Gene Therapy Report

Q4 2023-Q4 2027

Projected Treatments and Launch Timelines

CVS Health

2023 PROJECTED LAUNCHES

Gene therapies for sickle cell disease may soon be approved.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
4Q	**exagamglogene autotemcel**	CRISPR Therapeutics/ Vertex Pharmaceuticals	Pending FDA approval 12/08/2023	New biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in patients ages 12 years and older with recurrent vaso-occlusive crises	Injection-IV, one-time	16,300 adult and pediatric patients
4Q	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Pending FDA approval 12/16/2023	Supplemental indication	No	Chimeric antigen receptor (CAR) T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory multiple myeloma who have received an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody	Injection-IV, one-time	77,700 adult patients

2024 PROJECTED LAUNCHES

2024 could see the first CAR T-cell therapy approved for chronic lymphocytic 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	**lovotibeglogene autotemcel** (beta-globin gene therapy)	Bluebird Bio	Pending FDA approval 12/20/2023	New biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in patients ages 12 years and older who have a history of vaso-occlusive events	Injection-IV, one-time	16,300 adult and pediatric patients
1Q	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Pending FDA approval 03/14/2024	Supplemental indication	No	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	141,000 adult patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 1, 2023.

2024 CONTINUED

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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	170–700 pediatric patients worldwide
1Q	**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	300 pediatric patients worldwide
2Q	**exagamglogene autotemcel**	CRISPR Therapeutics/ Vertex Pharmaceuticals	Pending FDA approval 03/30/2024	New biologic	No	Gene therapy, ex vivo	The treatment of transfusion-dependent beta thalassemia in patients ages 12 years and older	Injection-IV, one-time	1,500 adult and pediatric patients
2Q	**Carvykti** (ciltacabtagene autoleucel)	Janssen Pharmaceuticals/ Johnson & Johnson	Pending FDA approval 04/06/2024	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	145,000 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
2Q	**prademagene zamikeracel** (fka EB101)	Abeona Therapeutics	Pending FDA approval 05/26/2024	New biologic	Yes	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients ages 6 years and older	Topical, 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	450 adult and pediatric patients
3Q	**Elevidys** (delandistrogene moxeparvovec-rokl)	Sarepta Therapeutics	Phase III	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 pediatric males

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 1, 2023.

2024 CONTINUED

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
3Q	**Engensis** (donaperminogene seltoplasmid)	Helixmith	Phase III	New biologic	No	Gene therapy, in vivo	The treatment of diabetic peripheral neuropathy in adults	Injection-IM, multi-dose	5.5–11.2 million adult patients
3Q	**obecabtagene autoleucel**	Autolus Therapeutics	Phase I/II	New biologic	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory acute lymphoblastic leukemia in adults	Injection-IV, one-time	22,300–27,900 adult patients
3Q	**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	**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	34,000–49,000 adult patients
4Q	**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	100 pediatric patients worldwide
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	170 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 in adults	Injection-IV, one-time	42,700 adult patients

Plan sponsors should pay close attention to new treatment options with potential across key therapeutic areas. Read our Insights post, **"Next in Gene Therapy: 2024 Roundup,"** to learn more.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 1, 2023.

2025 PROJECTED LAUNCHES

An additional gene therapy for hemophilia A may be approved this year.

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2Q	**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
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 ages less than 5 years	Injection-IV, one-time	40 male newborns per year
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	**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	1,600–3,800 adult and pediatric 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	**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
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 2–17 years	Injection-Intrathecal, one-time	3,900 pediatric patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 1, 2023.

2026 PROJECTED LAUNCHES

The 2026 pipeline includes treatments for wet age-related macular degeneration, knee osteoarthritis, and prostate cancer.

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1H	**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
1Q	**ALLO501A**	Allogene Therapeutics	Phase I/II	New biologic	No	CAR T-cell therapy, in vivo	The treatment of relapsed or refractory large B-cell lymphoma in adults who have received at least 2 prior lines of therapy	Injection-IV, one-time	4,200–12,700 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
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

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2027 PROJECTED LAUNCHES

Two off-the-shelf CAR T-cell therapies may be approved.

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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	6,500–8,600 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 2–21 years	Injection-IV, one-time	260 pediatric patients
4Q	**CTX110**	CRISPR Therapeutics	Phase I/II	New biologic	No	CAR T-cell therapy, in vivo	The treatment of relapsed or refractory large B-cell lymphoma in adults who have received at least 2 prior lines of therapy	Injection-IV, multi-dose	4,200–12,700 adult patients

Information compiled from external sources. Manufacturer drug launch dates are subject to change without notice. Some products may not be dispensed by CVS Specialty Pharmacy.
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Source: RxPipeline, CVS Health Clinical Affairs. Information current as of November 1, 2023.

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