American Society of Gene + Cell Therapy

CITELINE
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Gene, Cell, & RNA Therapy Landscape Report

Q4 2025 Quarterly Data Report

January 2026

Contents

Introduction

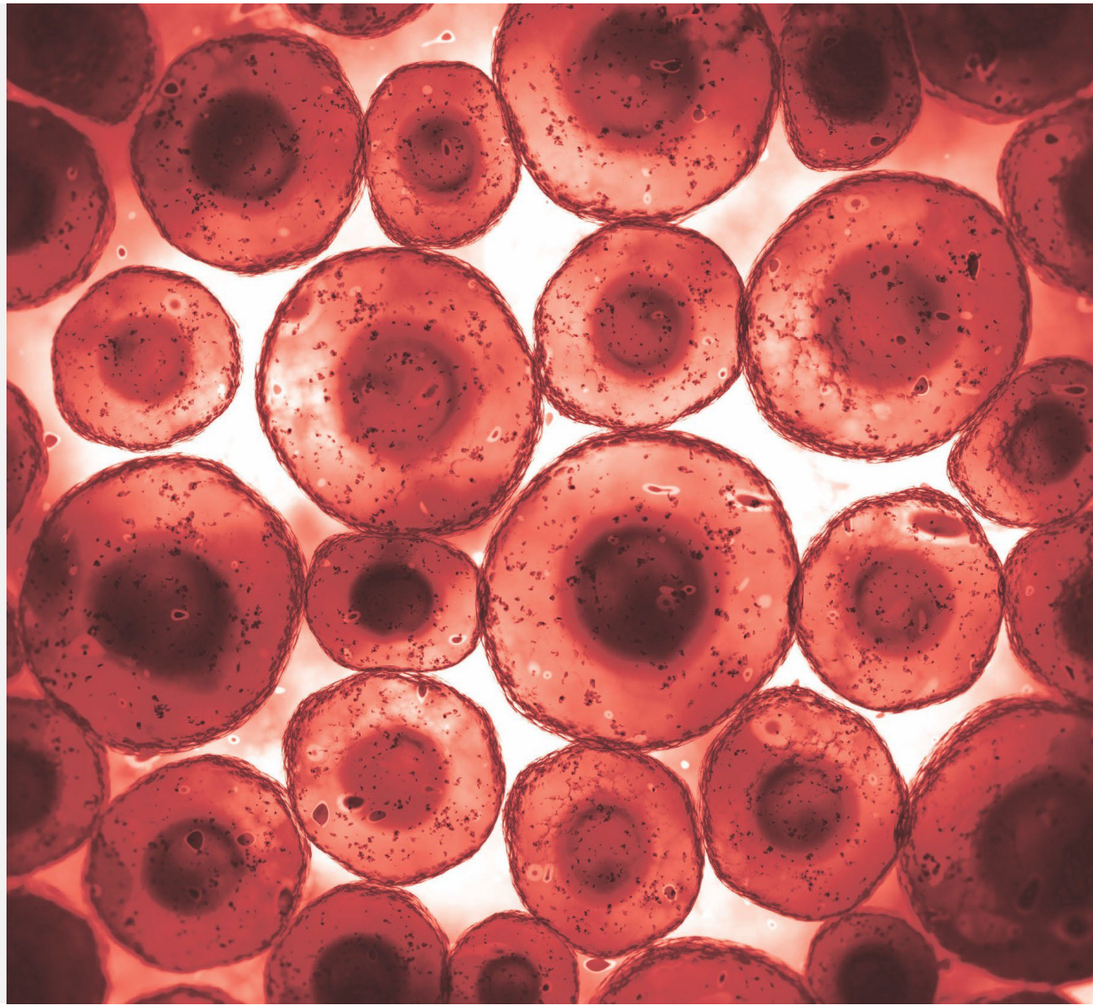
The fourth quarter of 2025 marked continued regulatory progress across the gene, cell, and RNA therapy landscape, with three new approvals spanning gene and RNA modalities. In gene therapy, regulatory agencies in both China and the United States advanced novel treatments for rare and serious conditions. The FDA approved GSK's Waskyra, a hematopoietic stem cell–based gene therapy for Wiskott-Aldrich syndrome, while China's NMPA approved Chongqing Precision Biotech's Pulidekai, a CD19 CAR-T therapy for relapsed or refractory acute lymphoblastic leukemia. In RNA therapeutics, the FDA also granted marketing approval to Arrowhead Pharmaceuticals' Redemplo for familial chylomicronemia syndrome.

Despite these regulatory milestones, the overall development pipeline continued to contract modestly in Q4. The total number of gene, cell, and RNA therapies in development declined to 2,041, representing a 4% decrease from Q1 2025 levels. This shift was largely driven by a reduction in preclinical programs, which fell from 66% to 60% of the pipeline, reflecting a broader recalibration of early-stage portfolios rather than downstream attrition. Notably, the proportion of therapies in Phase I through pre-registration remained relatively stable, underscoring continued commitment to advancing programs already in clinical development.

In contrast to the tightening pipeline, start-up financing activity strengthened further in Q4. Advanced molecular therapy companies raised $557.1 million across 14 transactions, representing a 141% increase in total value from the prior quarter. Dealmaking activity overall remained steady at 100 transactions, signaling sustained strategic interest amid a more selective development environment.

Taken together, Q4 2025 illustrates a field in transition — balancing near-term regulatory achievements, disciplined pipeline optimization, and renewed investor confidence as innovation continues to evolve across CGT modalities.

