130)**	Uniqure	Phase I/II	New biologic	Yes	Gene therapy, in vivo	The treatment of early Huntington's disease in patients ages 25–65 years	Injection–intracerebral, one-time	7,000 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	**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

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
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	27,900–55,800 adult patients
1Q	**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	1,200 pediatric patients
1Q	**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
1H	**avalotcagene ontaparvovec**	Ultragenyx Pharmaceutical	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of ornithine transcarbamylase (OTC) deficiency in patients ages 12 years and older	Injection–IV, one-time	3,600–5,700 adult and pediatric patients
1H	**olvimulogene nanivacirepvec (aka olvi-vec)**	Genelux	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of platinum-resistant or refractory ovarian cancer, in combination with platinum-based chemotherapy and bevacizumab	Injection–intraperitoneal, multi-dose	83,000 adult patients
1H	**surabgene lomparvovec (fka 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
2Q	**NTLA2002**	Intellia Therapeutics	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of hereditary angioedema in adults	Injection–IV, one-time	5,700 adult patients

2027 CONTINUED

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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	107,000 adult patients
3Q	**Tecartus (brexucabtagene autoleucel)**	Gilead Sciences/ Kite	Phase I/II	Supplemental indication	No	Chimeric antigen receptor (CAR) T-cell therapy/ Gene 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
2H	**Elevidys (delandistrogene moxeparvovec-rokl)**	Sarepta Therapeutics	Phase III	Supplemental indication	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystrophy with a confirmed mutation in the DMD gene in patients ages 3 years and younger	Injection–IV, one-time	640 pediatric patients
4Q	**cemacabtagene ansegedleucel**	Allogene Therapeutics	Phase II	New biologic	No	Chimeric antigen receptor (CAR) T-cell therapy/ Gene 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

2028 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
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	10.8 million adult patients
1Q	**RPL301**	Rocket Pharmaceuticals	Phase II	New biologic	No	Gene therapy, ex vivo	The treatment of pyruvate kinase (PK) deficiency in patients ages 8 years and older	Injection–IV, one-time	1,100–2,800 adult and pediatric 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 June 12, 2025.

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