Gene Therapy Report

Q1 2024-Q4 2026

Projected Treatments and Launch Timelines

CVS Health

2024 PROJECTED LAUNCHES

The 2024 pipeline includes treatments for multiple myeloma and leukemia.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Pending FDA approval 03/14/2024	Supplemental indication	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma in adults who have received a prior Bruton tyrosine kinase inhibitor and B-cell lymphoma 2 inhibitor	Injection-IV, one-time	145,000 additional adult patients
1Q	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Pending FDA approval 03/16/2024	Supplemental indication	No	CAR T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory multiple myeloma who have received 2 to 3 prior lines of therapy	Injection-IV, one-time	57,000 additional adult patients
1Q	**atidarsagene autotemcel**	Orchard Therapeutics	Pending FDA approval 03/18/2024	New biologic	No	Gene therapy, ex vivo	The treatment of metachromatic leukodystrophy in patients ages 6 years and younger with late infantile form without clinical manifestations, and in patients ages 6 years and younger with early juvenile form without clinical manifestations or with early clinical manifestations of the disease	Injection-IV, one-time	100–600 pediatric patients
1Q	**Kresladi** (marnetegragene autotemcel, fka RPL201)	Rocket Pharmaceuticals	Pending FDA approval 03/31/2024	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
2Q	**Carvykti** (ciltacabtagene autoleucel)	Janssen Pharmaceuticals/ Johnson & Johnson	Pending FDA approval 04/05/2024	Supplemental indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory multiple myeloma in patients who have received 1 to 3 prior lines of therapy	Injection-IV, one-time	130,000 additional adult patients
2Q	**fidanacogene elaparvovec**	Pfizer/Spark Therapeutics	Pending FDA approval 04/27/2024	New biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia B in adults	Injection-IV, one-time	1,300 adult patients

2024 CONTINUED

More than a dozen new therapies could gain approval in 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	**prademagene zamikeracel**	Abeona Therapeutics	Pending FDA approval 05/25/2024	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	**Elevidys** (delandistrogene moxeparvovec-rokl)	Sarepta Therapeutics	Pending FDA approval 06/22/2024	Supplemental indication	No	Gene therapy, in vivo	The treatment of ambulatory patients ages 6 to 7 years with Duchenne muscular dystrophy with a confirmed mutation in the DMD gene	Injection-IV, one-time	370 additional pediatric males
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
4Q	**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	450 adult and pediatric patients
4Q	**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
4Q	**UX111**	Abeona Therapeutics/ 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	**zevorcabtagene autoleucel**	CARsgen Therapeutics	Phase I/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	45,000 adult patients

2025 PROJECTED LAUNCHES

New treatments for hemophilia A and cutaneous melanoma could be approved in 2025.

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1Q	**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 follicular lymphoma or marginal zone lymphoma	Injection-IV, one-time	35,000–51,000 additional adult patients
1Q	**Upstaza** (eladocagene exuparvovec)	PTC Therapeutics	Phase II	New biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in patients ages 17 years and younger	Injection-Intracerebral, one-time	<50 pediatric patients
2Q	**fordadistrogene movaparvovec**	Pfizer	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of ambulatory patients with Duchenne muscular dystrophy	Injection-IV, one-time	850 pediatric males
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
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
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	13,000 adult patients
3Q	**giroctocogene fitelparvovec**	Pfizer/Sangamo BioSciences	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of hemophilia A in adults	Injection-IV, one-time	3,000 adult patients
3Q	**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	**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	2,800–6,400 adult and pediatric patients

2026 PROJECTED LAUNCHES

A gene therapy to treat knee osteoarthritis, which affects millions, could get approval in 2026.

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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	45,000 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
2Q	**VTX801**	Pfizer/Vivet Therapeutics	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of hepatolenticular degeneration (Wilson's Disease) in adults	Injection-IV, one-time	6,500–8,600 adult patients
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	3,600–5,700 adult and pediatric patients

 Source: RxPipeline, CVS Health Clinical Affairs. Information current as of February 21, 2024.

2026 CONTINUED

The 2026 pipeline includes a gene therapy for wet age-related macular degeneration.

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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

These breakthrough therapies offer great clinical potential, though at a high cost. Plan sponsors have a lot to consider in terms of coverage, affordability, and long-term value.

Read our Insights post, "**Gene and Genetically Modified Cellular Therapies: Coverage Considerations for Payors**," to learn more.