



# Gene Therapy Report

Projected treatments  
and launch timelines

Q2 2026–Q1 2029

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## 2026 Projected launches

Therapy name	<b>Casgevy (exagamglogene autotemcel)</b>
Manufacturer	CRISPR Therapeutics/Vertex Pharmaceuticals
Phase of development	Pending FDA approval
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo/stem cell therapy, autologous
Indication	The treatment of sickle cell disease in patients ages 5–11 years with recurrent vaso-occlusive crises
Route of administration and frequency	Injection-IV, one-time
FDA decision date	7/31/26
Projected launch	3Q 2026
Estimated potential U.S. candidates	4,400 pediatric patients

## 2026 Projected launches

Therapy name	<b>Casgevy (exagamglogene autotemcel)</b>
Manufacturer	CRISPR Therapeutics/Vertex Pharmaceuticals
Phase of development	Pending FDA approval
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo/stem cell therapy, autologous
Indication	The treatment of transfusion-dependent beta-thalassemia in patients ages 5–11 years
Route of administration and frequency	Injection-IV, one-time
FDA decision date	7/31/26
Projected launch	3Q 2026
Estimated potential U.S. candidates	120 pediatric patients

## 2026 Projected launches

Therapy name	<b>pariglasgene brecaparvovec</b>
Manufacturer	Ultragenyx Pharmaceutical
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of glycogen storage disease type 1a in patients ages 8 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	8/23/26
Projected launch	Q3 2026
Estimated potential U.S. candidates	2,500 adult and pediatric patients

## 2026 Projected launches

Therapy name	<b>rebisufligene etisparvovec</b>
Manufacturer	Ultragenyx Pharmaceutical
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of mucopolysaccharidosis type IIIA (also known as Sanfilippo syndrome type A)
Route of administration and frequency	Injection-IV, one-time
FDA decision date	9/19/26
Projected launch	Q3 2026
Estimated potential U.S. candidates	820–4,100 adult and pediatric patients

## 2026 Projected launches

Therapy name	<b>anitocabtagene autoleucel</b>
Manufacturer	Arcellx, Inc./Gilead Sciences/Kite
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of relapsed or refractory multiple myeloma in adults treated with at least 3 prior lines of therapy
Route of administration and frequency	Injection-IV, one-time
FDA decision date	12/23/26
Projected launch	Q4 2026
Estimated potential U.S. candidates	54,100 adult patients

## 2027 Projected launches

Therapy name	<b>arlocabtagene autoleucel</b>
Manufacturer	Bristol-Myers Squibb/Celgene
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of relapsed or refractory multiple myeloma in adults treated with at least 3 prior lines of therapy
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	1H 2027
Estimated potential U.S. candidates	54,100 adult patients

## 2027 Projected launches

Therapy name	<b>bidridistrogene xeboparvovec</b>
Manufacturer	Sarepta Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of limb girdle muscular dystrophy type 2E/R4 in ambulatory and non-ambulatory patients ages 4 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2027
Estimated potential U.S. candidates	<1,000 adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>isaralgagene civaparvovec</b>
Manufacturer	Sangamo BioSciences
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Fabry disease in adults
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2027
Estimated potential U.S. candidates	3,300 adult patients

## 2027 Projected launches

Therapy name	<b>Mogenry (sonpiretigene isteparvovec)</b>
Manufacturer	Nanoscope Therapeutics
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of retinitis pigmentosa in adults
Route of administration and frequency	Injection-Intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q1 2027
Estimated potential U.S. candidates	104,000 adult patients

## 2027 Projected launches

Therapy name	<b>AAVAIPL1</b>
Manufacturer	Eli Lilly/MeiraGTx
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Leber congenital amaurosis 4 caused by AIPL1 gene mutations in patients ages 3 years and younger
Route of administration and frequency	Injection-Intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	140–340 pediatric patients

## 2027 Projected launches

Therapy name	<b>revlucabtagene autoleucel</b>
Manufacturer	Immix Biopharma
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of relapsed or refractory light chain amyloidosis
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	11,500 adult patients

## 2027 Projected launches

Therapy name	<b>botaretigene sparoparvovec</b>
Manufacturer	MeiraGTx
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of X-linked retinitis pigmentosa due to RPGR mutations in patients ages 3 years and older
Route of administration and frequency	Injection-subretinal, one-time per eye
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	4,500–8,800 male adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>lonvoguran ziclumeran (NTLA-2002)</b>
Manufacturer	Intellia Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo/Kallikrein inhibitor
Indication	The treatment of hereditary angioedema in patients ages 16 and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	5,500 adult and adolescent patients