David Barrett, JD
CEO, ASGCT



Key takeaways from Q4 2025

Three new approvals across each of the gene, cell, and RNA categories took place in Q4 2025

- Two gene therapy approvals: in China, the NMPA approved Chongqing Precision Biotech's CAR-T therapy, Pulidekai, for relapsed or refractory acute lymphoblastic leukemia; while in the US, the FDA approved GSK's Waskyra, a gene therapy indicated for Wiskott-Aldrich syndrome
- One RNA therapy approval: the US FDA granted marketing approval for Arrowhead Pharmaceuticals' Redemplo in familial chylomicronemia syndrome (also known as lipoprotein lipase deficiency)
- There were no non-genetically modified cell therapy approvals during Q4 2025

Throughout 2025, the total number of gene, cell, and RNA therapies in development declined, driven by a shift away from preclinical programs

- The Q4 2025 gene, cell, and RNA pipeline included 2,041 therapies, down from 2,117 in Q1 2025, representing a 4% drop in the total number of therapies
- While the proportion of therapies in Phase I to pre-registration remained within 3% during this period, the proportion of preclinical therapies fell more noticeably, dropping from 66% to 60%

Start-up advanced molecular financing continues rebound

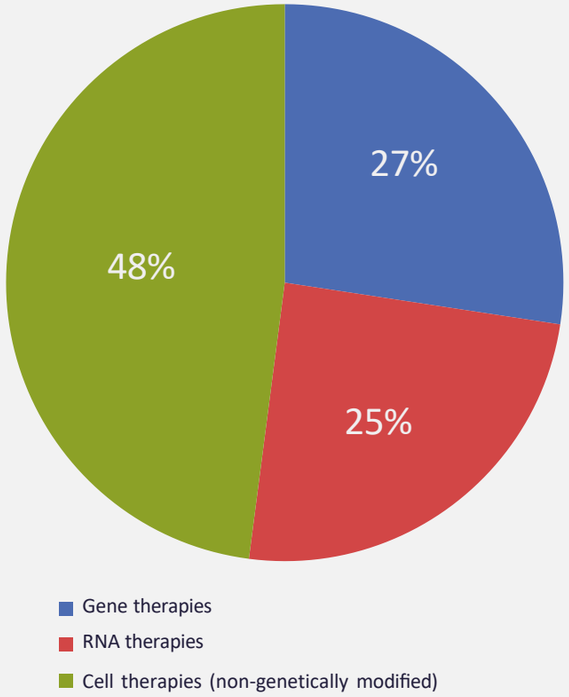
- Advanced molecular therapy start-up financing continued its quarterly upward trend, reaching 14 transactions in Q4 2025 together worth $557.1 million, a 27% increase in volume and 141% increase in value compared with the previous quarter
- The top start-up financings featured novel delivery systems, led by Soufflé Therapeutics, which raised $200 million to support work on cell-specific ligands to deliver siRNA-based medicines
- Overall, total dealmaking was virtually flat in Q4 2025 at 100 deals, just slightly ahead of Q3 2025's 99 in volume, but 12% behind 2024's closing quarter featuring 113 deals

Key highlights in Q4 2025

Approved gene, cell, and RNA therapies

Globally, for clinical use:

- 41 gene therapies have been approved (including genetically modified cell therapies)
  - GSK's Waskyra, a hematopoietic stem cell-based gene therapy, was approved in the US for Wiskott-Aldrich syndrome
  - Chongqing Precision Biotech's Pulidekai, a CD19 CAR-T cell therapy, was approved in China for relapsed or refractory acute lymphoblastic leukemia
- 37 RNA therapies have been approved
  - Arrowhead Pharmaceuticals' Redemplo was approved in the US for familial chylomicronemia syndrome
- 70 non-genetically modified cell therapies have been approved
  - No new approvals in Q4 2025



Source: Pharmaprojects | Citeline, December 2025

Approved gene therapies as of Q4 2025

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Gendicine	recombinant p53 gene	2004	Head and neck cancer	China	Shenzhen SiBiono GeneTech
Oncorine	E1B/E3 deficient adenovirus	2005	Head and neck cancer; nasopharyngeal cancer	China	Shanghai Sunway Biotech
Rexin-G	mutant cyclin-G1 gene	2006	Solid tumors	Philippines	Epeius Biotechnologies
Neovasculgen	vascular endothelial growth factor gene	2011	Peripheral vascular disease; limb ischemia	Russian Federation, Ukraine	Human Stem Cells Institute
Imlygic	talimogene laherparepvec	2015	Melanoma	US, EU, UK, Australia, China	Amgen
Strimvelis	autologous CD34+ enriched cells	2016	Adenosine deaminase deficiency	EU, UK	Orchard Therapeutics
Kymriah	tisagenlecleucel-t	2017	Acute lymphocytic leukemia; diffuse large B-cell lymphoma; follicular lymphoma	US, EU, UK, Japan, Australia, Canada, South Korea, Switzerland, Brazil	Novartis
Luxturna	voretigene neparvovec	2017	Leber's congenital amaurosis; retinitis pigmentosa	US, EU, UK, Australia, Brazil, Canada, South Korea, Japan, Russian Federation	Spark Therapeutics (Roche)
Yescarta	axicabtagene ciloleucel	2017	Diffuse large B-cell lymphoma; non-Hodgkin's lymphoma; follicular lymphoma	US, EU, UK, Japan, Canada, China, Australia, Brazil, Israel, Singapore, South Korea	Kite Pharma (Gilead)
Zolgensma	onasemnogene abeparvovec	2019	Spinal muscular atrophy	US, EU, UK, Japan, Australia, Canada, Brazil, Israel, Taiwan, South Korea, China	Novartis
Zynteglo	betibeglogene autotemcel	2019	Transfusion-dependent beta thalassemia	US	Genetix Pharmaceuticals (formerly bluebird bio)
Tecartus	brexucabtagene autoleucel	2020	Mantle cell lymphoma; acute lymphocytic leukemia	US, EU, UK, Australia, Canada, Brazil	Kite Pharma (Gilead)
Libmeldy	atidarsagene autotemcel	2020	Metachromatic leukodystrophy	EU, UK, Switzerland, US	Orchard Therapeutics

Text highlighted in yellow represents new approvals during Q4 2025.

