



Gene Therapy Report

Projected treatments
and launch timelines

Q1 2026–Q4 2028

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2026 Projected launches

Therapy name	Kresladi (marnetegrane 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 and frequency	Injection-IV, one time
FDA decision date	3/28/26
Projected launch quarter/half	Q1
Projected launch year	2026
Estimated potential U.S. candidates	150 pediatric patients

2026 Projected launches

Therapy name	DB-OTO
Manufacturer	Regeneron Pharmaceuticals
Phase of development	Pending FDA approval
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 and frequency	Injection-intracochlear, one time per ear
FDA decision date	4/1/26
Projected launch quarter/half	Q2
Projected launch year	2026
Estimated potential U.S. 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 without inhibitors
Route of administration and frequency	Injection-IV, one time
FDA decision date	4/10/26
Projected launch quarter/half	Q2
Projected launch year	2026
Estimated potential U.S. 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 and frequency	Injection-intratumoral, multi-dose
FDA decision date	4/10/26
Projected launch quarter/half	Q2
Projected launch year	2026
Estimated potential U.S. candidates	34,500 adult patients

2026 Projected launches

Therapy name	Casgevy (exagamglogene autotemcel)
Manufacturer	CRISPR Therapeutics/Vertex Pharmaceuticals
Phase of development	Phase III
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 to less than 12 years with recurrent vaso-occlusive crises
Route of administration and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	2H
Projected launch year	2026
Estimated potential U.S. candidates	7,700 pediatric patients

2026 Projected launches

Therapy name	Casgevy (exagamglogene autotemcel)
Manufacturer	CRISPR Therapeutics/Vertex Pharmaceuticals
Phase of development	Phase III
Type	Supplemental indication
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo/stem cell therapy, autologoU.S.
Indication	The treatment of transfusion-dependent beta-thalassemia in patients ages 5 to less than 12 years
Route of administration and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	2H
Projected launch year	2026
Estimated potential U.S. candidates	60 pediatric patients

2026 Projected launches

Therapy name	anitocabtagene autoleucel
Manufacturer	Arcellx, Inc./Gilead Sciences/Kite
Phase of development	Pending FDA approval
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 and frequency	Injection-IV, one time
FDA decision date	8/1/26
Projected launch quarter/half	Q3
Projected launch year	2026
Estimated potential U.S. candidates	51,300 adult patients

2026 Projected launches

Therapy name	pariglasgene brecaparvovec
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 quarter/half	Q3
Projected launch year	2026
Estimated potential U.S. 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 and frequency	Injection-IV, one time
FDA decision date	7/30/26
Projected launch quarter/half	Q3
Projected launch year	2026
Estimated potential U.S. candidates	820–4,100 adult and pediatric patients

2026 Projected launches

Therapy name	bidridistrogene xeboparvovec
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 nonambulatory patients ages 4 years and older
Route of administration and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2026
Estimated potential U.S. candidates	<1,000 adult and pediatric 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 and frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2026
Estimated potential U.S. candidates	14,600–19,500 adult patients

2026 Projected launches

Therapy name	sonporetigene 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 and frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2026
Estimated potential U.S. candidates	67,000–76,000 adult patients

2027 Projected launches

Therapy name	arlocabtagene autoleucel
Manufacturer	Bristol-Myers Squibb/Celgene
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 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 quarter/half	1H
Projected launch year	2027
Estimated potential U.S. candidates	51,300 adult patients

2027 Projected launches

Therapy name	AAVAIPL1
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
Route of administration and frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2027
Estimated potential U.S. candidates	100–340 pediatric patients

2027 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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2027
Estimated potential U.S. candidates	3,300 adult patients

2027 Projected launches

Therapy name	mivocabtagene autoleucel
Manufacturer	Kyverna Therapeutics
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 stiff person syndrome
Route of administration and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2027
Estimated potential U.S. candidates	3,600–5,600 adult patients

2027 Projected launches

Therapy name	revlucabtagene autoleucel
Manufacturer	Immix Biopharma
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Chimeric antigen receptor (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 quarter/half	Q1
Projected launch year	2027
Estimated potential U.S. candidates	11,500 adult patients

