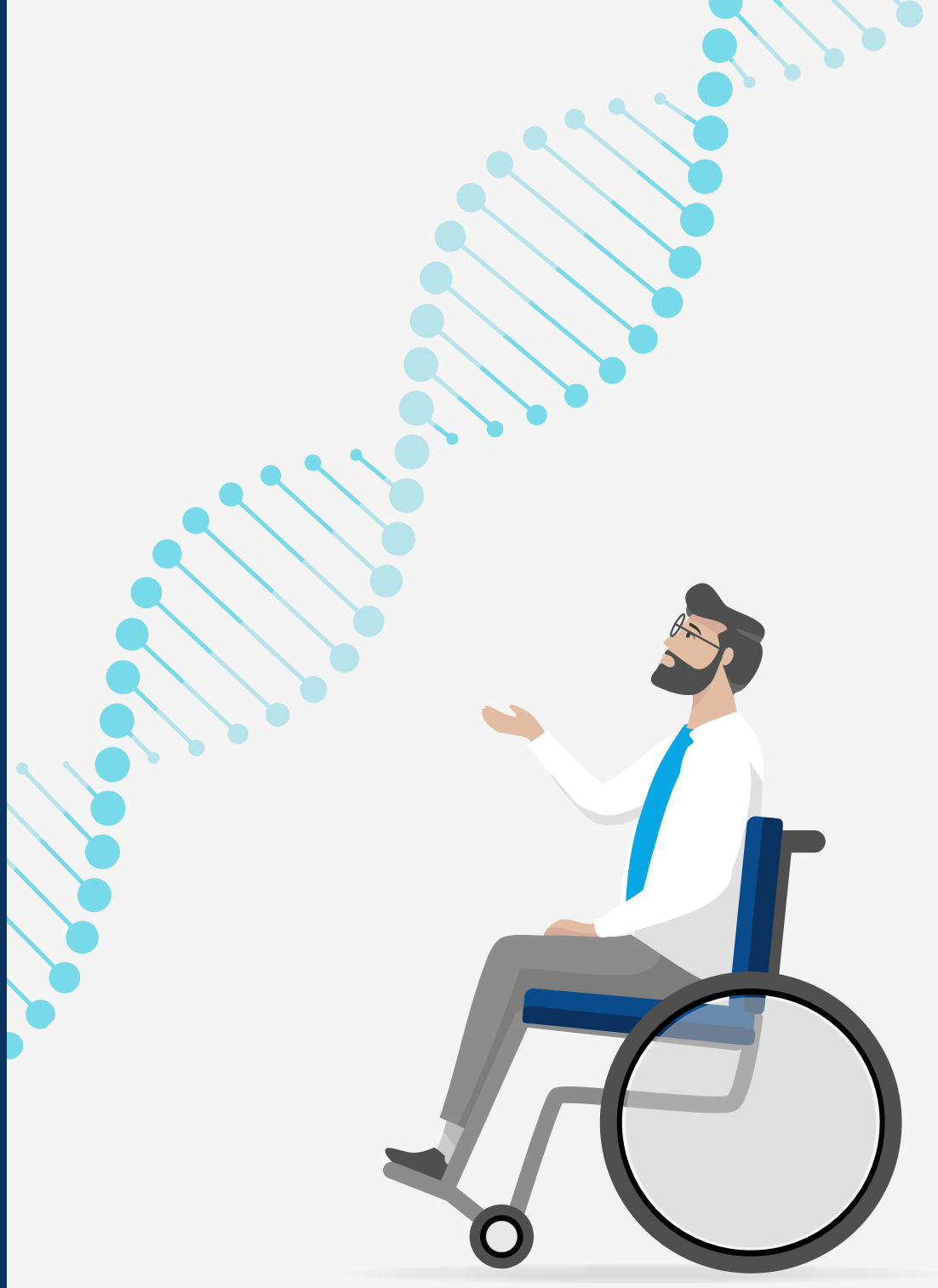
GENE THERAPY PIPELINE
3Q 2022–1Q 2027

Projected Treatments and Approval Timelines



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Projected Launch Year 2022

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
3Q	**Zynteglo** (betibeglogene autotemcel)	Bluebird Bio	Pending FDA approval	New Biologic	Yes	Gene therapy, ex vivo	The treatment of transfusion-dependent beta-thalassemia	Injection-IV, one-time	08/19/2022	1,450 adult and pediatric patients
4Q	**LentiD** (elivaldogene autotemcel)	Bluebird Bio	Pending FDA approval	New Biologic	Yes	Gene therapy, ex vivo	The treatment of early cerebral adrenoleukodystrophy in males aged 17 years and younger who do not have a HLA-matched sibling hematopoietic stem cell donor	Injection-IV, one-time	09/16/2022	700 pediatric males
4Q	**EtranaDez** (etranacogene dezaparvovec)	CSL Behring/ Uniqure	Pending FDA approval	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia B in adults	Injection-IV, one-time	11/24/2022	2,600 adult patients

The first gene therapy for hemophilia B could be approved by the end of 2022.

