Gene Therapy Report

Q2 2024-Q1 2027

Projected Treatments and Launch Timelines

CVS Health

2024 PROJECTED LAUNCHES

New treatments for lymphoma and leukemia are expected to obtain 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
2Q	**Elevidys** (delandistrogene moxeparvovec-rokl)	Sarepta Therapeutics	Pending FDA approval 06/21/2024	Supplemental indication	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystrophy with a confirmed mutation in the DMD gene	Injection-IV, one-time	8,200 adult and pediatric patients
2Q	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Pending FDA approval FL: 05/23/2024 MCL: 05/31/2024	Supplemental indication	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory follicular lymphoma (FL) or mantle cell lymphoma (MCL)	Injection-IV, one-time	26,300–42,100 adult patients
3Q	**Kresladi** (marnetegragene autotemcel, fka RPL201)	Rocket Pharmaceuticals	Pending FDA approval 06/30/2024	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
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	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory B-cell acute lymphoblastic leukemia in adults	Injection-IV, one-time	21,000 adult patients

2025 PROJECTED LAUNCHES

More than a dozen therapies could receive approval in 2025, including two treatments for retinitis pigmentosa.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**sonpiretigene isteparvovec**	Nanoscope Therapeutics	Phase II	New biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa in adults	Injection-Intraocular, one-time	63,000–72,000 adult patients
1Q	**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
1Q	**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
1Q	**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
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	**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	**cretostimogene grenadenorepvec**	Cold Genesys	Phase III	New biologic	Yes	Gene therapy, in vivo	The treatment of high-risk non-muscle invasive bladder cancer that is unresponsive to Bacillus Calmette-Guerin therapy	Injection-Intravesical, multi-dose	24,500–112,400 adult patients
2Q	**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

2025 CONTINUED

A new treatment for hemophilia A is in the pipeline for 2025.

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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
2Q	**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
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	**fordadistrogene movaparvovec**	Pfizer	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystrophy in ambulatory boys ages 4 to 7 years of age	Injection-IV, one-time	900 pediatric males
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
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	5,500–13,000 adult and pediatric patients

2026 PROJECTED LAUNCHES

The 2026 pipeline includes treatments for knee osteoarthritis and wet macular degeneration.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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	**Invossa** (tonogenchoncel-L)	Kolon Group	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of knee osteoarthritis	Injection-Intra-articular, one-time	15.9 million adult 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	**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

2026 CONTINUED

A new treatment for age-related macular degeneration could benefit up to 2 million patients.

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2H	**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	**detalimogene voraplasmid**	enGene Holdings	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of high-risk non-muscle invasive bladder cancer that is unresponsive to Bacillus Calmette-Guerin therapy	Injection-Intravesical, multi-dose	204,000 adult 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

Treatments for hereditary angioedema and Wilson's disease are in the 2027 pipeline.

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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

The high cost of gene and genetically modified cellular therapies can be a lot to absorb, even if they have the potential to offset future health care costs.

Read our Insights post, "**Minimizing the financial impact of breakthrough therapies**," to learn about a comprehensive approach to managing costs.

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