Approved gene therapies as of Q4 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Breyanzi	lisocabtagene maraleucel	2021	Diffuse large B-cell lymphoma; follicular lymphoma; chronic lymphocytic leukemia; mantle cell lymphoma; marginal zone lymphoma	US, Japan, EU, Switzerland, UK, Canada	Celgene (Bristol Myers Squibb)
Abecma	idecabtagene vicleucel	2021	Multiple myeloma	US, Canada, EU, UK, Japan, Israel, Switzerland	Genetix Biotherapeutics (formerly bluebird bio)
Delytact	teserpaturev	2021	Malignant glioma	Japan	Daiichi Sankyo
Relma-cel	relmacabtagene autoleucel	2021	Diffuse large B-cell lymphoma; follicular lymphoma; mantle cell lymphoma	China, Macao	JW Therapeutics
Skysona	elivaldogene autotemcel	2021	Early cerebral adrenoleukodystrophy (CALD)	US	Genetix Biotherapeutics (formerly bluebird bio)
Carvykti	ciltacabtagene autoleucel	2022	Multiple myeloma	US, EU, UK, Japan, Brazil, Australia, Canada, China	Legend Biotech
Upstaza	eladocagene exuparvovec	2022	Aromatic L-amino acid decarboxylase (AADC) deficiency	EU, UK, Israel, US, Brazil	PTC Therapeutics
Roctavian	valoctocogene roxaparvovec	2022	Hemophilia A	EU, US, Brazil	BioMarin
Hemgenix	etranacogene dezaparvovec	2022	Hemophilia B	US, EU, UK, Canada, Switzerland, Australia, Hong Kong, Saudi Arabia, South Korea, Taiwan	uniQure
Adstiladrin	nadofaragene firadenovec	2022	Bladder cancer	US	Merck & Co.
Elevidys	delandistrogene moxeparvovec	2023	Duchenne muscular dystrophy	US, Brazil, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Japan	Sarepta Therapeutics
Vyjuvek	beremagene geperpavec	2023	Dystrophic epidermolysis bullosa	US, EU, Japan	Krystal Biotech
Fucaso	equecabtagene autoleucel	2023	Multiple myeloma	China, Hong Kong, Macao	Nanjing IASO Biotechnology

Text highlighted in yellow represents new approvals during Q4 2025.

Approved gene therapies as of Q4 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Casgevy	exagamglogene autotemcel	2023	Sickle cell anemia; thalassemia	US, UK, United Arab Emirates, Bahrain, Saudi Arabia, EU, Canada, Switzerland, Qatar	CRISPR Therapeutics
Yorwidatm	inaticabtagene autoleucel	2023	Acute lymphocytic leukemia; Large B-cell lymphoma	China	Juventas Cell Therapy
Lyfgenia	lovotibeglogene autotemcel	2023	Sickle cell anemia	US	Genetix Biotherapeutics (formerly bluebird bio)
zevorcabtagene autoleucel	zevorcabtagene autoleucel	2024	Relapsed or refractory multiple myeloma	China	CARsgen Therapeutics
Tecelra	afamitresgene autoleucel	2024	Synovial sarcoma	US	Adaptimmune
Aucatzyl	obecabtagene autoleucel	2024	Acute lymphocytic leukemia	US, UK, EU	Autolus
Qartemi	varnimcabtagene autoleucel	2025	B-cell Non-Hodgkin's Lymphoma (B-NHL)	India, Spain	Immuneel Therapeutics
Encelto	revakinagene taroretcel	2025	Macular telangiectasia type 2 (MacTel)	US	Neurotech
BBM-H901	dalnacogene ponparvovec	2025	Hemophilia B	China	Belief BioMed
Zevaskyn	prademagene zamikeracel	2025	Recessive dystrophic epidermolysis bullosa (RDEB)	US	Abeona Therapeutics
Hicara	renikeolunsai	2025	Relapsed or refractory large B-cell lymphoma	China	Hrain Biotechnology
Papzimeos	zopapogene imadenovec	2025	Recurrent respiratory papillomatosis (RRP)	USA	Precigen
Pulidekai	pulkilumab	2025	Acute lymphocytic leukemia	China	Chongqing Precision Biotech
Waskyra	etuvetidigene autotemcel	2025	Wiskott-Aldrich syndrome	USA	GSK

Text highlighted in yellow represents new approvals during Q4 2025.

Source: Pharmaprojects | Citeline, December 2025

Approved RNA therapies as of Q4 2025

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Macugen	pegaptanib octasodium	2004	Wet age-related macular degeneration	US, EU, Canada, Argentina, Brazil, Hong Kong, Japan, Mexico, Pakistan, Peru, Philippines, Singapore, Switzerland, Thailand, Turkey, UK,	Gilead Sciences
Kynamro	mipomersen sodium	2013	Homozygous familial hypercholesterolemia	US, Mexico, Argentina, South Korea	Ionis Pharmaceuticals
Exondys 51	eteplirsen	2016	Dystrophy, Duchenne muscular	US	Sarepta Therapeutics
Spinraza	nusinersen	2016	Muscular atrophy, spinal	US, EU, UK, Canada, Japan, Brazil, Switzerland, Australia, South Korea, China, Argentina, Colombia, Taiwan, Turkey, Hong Kong, Israel	Ionis Pharmaceuticals
Ampligen	rintatolimod	2016	Chronic fatigue syndrome	Argentina	AIM ImmunoTech
Tegsedi	inotersen	2018	Amyloidosis, transthyretin-related hereditary	EU, UK, Canada, Brazil	Ionis Pharmaceuticals
Onpattro	patisiran	2018	Amyloidosis, transthyretin-related hereditary	US, EU, UK, Japan, Canada, Switzerland, Brazil, Taiwan, Israel, Turkey, Australia	Alnylam
Vyondys 53	golodirsen	2019	Dystrophy, Duchenne muscular	US	Sarepta Therapeutics
Waylivra	volanesorsen	2019	Hypertriglyceridemia; lipoprotein lipase deficiency	EU, UK, Brazil, Canada	Ionis Pharmaceuticals
Comirnaty	tozinameran	2020	Infection, coronavirus, novel coronavirus prophylaxis	UK, Bahrain, Israel, Canada, US, Rwanda, Serbia, United Arab Emirates, Macao, Taiwan, Mexico, Kuwait, Singapore, Saudi Arabia, Chile, Switzerland, EU, Ghana, Colombia, Philippines, Indonesia, Australia, Hong Kong, Peru, South Korea, New Zealand, Japan, Brazil, Sri Lanka, Vietnam, South Africa, Thailand, Oman, Egypt, Malaysia, China	BioNTech
Spikevax	COVID-19 vaccine, Moderna	2020	Infection, coronavirus, novel coronavirus prophylaxis	US, Canada, Israel, EU, Switzerland, Singapore, Qatar, Vietnam, UK, Philippines, Thailand, Japan, South Korea, Brunei, Paraguay, Taiwan, Botswana, India, Indonesia, Saudi Arabia, Mexico, Australia, Nigeria, Colombia	Moderna Therapeutics
Givlaari	givosiran	2020	Porphyria	US, EU, UK, Canada, Switzerland, Brazil, Israel, Japan, Australia	Alnylam

Text highlighted in yellow represents new approvals during Q4 2025.

