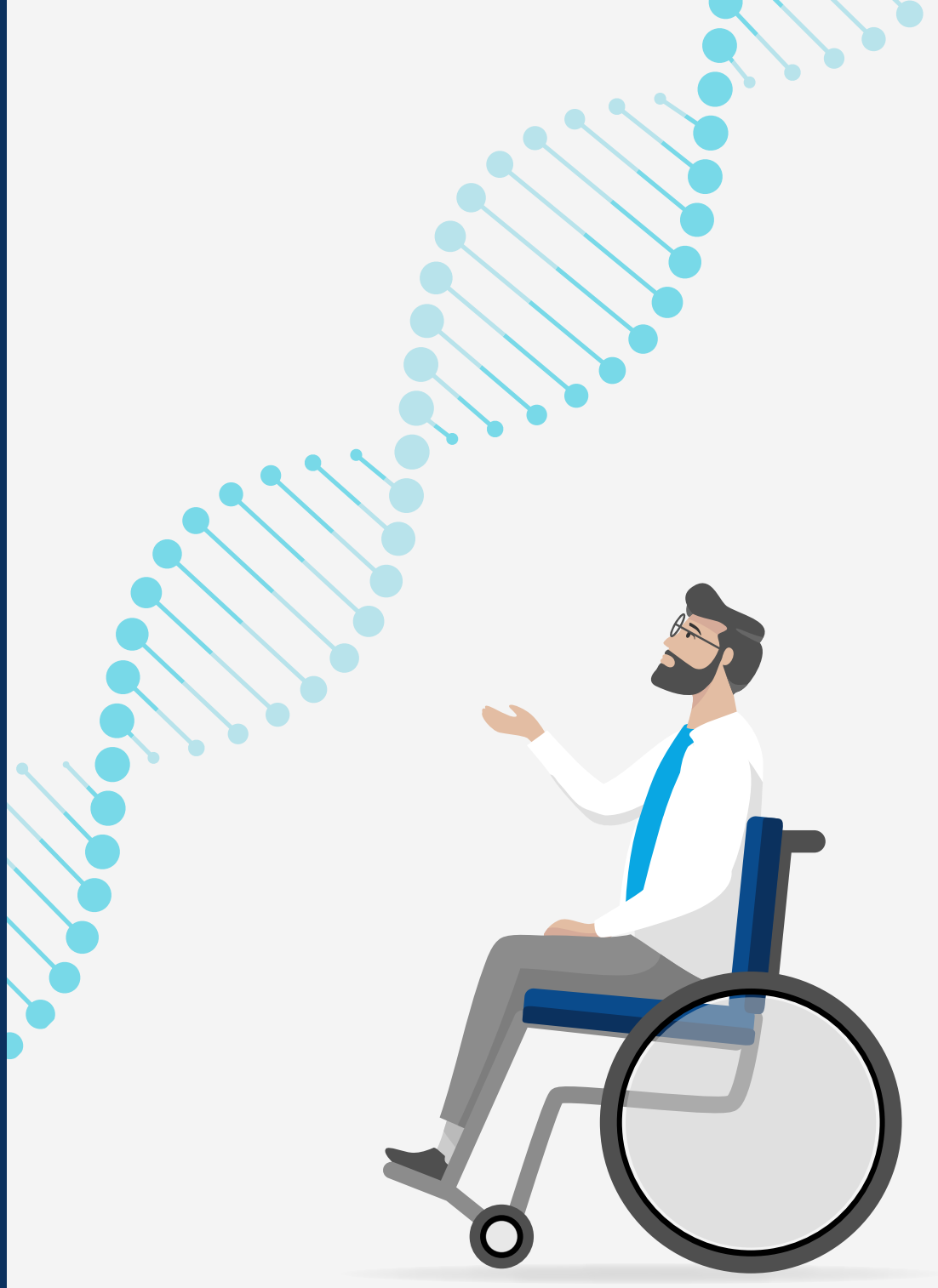
GENE THERAPY PIPELINE
4Q 2022–1Q 2027

Novel Treatment Options to Tackle Rare, Costly Conditions



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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
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 people living with hemophilia B is set to win approval at the end of the year.

Projected Launch Year 2023

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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	2/17/2023	1,000 adult and pediatric patients
1Q	**Roctavian** (valoctocogene roxaparvovec)	BioMarin Pharmaceutical	Pending FDA approval	New Biologic	Yes	Gene therapy, in vivo	The treatment of severe hemophilia A in adults	Injection-IV, one-time	3/31/2023	8,000 adult patients
2Q	**delandistrogene moxeparvovec**	Sarepta Therapeutics	Pending FDA approval	New Biologic	No	Gene therapy, in vivo	The treatment of Duchenne muscular dystophy in ambulatory patients	Injection-IV, one-time	5/29/2023	4,500 pediatric males
2Q	**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
2H	**Abecma** (idecabtagene vicleucel)	Bluebird Bio/ Bristol-Myers Squibb/Celgene	Phase III	Supplemental Indication	Yes	Chimeric antigen receptor (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		50,000 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		135,000 adult patients
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		150–700 pediatric patients

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 26, 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	**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		450 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
4Q	**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		800 pediatric patients
4Q	**exagamglogene autotemcel** (fka CTX001)	CRISPR Therapeutics/ Vertex Pharmaceuticals	Phase III	New Biologic	No	Gene therapy, ex vivo	The treatment of transfusion-dependent beta thalassemia in patients aged 12 years and older	Injection-IV, one-time		1,000 adult and pediatric patients
4Q	**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	**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	**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	**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		150–1,500 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		< 25 pediatric patients

A gene therapy for Duchenne muscular dystrophy and for hemophilia A may become available in 2023.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 26, 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
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	**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	**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	**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
1H	**zevorcabtagene autoleucel**	CARsgen Therapeutics	Phase I/II	New Biologic	No	CAR T-cell therapy, ex vivo	The treatment of adults with relapsed or refractory multiple myeloma after 3 prior lines of therapy	Injection-IV, one-time		40,000 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
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		4,500 pediatric males
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		70,000 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
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		1,500–4,000 adult and pediatric males
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		7.1–13.5 million adult 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

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 26, 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	**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		3,000 adult and pediatric 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

The 2024 pipeline includes therapies for advanced malignant melanoma, retinitis pigmentosa, and diabetic peripheral neuropathy.

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
1Q	**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		2,500–6,000 adult and pediatric patients
1H	**RGX121**	RegenxBio	Phase I/II	New Biologic	No	Gene therapy, in vivo	The treatment for mucopolysaccharidosis type II, also known as Hunter syndrome in pediatrics aged 5 years and less	Injection-Intracerebral, one-time		< 25 pediatric patients
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		1.1 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	**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

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 26, 2022.

Projected Launch Year 2025 (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
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 adult 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

Hunter syndrome and neovascular (wet) age-related macular degeneration are two conditions targeted by gene therapies looking for approval in 2025.

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 deficiency in patients aged 12 years and older	Injection-IV, one-time		4,300 adult and pediatric patients
1Q	**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 aged 2 to 18 years	Injection-Intrathecal, one-time		4,000 pediatric patients
2Q	**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

Notable among the pipeline for 2026 are treatments for pediatric patients.

Source: RxPipeline, CVS Health Clinical Affairs. Information current as of October 26, 2022.

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

With an active gene therapy clinical trial for Wilson disease, a new novel treatment could be approved in 2027.

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 October 26, 2022.

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