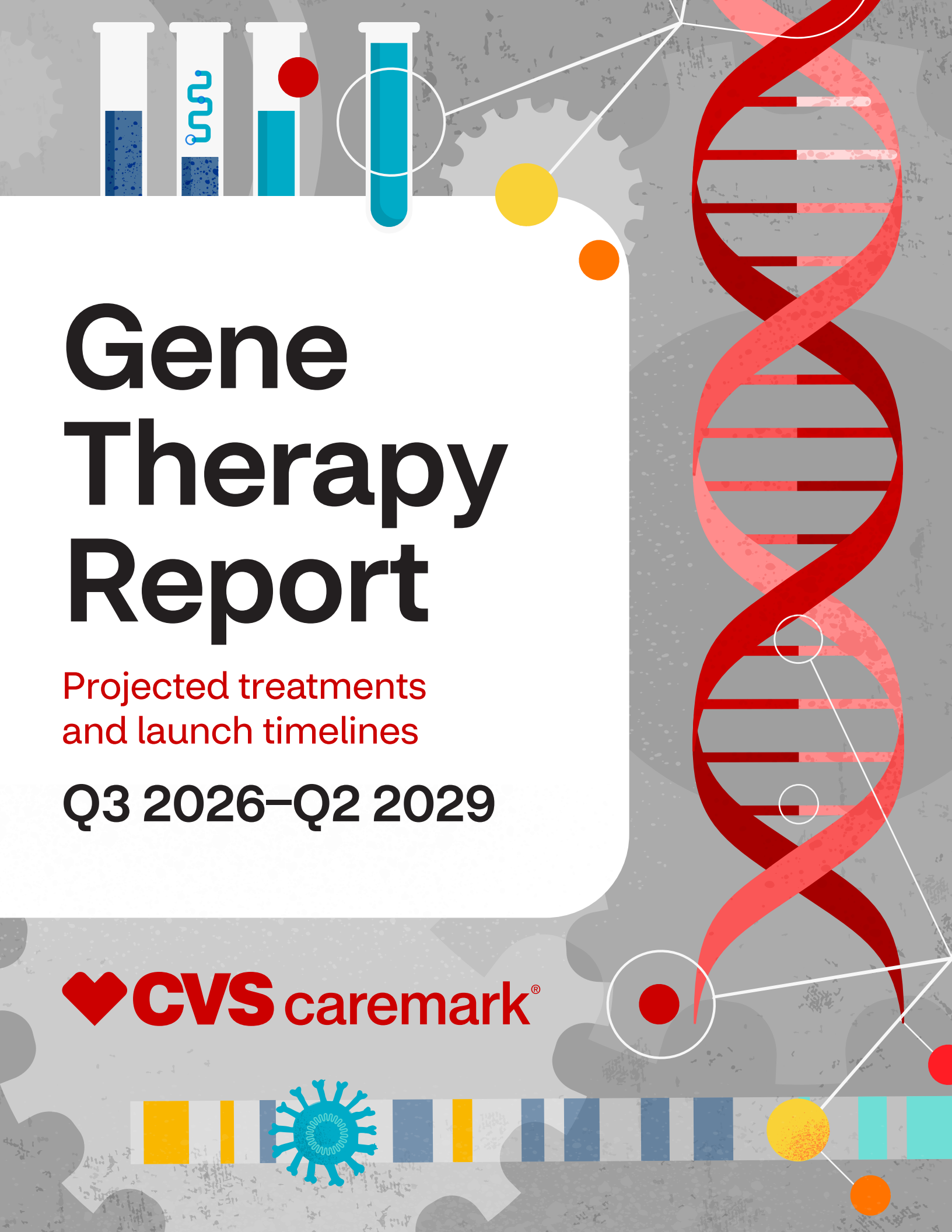


The background of the slide is a light gray with faint, large-scale gear patterns. At the top left, there are four test tubes: the first contains blue liquid, the second has a blue squiggly line representing a protein or virus, the third has blue liquid and a red dot, and the fourth is partially filled with blue liquid and is circled in white. To the right of the test tubes are two yellow circles and one orange circle. On the right side of the slide, a large red DNA double helix runs vertically. It has horizontal rungs, some of which are white and some are red. There are three white circles on the helix, each with a line pointing to a specific rung. At the bottom left, there is a horizontal bar with alternating yellow and blue segments, and a blue virus-like particle with spikes. At the bottom right, there is a red circle, a yellow circle, and an orange circle, each with a line pointing to a specific part of the DNA helix.

Gene Therapy Report

Projected treatments
and launch timelines

Q3 2026–Q2 2029

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2026 Projected launches

Therapy name	Genglycos (pariglasgene brecaparvovec-opnr)
Manufacturer	Ultragenyx Pharmaceutical
Phase of development	Launched
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	8/19/26
Estimated potential U.S. candidates	2,500 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) in patients ages 17 years and younger
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 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
Indication	The treatment of relapsed or refractory multiple myeloma in adults after at least 3 prior systemic therapies
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	isargalgene 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 adult males
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 male patients

2027 Projected launches

Therapy name	lonvoguran ziclumeran
Manufacturer	Intellia Therapeutics
Phase of development	Pending FDA approval
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	3/10/27
Projected launch	Q1 2027
Estimated potential U.S. candidates	5,500 adult and adolescent patients

2027 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 non-ambulatory patients ages 4 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	750–1,000 adult and pediatric patients

2027 Projected launches

Therapy name	botaretigene sparoparvovec
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-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	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–Bacillus Calmette-Guerin therapy in adults
Route of administration and frequency	Injection-intravesical, multi-dose
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	15,000–20,000 adult patients