Approved RNA therapies as of Q4 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Oxlumo	lumasiran	2020	Hyperoxaluria	EU, UK, US, Brazil, Canada, Australia	Alnylam
Viltepso	viltolarsen	2020	Dystrophy, Duchenne muscular	US, Japan	NS Pharma
Leqvio	inclisiran	2020	Atherosclerosis; heterozygous familial hypercholesterolemia; Mixed dyslipidemia	EU, UK, Australia, Canada, Israel, US, Saudi Arabia, Japan, China, South Korea	Alnylam
Amondys 45	casimersen	2021	Dystrophy, Duchenne muscular	US	Sarepta Therapeutics
Gennova COVID-19 vaccine	COVID-19 vaccine, Gennova Biopharmaceuticals	2022	Infection, coronavirus, novel coronavirus prophylaxis	India	Gennova Biopharmaceuticals (Emcure Pharmaceuticals)
Amvuttra	vutrisiran	2022	Amyloidosis, transthyretin-related hereditary; Amyloidosis, transthyretin-related wild-type	US, EU, UK, Brazil, Japan	Alnylam
Moderna Spikevax Bivalent Original/Omicron vaccine	COVID-19 bivalent original/Omicron vaccine, Moderna	2022	Infection, coronavirus, novel coronavirus prophylaxis	UK, Canada, Taiwan, Switzerland, Japan, EU, Australia, South Korea, Singapore, US	Moderna Therapeutics
ARCoV	COVID-19 vaccine, Suzhou Abogen Biosciences	2022	Infection, coronavirus, novel coronavirus prophylaxis	Indonesia	Suzhou Abogen Biosciences
CSPC Pharmaceutical COVID-19 vaccine	COVID-19 vaccine, CSPC Pharmaceutical	2023	Infection, coronavirus, novel coronavirus prophylaxis	China	CSPC Pharmaceutical
Izervay	avacincaptad pegol sodium	2023	Dry age-related macular degeneration	US, Japan	Archemix
Qalsody	tofersen	2023	Amyotrophic lateral sclerosis	US, EU, Japan, China, Canada, South Korea	Ionis Pharmaceuticals
ARCT-154	COVID-19 mRNA vaccine, Arcturus	2023	Infection, coronavirus, novel coronavirus prophylaxis	Japan, EU	Arcturus Therapeutics

Text highlighted in yellow represents new approvals during Q4 2025.

Approved RNA therapies as of Q4 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Daichirona	COVID-19 vaccine, Daiichi Sankyo	2023	Infection, coronavirus, novel coronavirus prophylaxis	Japan	Daiichi Sankyo
Wainua	eplontersen	2023	Transthyretin-related hereditary amyloidosis	US, Canada, EU, UK	Ionis Pharmaceuticals
Rivfloza	nedosiran	2023	Hyperoxaluria	US	Dicerna Pharmaceuticals (Novo Nordisk)
SYS-6006.32	Bivalent COVID-19 mRNA vaccine, CSPC Pharmaceutical	2023	Infection, coronavirus, novel coronavirus prophylaxis	China	CSPC Pharmaceutical
RQ-3033	COVID-19 mRNA vaccine, Walvax Biotechnology	2023	Infection, coronavirus, novel coronavirus prophylaxis	China	Walvax Biotechnology
Rytelo	imetelstat	2024	Myelodysplastic syndrome	US, EU	Geron
mRESVIA	respiratory syncytial virus vaccine, Moderna Therapeutics	2024	Respiratory syncytial virus prophylaxis	US, EU, Canada, Qatar, Taiwan, UAE, UK, Australia, Switzerland, Japan	Moderna Therapeutics
Tryngolza	olezarsen	2024	Lipoprotein lipase deficiency	US, EU	Ionis Pharmaceuticals
Qfitlia	fitusiran	2025	Hemophilia A & B	US	Alnylam
mNexspike	COVID-19 next generation vaccine, Moderna Therapeutics	2025	Infection, coronavirus, novel coronavirus prophylaxis	US	Moderna Therapeutics
Dawnzera	donidalorsen	2025	Angioedema, hereditary	US	Ionis Pharmaceuticals
Redemplo	plozasiran	2025	Lipoprotein lipase deficiency	US	Arrowhead Pharmaceuticals

Text highlighted in yellow represents new approvals during Q4 2025.

Source: Pharmaprojects | Citeline, December 2025

Key highlights in Q4 2025 (Noteworthy events that happened in Q4 2025)

Drug	Event Type	Indication	Molecule	Event Date
Wainua	Regulatory - Approval (China)	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	Antisense	12/25/2025
Bemdaneprocel	Regulatory - Sakigake Designation (Japan)	Parkinson's Disease (PD)	Cellular	12/17/2025
Telomelysin	Regulatory - J-NDA Filing (Japan)	Esophageal Cancer	Viral Gene Therapy	12/15/2025
Telomelysin	Regulatory - Orphan Drug Designation (Japan)	Esophageal Cancer	Viral Gene Therapy	12/12/2025
CK0804	Regulatory - Orphan Drug Designation (U.S.)	Myelofibrosis (MF)	Cellular	12/10/2025
Rese-cel	Regulatory - Orphan Drug Designation (U.S.)	Myasthenia Gravis (MG)	Cellular	12/10/2025
AB-1002	Regulatory - Sakigake Designation (Japan)	Chronic Heart Failure - Reduced Ejection Fraction (Chronic HFrEF)	Viral Gene Therapy	12/09/2025
AB-1005	Regulatory - Sakigake Designation (Japan)	Parkinson's Disease (PD)	Viral Gene Therapy	12/09/2025
Waskyra	Regulatory - Approval (U.S.)	Wiskott-Aldrich Syndrome	Cellular	12/09/2025
CHM 2101	Regulatory - Orphan Drug Designation (U.S.)	Gastric Cancer	Cellular	12/04/2025
SGT-212	Regulatory - Orphan Drug Designation (U.S.)	Friedreich's Ataxia	Viral Gene Therapy	12/04/2025
ION-373	Regulatory - Breakthrough Therapy Designation (U.S.)	Acid Sphingomyelinase Deficiency (ASMD)	Antisense	12/02/2025
LTS-101	Regulatory - Fast Track Status	Neuronal Ceroid Lipofuscinosis (NCL)	Viral Gene Therapy	12/02/2025
LTS-101	Regulatory - Rare Pediatric Disease (RPD) Designation	Neuronal Ceroid Lipofuscinosis (NCL)	Viral Gene Therapy	12/02/2025
NXC-201	Regulatory - Orphan Drug Designation (U.S.)	Amyloid light-chain (AL) Amyloidosis	Cellular	12/02/2025
Redemplo	Regulatory - Breakthrough Therapy Designation (U.S.)	Metabolic - General	siRNA/RNAi	12/02/2025
SGT-212	Regulatory - Rare Pediatric Disease (RPD) Designation	Friedreich's Ataxia	Viral Gene Therapy	12/01/2025