Projected Launch Year 2023

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**Breyanzi** (lisocabtagene maraleucel)	Bristol-Myers Squibb	Phase I/II	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	Injection-IV, one-time		135,000 adult patients
1Q	**Vyjuvek** (beremagene geperpavec)	Krystal Biotech	Pending FDA approval	New Biologic	No	Gene therapy, in vivo	The treatment of dystrophic epidermolysis bullosa in patients aged 6 months and older	Topical, multi-dose	02/22/2023	900 adult and pediatric patients
1Q	**Roctavian** (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time		7,500 adult patients
1Q	**Instiladrin** (nadofaragene firadenovec)	FKD Therapies/ Ferring Pharmaceuticals	Pending FDA approval	New Biologic	Yes	Gene therapy, in vivo	The treatment of high-grade, non-muscle invasive, bacillus Calmette-Guérin (BCG)-refractory bladder cancer in adults	Injection-Intravesical, multi-dose		60,000 adult patients
3Q	**EB101**	Abeona Therapeutics	Phase III	New Biologic	Yes	Gene therapy, ex vivo	The treatment of recessive dystrophic epidermolysis bullosa in patients aged 6 years and older	Topical, one-time		400 adult and pediatric patients
3Q	**JNJ64400141**	Janssen Pharmaceuticals/ Johnson & Johnson	Phase III	New Biologic	Yes	Gene therapy, in vivo	The prevention of respiratory syncytial virus-mediated lower respiratory tract disease in adults aged 60 years or older	Injection-IM, multi-dose		34 million adult patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of July 12, 2022.

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Projected Launch Year 2023 (continued)

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
3Q	**atidarsagene autotemcel**	Orchard Therapeutics	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of metachromatic leukodystrophy in patients aged 6 years or younger with late infantile form without clinical manifestations, and in patients aged 6 years or younger with early juvenile form without clinical manifestations or with early clinical manifestations of the disease	Injection-IV, one-time		400–1,700 pediatric patients worldwide
3Q	**eladocagene exuparvovec**	PTC Therapeutics	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of aromatic L-amino acid decarboxylase deficiency in patients aged 17 years and younger	Injection-Intracerebral, one-time		100 pediatric patients worldwide
3Q	**exagamglogene autotemcel** (fka CTX001)	CRISPR Therapeutics/ Vertex Pharmaceuticals	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of transfusion-dependent beta thalassemia (TDT) in patients aged 12 years and older	Injection-IV, one-time		1,000 adult and pediatric patients
3Q	**exagamglogene autotemcel** (fka CTX001)	CRISPR Therapeutics/ Vertex Pharmaceuticals	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in patients aged 12 years and older	Injection-IV, one-time		58,000 adult and pediatric patients
4Q	**olenasufligene relduparvovec**	Lysogene	Phase II/III	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo type A) in pediatrics aged 6 months and older	Injection-Intracerebral, one-time		200–1,800 pediatric patients
4Q	**lovotibeglogene autotemcel** (beta-globin gene therapy)	Bluebird Bio	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of sickle cell disease in adults and pediatrics	Injection-IV, one-time		58,000 adult and pediatric patients
4Q	**RPL102**	Rocket Pharmaceuticals	Phase II	New Biologic	No	Gene therapy, ex vivo	The treatment of Fanconi anemia in patients aged 1–17 years	Injection-IV, one-time		<1,000 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 aged 3 years and older	Injection-Intraocular, one-time		54,600–62,400 adult and pediatric patients
4Q	**RPL201**	Rocket Pharmaceuticals	Phase I/II	New Biologic	No	Gene therapy, ex vivo	The treatment of severe leukocyte adhesion deficiency type 1 in pediatrics aged 3 months and older	Injection-IV, one-time		300 pediatric patients worldwide

Treatments seeking approval in 2023 include the first expected sickle cell disease gene therapy.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of July 12, 2022.