2027 Projected launches

Therapy name	ifezuntirgene inilparvovec
Manufacturer	Uniqure
Phase of development	Pending FDA approval
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of early Huntington's disease in patients ages 25–65 years
Route of administration and frequency	Injection-intracerebral, one-time
FDA decision date	5/2/27
Projected launch	Q2 2027
Estimated potential U.S. candidates	7,000 adult patients

2027 Projected launches

Therapy name	Mogenry (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 and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q2 2027
Estimated potential U.S. candidates	104,000 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 adults, 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 female 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 intermediate-to high-risk, localized, prostate cancer in adults, 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 male 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
Indication	The treatment of relapsed or refractory multiple myeloma in adults after at least 3 prior systemic therapies
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	54,100 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	Q3 2027
Estimated potential U.S. candidates	230 adult and pediatric patients

2027 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-Guerin-refractory bladder cancer with carcinoma in-situ with or without Ta or T1 papillary tumors in adults
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	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	Q3 2027
Estimated potential U.S. candidates	2,600–5,300 male adult and pediatric patients

2027 Projected launches

Therapy name	meduretgene parvec (FKA 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 in patients ages 1–4 years
Route of administration and frequency	Injection-intraocular, one-time per eye
FDA decision date	TBD
Projected launch	Q3 2027
Estimated potential U.S. candidates	140–340 pediatric 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
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	ristoglogene autogetemcel
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	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
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	5,800 young adult and pediatric 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 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

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	Q3 2027
Estimated potential U.S. candidates	1,000 male pediatric patients

2028 Projected launches

Therapy name	rescabtagene autoleucel
Manufacturer	Cabaletta Bio, Inc.
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	CD19 antagonist/Chimeric antigen receptor (CAR) T-cell therapy, autologous
Indication	The treatment of active dermatomyositis in adults, anti-synthetase syndrome in adults, or juvenile dermatomyositis in patients ages 6 years and older, 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	60,100 adult and pediatric patients

2028 Projected launches

Therapy name	AAV-AQP1
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	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 in pediatric patients up to age 30 months
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	Invossa (tonogenchancel-L)
Manufacturer	Kolon Group
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Cellular therapy, allogeneic/Gene therapy, ex vivo
Indication	The treatment of mild-to-moderate (KL grades 2 and 3) knee osteoarthritis
Route of administration and frequency	Injection-intra-articular, one-time per knee
FDA decision date	TBD
Projected launch	Q2 2028
Estimated potential U.S. candidates	10.3 million adult patients

2028 Projected launches

Therapy name	ixoberogene soroparvovec
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	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	Q1 2028
Estimated potential U.S. candidates	32,300–40,400 adult and pediatric patients

2028 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
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	Q1 2028
Estimated potential U.S. candidates	11,500 adult 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
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	avalotcogene 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 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	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
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	Q2 2028
Estimated potential U.S. candidates	7,100–14,200 adult patients

2028 Projected launches

Therapy name	lonimecogene renparvovec
Manufacturer	Taysha Gene Therapies
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of Rett syndrome in females ages 2–21 years
Route of administration and frequency	Injection-intrathecal, one-time
FDA decision date	TBD
Projected launch	Q2 2028
Estimated potential U.S. candidates	2,000–4,000 female pediatric and young 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 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	LX2006
Manufacturer	Lexeo Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	Yes
Drug class	Gene therapy, in vivo
Indication	The treatment of Friedreich ataxia cardiomyopathy in patients ages 6 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2028
Estimated potential U.S. candidates	4,100–9,700 pediatric and adult patients

2028 Projected launches

Therapy name	nexiguran ziclumeran
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	rondecabtagene autoleucel
Manufacturer	Lyell Immunopharma
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, autologous
Indication	The treatment of relapsed or refractory large B-cell lymphoma in adults who have received 2 or more prior lines of therapy
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q3 2028
Estimated potential U.S. candidates	3,500 adult patients

2028 Projected launches

Therapy name	zunibergene rocparvovec (FKA 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 wet age-related macular degeneration in adults
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	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	Q4 2028
Estimated potential U.S. candidates	6,300–31,400 male 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	Q4 2028
Estimated potential U.S. candidates	470 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
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	lasmecabtagene timgedleucel
Manufacturer	Collectis
Phase of development	Phase I/II
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, allogeneic
Indication	The treatment of relapsed or refractory B-cell, CD22-positive acute lymphoblastic leukemia in patients ages 15 years and older
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	1H 2029
Estimated potential U.S. candidates	15,900 adolescent and adult patients

2029 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
Indication	Consolidation therapy in adults with minimal residual disease after response–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	9,000 adult patients

2029 Projected launches

Therapy name	nexiguran ziclumeran
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

2029 Projected launches

Therapy name	arlocabtagene autoleucel
Manufacturer	Bristol-Myers Squibb/Celgene
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Chimeric antigen receptor (CAR) T-cell therapy, autologous
Indication	The treatment of relapsed or refractory multiple myeloma in adults who have received 1–3 prior treatments
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2029
Estimated potential U.S. candidates	98,600 adult patients

2029 Projected launches

Therapy name	OTL203
Manufacturer	Fondazione Telethon/Orchard Therapeutics
Phase of development	Phase III
Type	New biologic
Breakthrough therapy designation	No
Drug class	Gene therapy, ex vivo
Indication	The treatment of mucopolysaccharidosis (MPS) type I, Hurler variance, in pediatric patients up to age 30 months
Route of administration and frequency	Injection-IV, one-time
FDA decision date	TBD
Projected launch	Q2 2029
Estimated potential U.S. candidates	20 pediatric patients annually

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