Gene Therapy Report

Q3 2023-Q2 2027

Projected Treatments and Approval Timelines

CVS Health

2023 PROJECTED APPROVALS

The 2023 pipeline includes the first gene therapies to treat sickle cell disease.

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	Injection-IV, one-time	42,500 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	78,000 adult patients
4Q	**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	Injection-IV, one-time	42,500 adult and pediatric patients

Gene therapy to treat sickle cell disease (SCD) — which affects millions of people worldwide, including about 100,000 Americans — could soon be a reality. Two potential therapies could receive U.S. Food & Drug Administration approval by the end of the year.

Read our Insights post, **"Gene Therapy for Sickle Cell Disease,"** to learn more.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of August 17, 2023.

2024 PROJECTED APPROVALS

A gene therapy to treat diabetic peripheral neuropathy could get 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	**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	2,600 adult patients
2Q	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	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
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	400 adult and pediatric patients
2Q	**EB101**	Abeona Therapeutics	Phase III	New biologic	Yes	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients ages 6 years and older	Topical, one-time	400 adult and pediatric patients
2Q	**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
2Q	**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	50,000 adult patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of August 17, 2023.

2024 CONTINUED

Several CAR T-cell therapies are in the 2024 pipeline.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
2Q	**RPL201**	Rocket Pharmaceuticals	Phase I/II	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	**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	200 pediatric patients
2H	**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
2H	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Phase I/II	Supplemental indication	No	CAR T-cell therapy, ex vivo	The treatment of relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma in adults	Injection-IV, one-time	140,000 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
3Q	**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	**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	43,000 adult patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of August 17, 2023.

2025 PROJECTED APPROVALS

A new treatment for hemophilia A could be approved in 2025.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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	6,000 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	4,000 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	6,000 adult and pediatric patients
2Q	**resamirigene bilparvovec**	Astellas Pharma/ Audentes Therapeutics	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	8,000 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	6,000 adult and pediatric patients
3Q	**RGX121**	RegenxBio	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment for mucopolysaccharidosis type II, also known as Hunter syndrome, in patients ages 5 years and under	Injection-Intracerebral, one-time	< 25 pediatric patients
4Q	**Zolgensma** (onasemnogene abeparvovec-xioi)	AveXis/Novartis	Phase I	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	4,000 pediatric patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of August 17, 2023.

2026 PROJECTED APPROVALS

2026 could see the approval of a gene therapy to treat knee osteoarthritis.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
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	4,300 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	16 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	73,000 adult patients
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
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	1.3 million adult patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of August 17, 2023.

2027 PROJECTED APPROVALS

The 2027 pipeline includes a new therapy for Wilson's disease.

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**UX701**	Ultragenyx Pharmaceutical	Phase I/II	New biologic	No	Gene therapy, in vivo	The treatment of Wilson's disease	Injection-IV, one-time	8,500 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	300 pediatric 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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