Key highlights in Q4 2025 (Noteworthy events that happened in Q4 2025) *continued*

Drug	Event Type	Indication	Molecule	Event Date
VGN-R09b	Regulatory - Rare Pediatric Disease (RPD) Designation	Metabolic - General	Viral Gene Therapy	12/01/2025
VX-670	Regulatory - Orphan Drug Designation (Japan)	Myotonic Muscular Dystrophy	Oligonucleotide	11/27/2025
Rese-cel	Regulatory - Orphan Drug Designation (Europe)	Systemic Sclerosis	Cellular	11/21/2025
Redemplo	Regulatory - Approval (U.S.)	Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	siRNA/RNAi	11/18/2025
MB-105 (March Bio)	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Adult T-cell Leukemia/Lymphoma (ATL)	Cellular	11/11/2025
SGT-003	Regulatory - Innovative Licensing and Access Pathway (ILAP) (U.K.)	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	11/06/2025
NRTX-1001	Regulatory - PRIME Designation (Europe)	Partial / Focal Seizures (Epilepsy)	Cellular	11/05/2025
BB-301	Regulatory - Fast Track Status	Oculopharyngeal Muscular Dystrophy (OPMD)	Viral Gene Therapy	11/03/2025
Kygevvi	Regulatory - Approval (U.S.)	Metabolic - General	Other Nucleic Acid	11/03/2025
Kygevvi	Regulatory - Rare Pediatric Disease (RPD) Designation	Metabolic - General	Other Nucleic Acid	11/03/2025
GTX-102	Regulatory - Orphan Drug Designation (Europe)	Angelman Syndrome	Antisense	10/30/2025
Lenmeldy	Regulatory - Orphan Drug Designation (Japan)	Metachromatic Leukodystrophy	Cellular	10/28/2025
NEU-001, Neurenati Therapeutics	Regulatory - Orphan Drug Designation (Europe)	Hirschsprung's Disease	Cellular	10/28/2025
HM2003, CirCode	Regulatory - Orphan Drug Designation (U.S.)	Buerger's Disease	Other Nucleic Acid	10/23/2025
ADX-324	Regulatory - Orphan Drug Designation (U.S.)	Hereditary Angioedema (HAE)	siRNA/RNAi	10/22/2025
OTL-203	Regulatory - Orphan Drug Designation (Europe)	Mucopolysaccharidosis I (MPS I; Hurler Syndrome)	Cellular	10/22/2025
RBD1016	Regulatory - Orphan Drug Designation (Europe)	Hepatitis D (HDV) (Antiviral)	siRNA/RNAi	10/22/2025

Key highlights in Q4 2025 (Noteworthy events that happened in Q4 2025) *continued*

Drug	Event Type	Indication	Molecule	Event Date
iduronicrin genleukocel-T	Regulatory - Fast Track Status	Mucopolysaccharidosis I (MPS I; Hurler Syndrome)	Cellular	10/21/2025
CK0803	Regulatory - Orphan Drug Designation (U.S.)	Amyotrophic Lateral Sclerosis (ALS)	Cellular	10/20/2025
AlloNK	Regulatory - Fast Track Status	Rheumatoid Arthritis (RA)	Cellular	10/16/2025
LTS-101	Regulatory - Orphan Drug Designation (U.S.)	Neuronal Ceroid Lipofuscinosis (NCL)	Viral Gene Therapy	10/16/2025
MVX-220	Regulatory - Orphan Drug Designation (U.S.)	Angelman Syndrome	Viral Gene Therapy	10/16/2025
SGB-9768	Regulatory - Orphan Drug Designation (U.S.)	C3 Glomerulopathy (C3G), including Dense Deposit Disease (DDD) and C3 Glomerulonephritis (C3GN)	siRNA/RNAi	10/16/2025
NG-350A	Regulatory - Fast Track Status	Colorectal Cancer (CRC)	Viral Gene Therapy	10/14/2025
Orca-T	Regulatory - Priority Review	Acute Myelogenous Leukemia (AML)	Cellular	10/06/2025
AVB-114	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Crohn's Disease	Cellular	10/03/2025
MNV-201	Regulatory - Orphan Drug Designation (U.S.)	Myelodysplastic Syndrome (MDS)	Cellular	10/03/2025
TSHA-102	Regulatory - Breakthrough Therapy Designation (U.S.)	Rett Syndrome	Viral Gene Therapy	10/02/2025

Source: Biomedtracker | Evaluate, December 2025

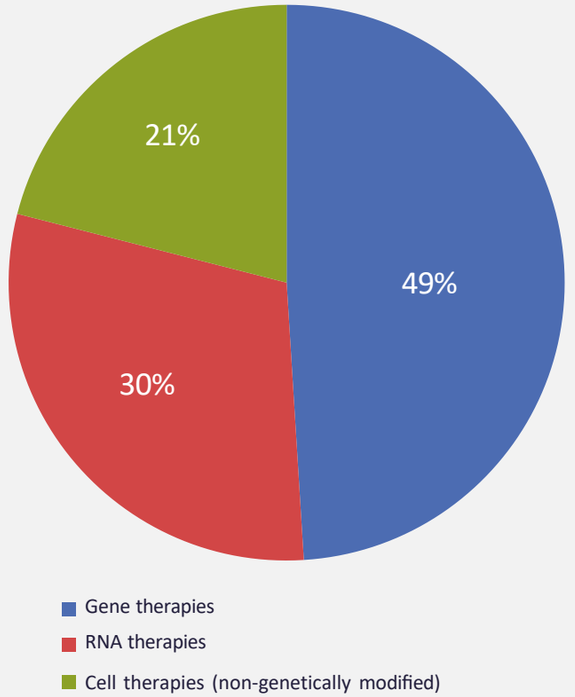
Pipeline overview

Pipeline of gene, cell, and RNA therapies

4,164 therapies are in development, ranging from preclinical through pre-registration