2027 Projected launches

Therapy name	beremagene geperpavec-svdt
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 quarter/half	Q2
Projected launch year	2027
Estimated potential U.S. candidates	200 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 and frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch quarter/half	Q2
Projected launch year	2027
Estimated potential U.S. candidates	14,600–19,500 adult patients

2027 Projected launches

Therapy name	lonvoguran ziclumeran
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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q2
Projected launch year	2027
Estimated potential U.S. candidates	5,300 adult patients

2027 Projected launches

Therapy name	RGX202
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 quarter/half	Q2
Projected launch year	2027
Estimated potential U.S. candidates	1,200 pediatric 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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	2H
Projected launch year	2027
Estimated potential U.S. candidates	2,600–4,100 adult and pediatric 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 and frequency	Injection-intraperitoneal, one time
FDA decision date	TBD
Projected launch quarter/half	2H
Projected launch year	2027
Estimated potential U.S. candidates	39,800 adult patients

2027 Projected launches

Therapy name	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 and frequency	Injection-intratumoral, multi-dose
FDA decision date	TBD
Projected launch quarter/half	Q3
Projected launch year	2027
Estimated potential U.S. 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, 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 quarter/half	Q3
Projected launch year	2027
Estimated potential U.S. candidates	2,800 pediatric and adult 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 and frequency	Injection-intraparotid, one time per gland
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2027
Estimated potential U.S. candidates	153,000 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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2027
Estimated potential U.S. 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 and frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2027
Estimated potential U.S. candidates	3,300–7,600 adult and pediatric patients

2028 Projected launches

Therapy name	OCU410ST
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 quarter/half	1H
Projected launch year	2028
Estimated potential U.S. candidates	32,300–40,400 adult and pediatric 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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2028
Estimated potential U.S. 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	Cellular therapy, allogeneic to drug class/ gene therapy, in vivo
Indication	The treatment of knee osteoarthritis
Route of administration and frequency	Injection-intra-articular, one time per knee
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2028
Estimated potential U.S. candidates	11.7 million 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 and frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2028
Estimated potential U.S. candidates	2 million adult patients

2028 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 3 years and older
Route of administration and frequency	Injection-intraocular, one time per eye
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2028
Estimated potential U.S. candidates	82,300–94,000 adult and pediatric patients

2028 Projected launches

Therapy name	soficabtagene geleucel
Manufacturer	Wugen
Phase of development	Phase II
Type	New biologic
Breakthrough therapy designation	Yes
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 and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q1
Projected launch year	2028
Estimated potential U.S. 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	B-cell maturation antigen (BCMA) inhibitor/ 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 and frequency	Injection-IV, multi-dose
FDA decision date	TBD
Projected launch quarter/half	Q2
Projected launch year	2028
Estimated potential U.S. candidates	71,000 adult patients

2028 Projected launches

Therapy name	surabgene lomparvovec
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 quarter/half	Q2
Projected launch year	2028
Estimated potential U.S. candidates	2 million adult patients

2028 Projected launches

Therapy name	4D150
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 quarter/half	Q4
Projected launch year	2028
Estimated potential U.S. candidates	2 million adult patients

2028 Projected launches

Therapy name	ATSN201
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 quarter/half	Q4
Projected launch year	2028
Estimated potential U.S. candidates	6,300–31,400 adult and pediatric patients

2028 Projected launches

Therapy name	dabocemagene autoficel
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 quarter/half	Q4
Projected launch year	2028
Estimated potential U.S. candidates	460 adult and pediatric patients

2028 Projected launches

Therapy name	mivocabtagene autoleucel
Manufacturer	Kyverna Therapeutics
Phase of development	Phase II/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 or MU.S.K antibody-positive
Route of administration and frequency	Injection-IV, one time
FDA decision date	TBD
Projected launch quarter/half	Q4
Projected launch year	2028
Estimated potential U.S. candidates	7,800–15,500 adult patients

Abbreviations

FDA - U.S. Food and Drug Administration

IV - Intravenous

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