## 2027 Projected launches

Therapy name	<b>olvimulogene nanivacirepvec</b>
Manufacturer	Genelux
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of adults with platinum-resistant or refractory ovarian cancer, in combination with platinum-based chemotherapy and bevacizumab
Route of administration and frequency	Injection-intraperitoneal, one-time
FDA decision date	TBD
Projected launch	2H 2027
Estimated potential U.S. candidates	40,800 adult patients

## 2027 Projected launches

Therapy name	<b>cretostimogene grenadenorepvec</b>
Manufacturer	CG Oncology
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of high-risk, non-muscle invasive, Bacillus Calmette-Guerin-refractory bladder cancer with carcinoma in-situ with or without Ta or T1 papillary tumors
Route of administration and frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	15,000–20,000 adult patients

## 2027 Projected launches

Therapy name	<b>mivocabtagene autoleucel</b>
Manufacturer	Kyverna Therapeutics
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of stiff person syndrome in adults
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	3,600–5,600 adult patients

## 2027 Projected launches

Therapy name	<b>detalimogene voraplasmid</b>
Manufacturer	enGene Holdings
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of high-risk non-muscle invasive bladder cancer with carcinoma in situ with or without papillary tumors that's unresponsive to Bacillus Calmette-Guerin (BCG) therapy
Route of administration and frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	15,000–20,000 adult patients

## 2027 Projected launches

Therapy name	<b>aglatimagene besadenovec</b>
Manufacturer	Candel Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The first-line treatment of adults with intermediate- to high-risk, localized, prostate cancer, in combination with external beam radiation therapy and valacyclovir
Route of administration and frequency	Injection-intratumoral, multi-dose
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	70,100 adult patients

## 2027 Projected launches

Therapy name	<b>ristoglogene autogetemcel</b>
Manufacturer	Beam Therapeutics
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of severe sickle cell disease in patients ages 12 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	13,300 adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>Tecartus (brexucabtagene autoleucel)</b>
Manufacturer	Gilead Sciences/Kite
Phase of development	Phase I/II
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of relapsed or refractory B-cell precursor acute lymphoblastic leukemia in patients ages 2–21 years
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	2,800 young adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>RGX202</b>
Manufacturer	RegenxBio
Phase of development	Phase II/III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Duchenne muscular dystrophy in ambulatory patients ages 1–11 years
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q4 2027
Estimated potential U.S. candidates	1,000 male pediatric patients

## 2027 Projected launches

Therapy name	<b>beremagene geperpavec-svdt</b>
Manufacturer	Krystal Biotech
Phase of development	Phase III
Type	New formulation
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment and prevention of corneal abrasions in patients ages 6 months and older with dystrophic epidermolysis bullosa with mutations in the COL7A1 gene
Route of administration and frequency	Ocular, multi-dose
FDA decision date	TBD
Projected launch	Q4 2027
Estimated potential U.S. candidates	230 adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>laruparetigene zosaparvovec</b>
Manufacturer	Beacon Therapeutics
Phase of development	Phase II/III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of X-linked retinitis pigmentosa in males ages 8–50 years with a mutation in the RPGR gene
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q4 2027
Estimated potential U.S. candidates	2,600–5,300 male adult and pediatric patients

## 2027 Projected launches

Therapy name	<b>OCU400</b>
Manufacturer	Ocugen
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of retinitis pigmentosa in patients ages 3 years and older
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q4 2027
Estimated potential U.S. candidates	129,000 adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>rescabtagene autoleucel</b>
Manufacturer	Cabaletta Bio, Inc.
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	CD19 antagonist/CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of active dermatomyositis or anti-synthetase syndrome who have had an inadequate response to, or are intolerant of, one or more prior systemic therapies
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	1H 2028
Estimated potential U.S. candidates	58,900 adult patients

## 2028 Projected launches

Therapy name	<b>OCU410ST</b>
Manufacturer	Ocugen
Phase of development	Phase II/III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Stargardt disease in patients ages 5 years and older
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	32,300–40,400 adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>AAV-AQP1</b>
Manufacturer	MeiraGTx
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of radiation-induced late xerostomia in adults
Route of administration and frequency	Injection-intraparotid, one-time per gland
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	156,000 adult patients

## 2028 Projected launches

Therapy name	<b>BBP812</b>
Manufacturer	BridgeBio
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Canavan disease in pediatrics up to 30 months of age
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	40 pediatric patients annually

## 2028 Projected launches

Therapy name	<b>Invossa (tonogenchancel-L)</b>
Manufacturer	Kolon Group
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Cellular therapy, allogeneic/gene therapy, in vivo
Indication	The treatment of mild-to-moderate (KL grades 2 & 3) knee osteoarthritis
Route of administration and frequency	Injection-intra-articular, one-time per knee
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	10.3 million adult patients