- 2,040 gene therapies (including genetically modified cell therapies such as CAR-T cell therapies) are in development, accounting for 49% of gene, cell, and RNA therapies
- 875 non-genetically modified cell therapies are in development, accounting for 21% of gene, cell, and RNA therapies

Pipeline therapies by category



Source: Pharmaprojects | Citeline, December 2025

Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Gene therapy pipeline: quarterly comparison

- A decrease in the number of gene therapy programs was seen at all stages of pipeline development except for Phase III assets, which increased by two
- Notably, rondecabtagene autoleucel (Lyell Immunopharma) and NGN-401 (Neurogene) advanced to Phase III status for B-cell lymphoma and Rett syndrome, respectively
- Therapies currently in pre-registration:

In the US

- marnetegragene autotemcel (Rocket Pharmaceuticals)
- clemidsogene lanparvovec (Regenxbio)
- vusolimogene oderparepvec (Replimune)
- sonpiretigene isteparvove (Nanoscope Therapeutics)
- pariglasgene brecaparvovec (Ultragenyx)
- cretostimogene grenadenorepvec (CG Oncology)
- isaralgagene civaparvovec (Sangamo Therapeutics)

In China

- satricabtagene autoleucel (CARsgen Therapeutics)
- donaperminogene seltoplasmid (Helixmith)
- IM-19 (Imunopharm)

In South Korea

- Anbal-cel (Curocell)

In Japan

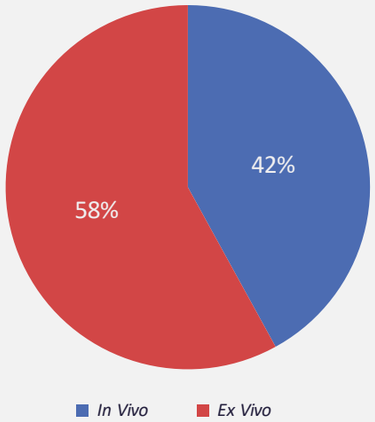
- suratadenoturev (Oncolys BioPharma)

Global Status	Q4 2024	Q1 2025	Q2 2025	Q3 2025	Q4 2025
Preclinical	1,424	1,432	1,461	1,346	1,227
Phase I	341	350	361	377	397
Phase II	306	319	330	347	355
Phase III	35	41	45	47	49
Pre-registration	11	13	13	11	12
Total	2,041	2,117	2,155	2,129	2,040

Source: Pharmaprojects | Citeline, December 2025

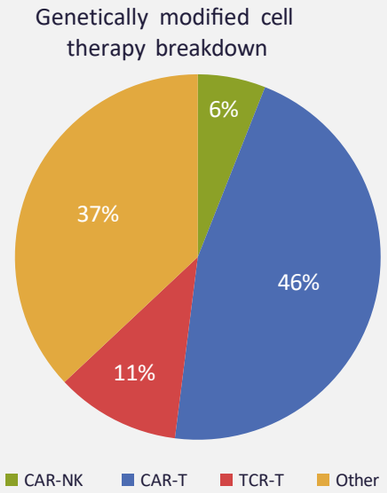
Genetic modification: *In vivo* vs. *Ex vivo*

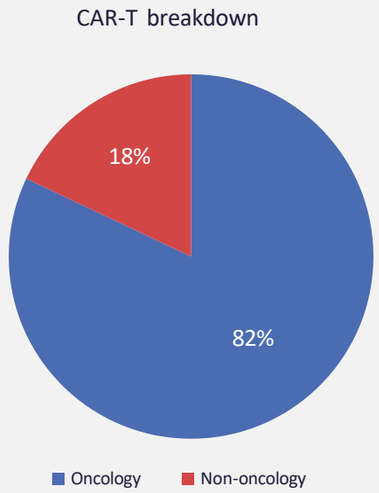
- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q4 2025, *In vivo* delivery techniques were used in 42% of gene therapies



Gene therapy breakdown: CAR-Ts continue to dominate the pipeline

- CAR-T cell therapies remained the most common technology used in the pipeline of genetically modified cell therapies (preclinical through to pre-registration), representing 46%, followed by the "other" category at 37%, which includes a list of less commonly used technologies such as TCR-NK, CAR-M, and TAC-T
- 82% of CAR-T cell therapies are in development for cancer indications; some CAR-T therapies are also in development for non-oncology diseases, while others are in development for only non-oncology indications, such as lupus, scleroderma, and myasthenia gravis