Projected Launch Year 2024

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Phase III	Supplemental Indication	Yes	CAR T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory multiple myeloma after 2 or 3 prior lines of therapy	Injection-IV, one-time		9,000 adult patients
1Q	**fidanacogene elaparvovec**	Pfizer/Spark Therapeutics	Phase III	New Biologic	Yes	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		2,600 adult patients
1Q	**Tavo** (tavokinogene telsaplasmid)	Merck/ OncoSec	Phase II	New Biologic	No	Gene therapy, in vivo	The treatment of advanced or metastatic malignant melanoma in adults whose cancer has progressed on a checkpoint inhibitor, in combination with Keytruda (pembrolizumab)	Injection, multi-dose		177,000 adult patients
1Q	**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 aged younger than 5 years	Injection-IV, one-time		40 male newborns per year
1Q	**ofranergene obadenovec**	VBL Therapeutics	Phase III	New Biologic	No	Angiogenesis inhibitor/Gene therapy, in vivo	The treatment of recurrent platinum-resistant ovarian cancer, in combination with paclitaxel	Injection-IV, multi-dose		15,000 adult females
1Q	**UX111** (fka ABO-102)	Abeona Therapeutics/ Ultragenyx Pharmaceutical	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of mucopolysaccharidosis type 3A (also known as Sanfilippo syndrome type A)	Injection-IV, one-time		200–1,800 pediatric patients
2H	**laruparetigene zosaparvovec**	Applied Genetic Technologies Corp.	Phase II/III	New Biologic	No	Gene therapy, in vivo	The treatment of X-linked retinitis pigmentosa in males aged 8–50 years with a mutation in the RPGR gene	Injection-Intraocular, one-time		17,000–22,000 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	**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		7.1–13.5 million adult patients
2Q	**GS030**	GenSight Biologics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of retinitis pigmentosa in adults	Injection-Intraocular, one-time		65,000 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		30,000 adult 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 aged 7 years and older	Injection-Intradermal, multi-dose		400 adult and pediatric patients
3Q	**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		125,000 adult patients
4Q	**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 aged 2–21 years	Injection-IV, one-time		8,000 pediatric patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of July 12, 2022.

Projected Launch Year 2024 (continued)

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
4Q	**delandistrogene moxeparvovec**	Sarepta Therapeutics	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystophy in ambulatory patients	Injection-IV, one-time		4,000 pediatric males
4Q	**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		7,500 adult patients
4Q	**pariglasgene brecaparvovec**	Ultragenyx Pharmaceutical	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of glycogen storage disease type 1a in patients aged 8 years and older	Injection-IV, one-time		6,000 adult and pediatric patients

Notable among the 2024 pipeline are therapies for Duchenne muscular dystrophy.

Projected Launch Year 2025

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
1H	**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
1H	**verbrinacogene setparvovec**	Freeline Therapeutics	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment of hemophilia B in adults	Injection-IV, one-time		2,600 adult patients
2Q	**nadofaragene firadenovec**	Trizell	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of malignant pleural mesothelioma	Injection-Intrapulmonary, one-time		2,400 adults patients per year
4Q	**Invossa (tonogenchoncel-L)**	Kolon Group	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of knee osteoarthritis	Injection-Intra-articular, one-time		13 million adult 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		126,000–197,000 adult patients

Conditions targeted by gene therapies seeking approval in 2025 include neovascular (wet) age-related macular degeneration.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of July 12, 2022.

Projected Launch Year 2026

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	ESTIMATED POTENTIAL U.S. CANDIDATES
1Q	**avalotcagene ontaparvovec**	Ultragenyx Pharmaceutical	Phase III	New Biologic	No	Gene therapy, in vivo	The treatment of ornithine transcarbamylase (OTC) deficiency in patients aged 12 years and older	Injection-IV, one-time		4,300 adult and pediatric patients

This gene therapy is aimed at improving the quality of life for patients with a rare metabolic disorder.

Projected Launch Year 2027

QUARTER	THERAPY NAME	MANUFACTURER	PHASE OF DEVELOPMENT	TYPE	BREAKTHROUGH THERAPY DESIGNATION	DRUG CLASS	INDICATION	ROUTE OF ADMINISTRATION & FREQUENCY	FDA DECISION DATE	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 in adults	Injection-IV, one-time		8,500 adult patients

The treatment options for Wilson disease patients could expand with a new treatment in 2027, which can potentially reduce the need for liver transplants for those with severe disease.

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 July 12, 2022.

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