Gene Therapy Report

Projected treatments and launch timelines

Q4 2025–Q3 2028

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2025 Projected launches

Therapy name	**Breyanzi (lisocabtagene maraleucel)**
Manufacturer	Bristol-Myers Squibb
Phase of development	Pending FDA approval
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of adults with relapsed or refractory marginal zone lymphoma who have received at least 2 prior lines of systemic therapy
Route of administration & frequency	Injection-IV, one time
FDA decision date	12/5/25
Projected launch quarter/half	4Q
Projected launch year	2025
Estimated potential US candidates	11,700 adult patients

2026 Projected launches

Therapy name	**clemidsogene lanparvovec**
Manufacturer	Nippon Shinyaku/RegenxBio
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 II (Hunter syndrome) in patients ages 5 years and younger
Route of administration & frequency	Injection-intracerebral, one time
FDA decision date	2/8/26
Projected launch quarter/half	1Q
Projected launch year	2026
Estimated potential US candidates	<25 pediatric patients

2026 Projected launches

Therapy name	**etuvetidigene autotemcel**
Manufacturer	Fondazione Telethon
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of Wiskott-Aldrich syndrome
Route of administration & frequency	Injection-IV, one time
FDA decision date	3/11/26
Projected launch quarter/half	1Q
Projected launch year	2026
Estimated potential US candidates	430 adult and pediatric patients

2026 Projected launches

Therapy name	**Kresladi (marnetegragene autotemcel)**
Manufacturer	Rocket Pharmaceuticals
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of severe leukocyte adhesion deficiency type 1 in patients ages 3 months and older
Route of administration & frequency	Injection-IV, one time
FDA decision date	3/28/26
Projected launch quarter/half	1Q
Projected launch year	2026
Estimated potential US candidates	150 pediatric patients

2026 Projected launches

Therapy name	**DB-OTO**
Manufacturer	Regeneron Pharmaceuticals
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of congenital hearing loss due to mutations of the otoferlin gene in patients ages 17 years and younger
Route of administration & frequency	Injection-intracochlear, one time per ear
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2026
Estimated potential US candidates	800 – 6,400 pediatric patients

2026 Projected launches

Therapy name	**dalnacogene ponparvovec**
Manufacturer	Belief Biomed Inc./Takeda
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of hemophilia B in adults
Route of administration & frequency	Injection-IV, one time
FDA decision date	4/10/26
Projected launch quarter/half	2Q
Projected launch year	2026
Estimated potential US candidates	2,200 adult patients

2026 Projected launches

Therapy name	**vusolimogene oderparepvec**
Manufacturer	Replimune Group Inc.
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of unresectable or metastatic cutaneous melanoma after progression on anti-PD1 therapy, in combination with nivolumab (e.g., Opdivo, Opdivo Qvantig)
Route of administration & frequency	Injection-intratumoral, multi-dose
FDA decision date	4/10/26
Projected launch quarter/half	2Q
Projected launch year	2026
Estimated potential US candidates	78,800 adult patients

2026 Projected launches

Therapy name	**anitocabtagene autoleucel**
Manufacturer	Arcellx, Inc./Gilead Sciences/Kite
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, autologous/gene therapy, ex vivo
Indication	The treatment of relapsed or refractory multiple myeloma after at least 3 prior systemic therapies in adults
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2026
Estimated potential US candidates	51,300 adult patients

2026 Projected launches

Therapy name	**cretostimogene grenadenorepvec**
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-Guérin (BCG)-refractory bladder cancer with carcinoma in situ with or without Ta or T1 papillary tumors
Route of administration & frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2026
Estimated potential US candidates	14,600 – 19,500 adult patients

2026 Projected launches

Therapy name	**pariglasgene brecaparvovec**
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 glycogen storage disease type 1a in patients ages 8 years and older
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2026
Estimated potential US candidates	3,100 adult and pediatric patients

2026 Projected launches

Therapy name	**rebisufligene etisparvovec**
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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2026
Estimated potential US candidates	820 – 4,100 adult and pediatric patients

2026 Projected launches

Therapy name	**bidridistrogene xeboparvovec (SRP-9003)**
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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	4Q
Projected launch year	2026
Estimated potential US candidates	<1,000 adult and pediatric patients

2026 Projected launches

Therapy name	**isaralgagene civaparvovec**
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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	4Q
Projected launch year	2026
Estimated potential US candidates	3,300 adult patients

2026 Projected launches

Therapy name	**sonpiretigene isteparvovec**
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 & frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	4Q
Projected launch year	2026
Estimated potential US candidates	67,000 – 76,000 adult patients

2027 Projected launches

Therapy name	**olvimulogene nanivacirepvec**
Manufacturer	Genelux
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of platinum-resistant or refractory ovarian cancer, in combination with platinum-based chemotherapy and bevacizumab
Route of administration & frequency	Injection-intraperitoneal, one time
FDA decision date	TBD
Projected launch quarter/half	1H
Projected launch year	2027
Estimated potential US candidates	39,800 adult patients