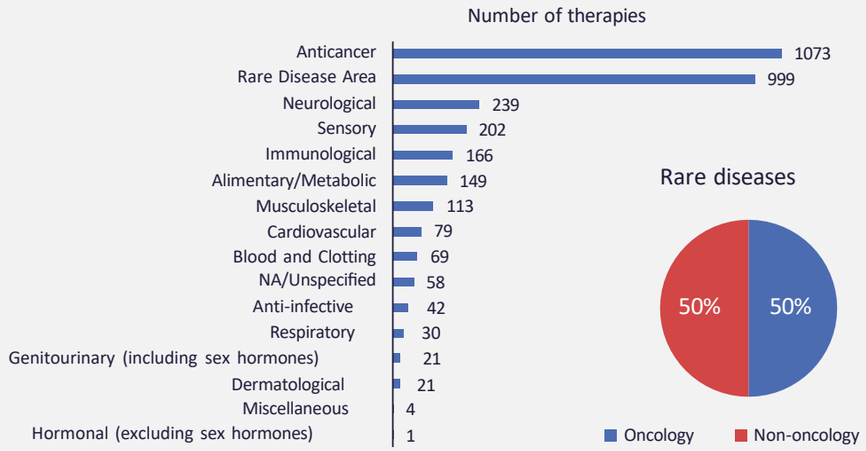


Source: Cell and Gene Therapy dashboard | Citeline, December 2025

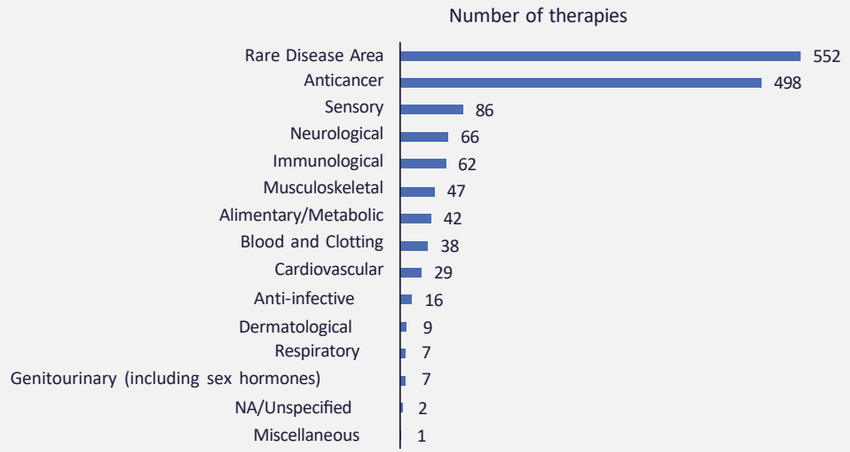
Gene therapy pipeline: most commonly targeted therapeutic areas

- Oncology and rare diseases remained the top areas of gene therapy development in both the overall pipeline (preclinical to pre-registration) and in the clinic (Phase I to pre-registration)
- Development for rare diseases was split equally between oncology and non-oncology rare diseases, with oncology representing two percentage points lower than the previous quarter

Number of therapies from preclinical through pre-registration



Therapies in the clinic (excludes preclinical development)



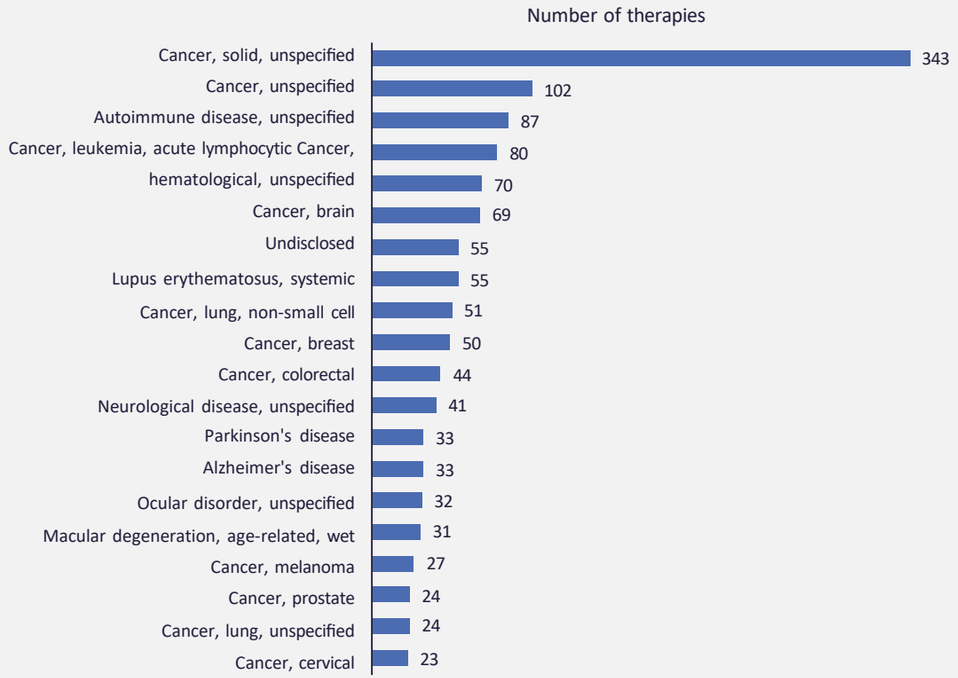
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

Gene therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q4 2025 were:

- Acute lymphocytic leukemia
- Brain cancer
- Systemic lupus erythematosus

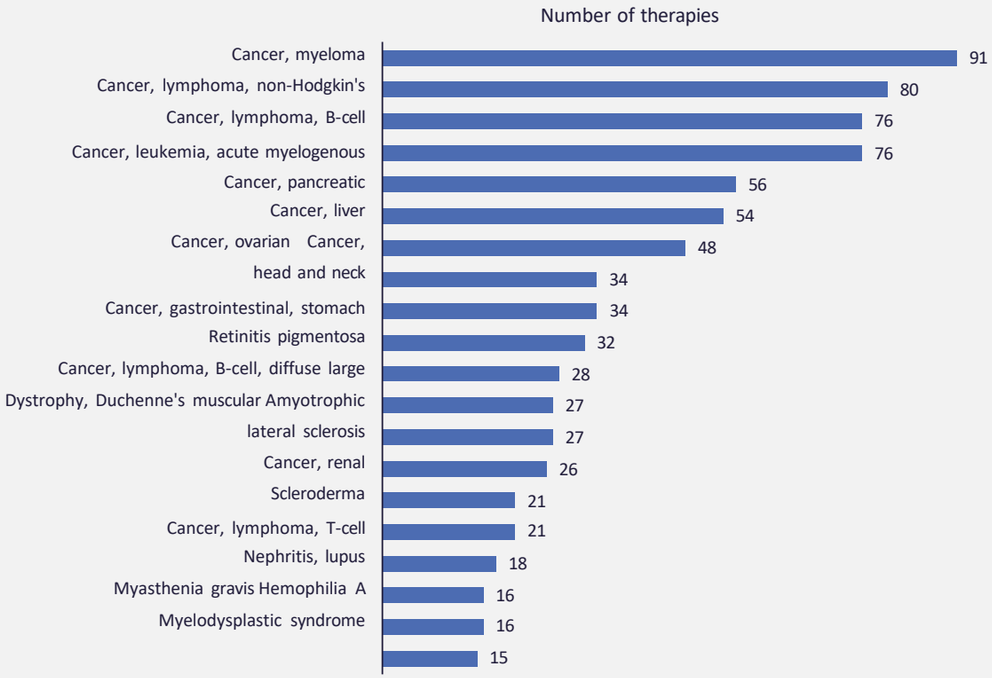


Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

Gene therapy pipeline: most common rare diseases targeted

- For the 1,395 pipeline (preclinical to pre-registration) gene therapies being developed for rare diseases, nine out of the top 10 rare diseases were oncological, all the same as last quarter
- The top five rare diseases for which gene therapies are being developed:
  - Myeloma
  - Non-Hodgkin’s lymphoma
  - B-cell lymphoma
  - Acute myelogenous leukemia
  - Pancreatic cancer