## 2028 Projected launches

Therapy name	<b>ixoberogene soroparvovec</b>
Manufacturer	Adverum Biotechnologies/Eli Lilly
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of neovascular (wet) age-related macular degeneration
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	2 million adult patients

## 2028 Projected launches

Therapy name	<b>soficabtagene geleucel</b>
Manufacturer	Wugen
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	CAR T-cell therapy, allogeneic/gene therapy, in vivo
Indication	The treatment of relapsed or refractory T-cell acute lymphoblastic leukemia or lymphoblastic lymphoma in patients ages 1 year and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	4,800–10,100 adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>descartes08</b>
Manufacturer	Cartesian Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	B-cell maturation antigen (BCMA) inhibitor/CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of generalized myasthenia gravis in adults who are anti-AChR antibody-positive
Route of administration and frequency	Injection-IV, multi-dose
FDA decision date	TBD
Projected launch	Q1 2028
Estimated potential U.S. candidates	71,000 adult patients

## 2028 Projected launches

Therapy name	<b>avalotcogene ontaparvovec</b>
Manufacturer	Ultragenyx Pharmaceutical
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of late-onset ornithine transcarbamylase deficiency in patients ages 12 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2028
Estimated potential U.S. candidates	2,600–4,100 adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>surabgene lomparvovec (RGX314)</b>
Manufacturer	AbbVie/ RegenxBio
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of neovascular (wet) age-related macular degeneration
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q2 2028
Estimated potential U.S. candidates	2 million adult patients

## 2028 Projected launches

Therapy name	<b>nexiguran ziclumeran</b>
Manufacturer	Intellia Therapeutics/Regeneron Pharmaceuticals
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of hereditary transthyretin amyloidosis with polyneuropathy in adults
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2028
Estimated potential U.S. candidates	5,100 adult patients

## 2028 Projected launches

Therapy name	<b>4D150</b>
Manufacturer	4D Molecular Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of neovascular (wet) age-related macular degeneration
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q4 2028
Estimated potential U.S. candidates	2 million adult patients

## 2028 Projected launches

Therapy name	<b>ATSN201</b>
Manufacturer	Atsena Therapeutics
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of RS1-associated X-linked retinoschisis in males ages 6 years and older
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q4 2028
Estimated potential U.S. candidates	6,300–31,400 male adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>dabocemagene autoficel</b>
Manufacturer	Castle Creek Pharma
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of recessive dystrophic epidermolysis bullosa in patients ages 2 years and older
Route of administration and frequency	Injection-intradermal, multi-dose
FDA decision date	TBD
Projected launch	Q4 2028
Estimated potential U.S. candidates	470 adult and pediatric patients

## 2028 Projected launches

Therapy name	<b>mivocabtagene autoleucel</b>
Manufacturer	Kyverna Therapeutics
Phase of development	Phase II/III
Type	New biologic
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of generalized myasthenia gravis in adults who are anti-AChR or MuSK antibody-positive
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q4 2028
Estimated potential U.S. candidates	7,800–15,500 adult patients

## 2029 Projected launches

Therapy name	<b>cemacabtagene ansegedleucel</b>
Manufacturer	Allogene Therapeutics
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	CAR T-cell therapy, allogeneic/gene therapy, ex vivo
Indication	Consolidation therapy in adults with minimal residual disease after response to first-line treatment of large B-cell lymphoma
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2029
Estimated potential U.S. candidates	10,200 adult patients

## 2029 Projected launches

Therapy name	<b>nexiguran ziclumeran</b>
Manufacturer	Intellia Therapeutics/Regeneron Pharmaceuticals
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of transthyretin amyloidosis with cardiomyopathy in adults
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q1 2029
Estimated potential U.S. candidates	50,000–150,000 adult patients

# Abbreviations

**CAR** – Chimeric antigen receptor

**FDA** – U.S. Food and Drug Administration

**IV** – Intravenous

The information contained herein is compiled from independent clinical sources and is provided for informational purposes only. Due to circumstances beyond CVS Health's control, prospective drug launch dates are subject to change without notice. This information should not be solely relied upon for decision-making purposes. This document includes products that may fall under a general specialty or non-specialty drug benefit. All products contained herein may not be provided by CVS Specialty Pharmacy. This document contains references to brand-name prescription drugs that are trademarks or registered trademarks of pharmaceutical manufacturers not affiliated with CVS Health. CVS Health Pipeline Services. ©2026 CVS Health and/or one of its affiliates. All rights reserved. Source: RxPipeline, CVS Health Clinical Affairs. Information current as of May 2026.