2027 Projected launches

Therapy name	**OCU400**
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 5 years and older
Route of administration & frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2027
Estimated potential US candidates	80,000 – 92,000 adult and pediatric patients

2027 Projected launches

Therapy name	**RGX202**
Manufacturer	RegenxBio
Phase of development	Phase I/II
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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2027
Estimated potential US candidates	1,200 pediatric patients

2027 Projected launches

Therapy name	**RPA501**
Manufacturer	Rocket Pharmaceuticals
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Danon disease in males ages 8 years and older
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2027
Estimated potential US candidates	7,500 – 15,000 adult and pediatric patients

2027 Projected launches

Therapy name	**detalimogene voraplasmid**
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 is unresponsive to Bacillus Calmette-Guérin therapy
Route of administration & frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch quarter/half	2Q
Projected launch year	2027
Estimated potential US candidates	14,600 – 19,500 adult patients

2027 Projected launches

Therapy name	**lonvoguran ziclumeran (NTLA-2002)**
Manufacturer	Intellia Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of hereditary angioedema in adults
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	2Q
Projected launch year	2027
Estimated potential US candidates	5,300 adult patients

2027 Projected launches

Therapy name	**avalotcagene ontaparvovec**
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 ornithine transcarbamylase deficiency in patients ages 12 years and older
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	2H
Projected launch year	2027
Estimated potential US candidates	2,600 – 4,100 adult and pediatric patients

2027 Projected launches

Therapy name	**AAVAQP1**
Manufacturer	MeiraGTx
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of radiation-induced late xerostomia in adults
Route of administration & frequency	Injection-intraparotid, one time per gland
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2027
Estimated potential US candidates	153,000 adult patients

2027 Projected launches

Therapy name	**ProstAtak (aglatimagene besadenovec)**
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 & frequency	Injection-intratumoral, multi-dose
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2027
Estimated potential US candidates	75,000 adult patients

2027 Projected launches

Therapy name	**Tecartus (brexucabtagene autoleucel)**
Manufacturer	Gilead Sciences/Kite
Phase of development	Phase I/II
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, allogeneic/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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	3Q
Projected launch year	2027
Estimated potential US candidates	2,800 pediatric and adult patients

2027 Projected launches

Therapy name	**cemacabtagene ansegedleucel**
Manufacturer	Allogene Therapeutics
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	4Q
Projected launch year	2027
Estimated potential US candidates	10,200 adult patients

2027 Projected launches

Therapy name	**laruparetigene zosaparvovec**
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 & frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	4Q
Projected launch year	2027
Estimated potential US candidates	3,300 – 7,600 adult and pediatric patients

2028 Projected launches

Therapy name	**AAVGAD**
Manufacturer	MeiraGTx
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of Parkinson's disease
Route of administration & frequency	Injection-intracerebral, one time
FDA decision date	TBD
Projected launch quarter/half	1H
Projected launch year	2028
Estimated potential US candidates	740,000 adult patients

2028 Projected launches

Therapy name	**BBP812**
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
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2028
Estimated potential US candidates	40 pediatric patients annually

2028 Projected launches

Therapy name	**Invossa (tonogenchoncel-L)**
Manufacturer	Kolon Group
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, in vivo
Indication	The treatment of knee osteoarthritis
Route of administration & frequency	Injection-intra-articular, one time per knee
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2028
Estimated potential US candidates	11.7 million adult patients

2028 Projected launches

Therapy name	**RPL301**
Manufacturer	Rocket Pharmaceuticals
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of pyruvate kinase deficiency in patients ages 8 years and older
Route of administration & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2028
Estimated potential US candidates	590 – 1,600 adult and pediatric patients

2028 Projected launches

Therapy name	**soficabtagene geleucel**
Manufacturer	Wugen
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (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 & frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	1Q
Projected launch year	2028
Estimated potential US candidates	4,800 – 10,100 adult and pediatric patients

2028 Projected launches

Therapy name	**Descartes08**
Manufacturer	Cartesian Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (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 & frequency	Injection-IV, multi-dose
FDA decision date	TBD
Projected launch quarter/half	2Q
Projected launch year	2028
Estimated potential US candidates	71,000 adult patients

2028 Projected launches

Therapy name	**ixoberogene soroparvovec**
Manufacturer	Adverum Biotechnologies
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 & frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	2Q
Projected launch year	2028
Estimated potential US candidates	2 million adult patients

2028 Projected launches

Therapy name	**surabgene lomparvovec (RGX314)**
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 & frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	2Q
Projected launch year	2028
Estimated potential US candidates	2 million adult patients

Abbreviations

FDA - U.S. Food and Drug Administration

IV - Intravenous

Proactive surveillance of the drug pipeline can help inform your pharmacy benefits strategy.

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Source: RxPipeline, CVS Health Clinical Affairs. Information current as of December 2, 2025.

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