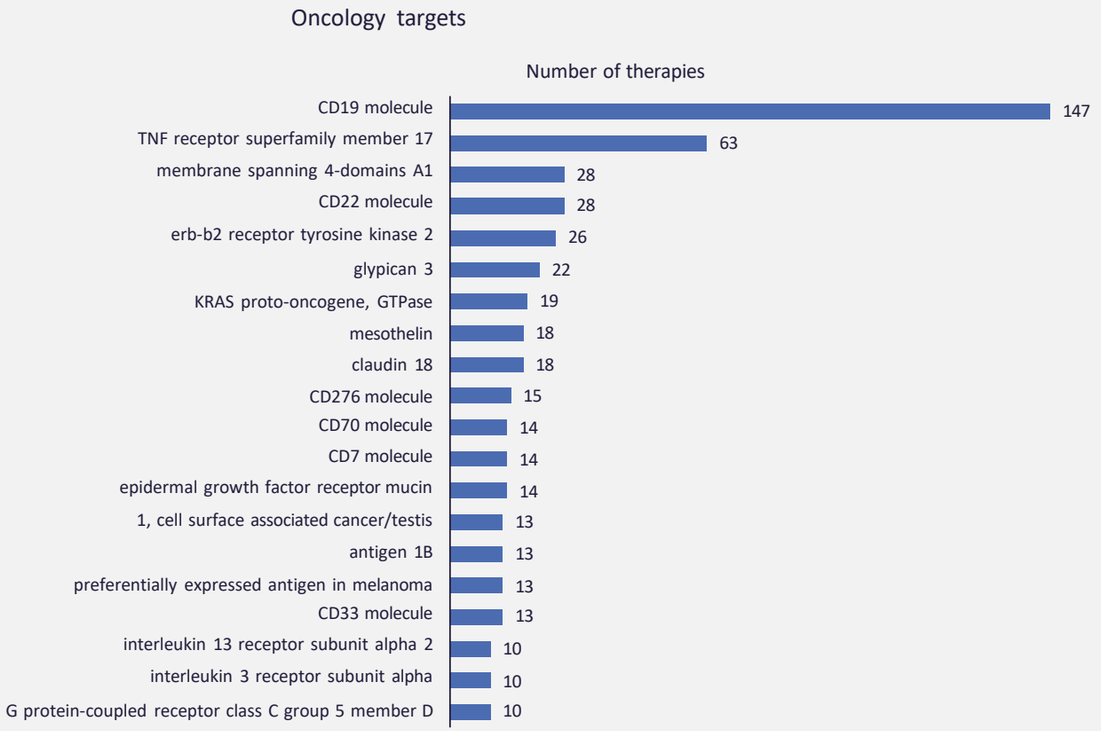
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

Gene therapy pipeline: most common targets

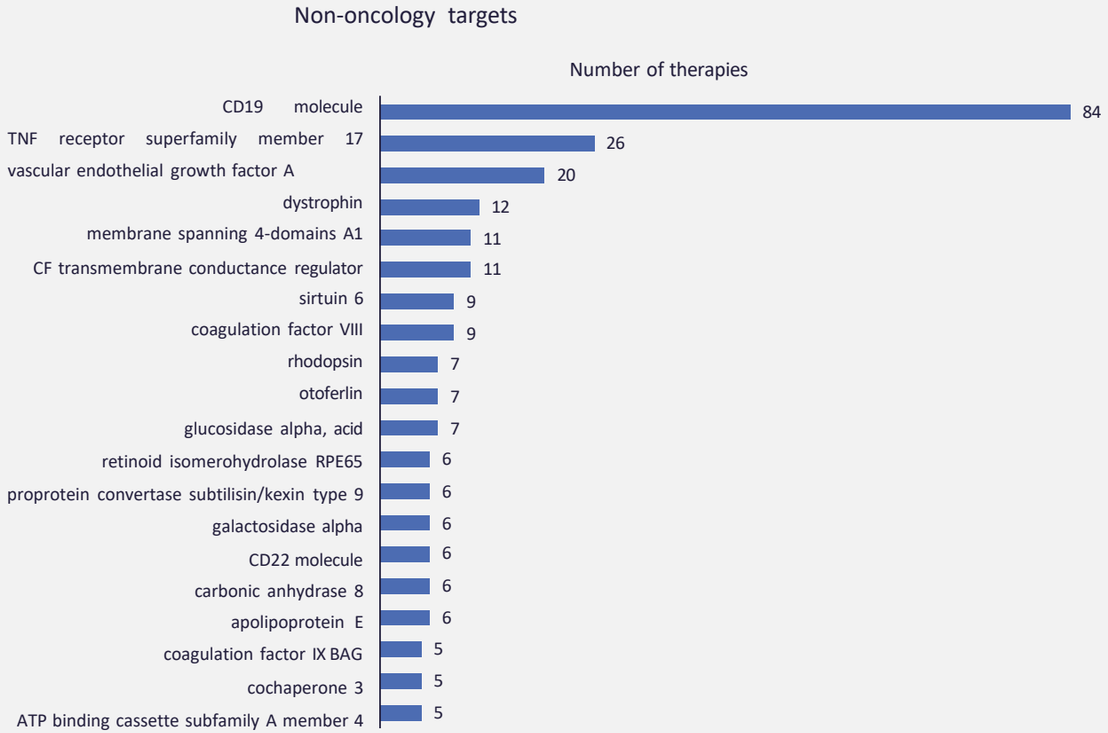
Of the gene therapies at preclinical through pre-registration for which targets were disclosed:

- CD19 molecule and B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17, remained the top two most common targets for oncology indications
- CD19 molecule, TNF receptor superfamily member 17, and vascular endothelial growth factor A continued to be the top three most common targets for non-oncology indications



Source: Pharmaprojects | Citeline, December 2025

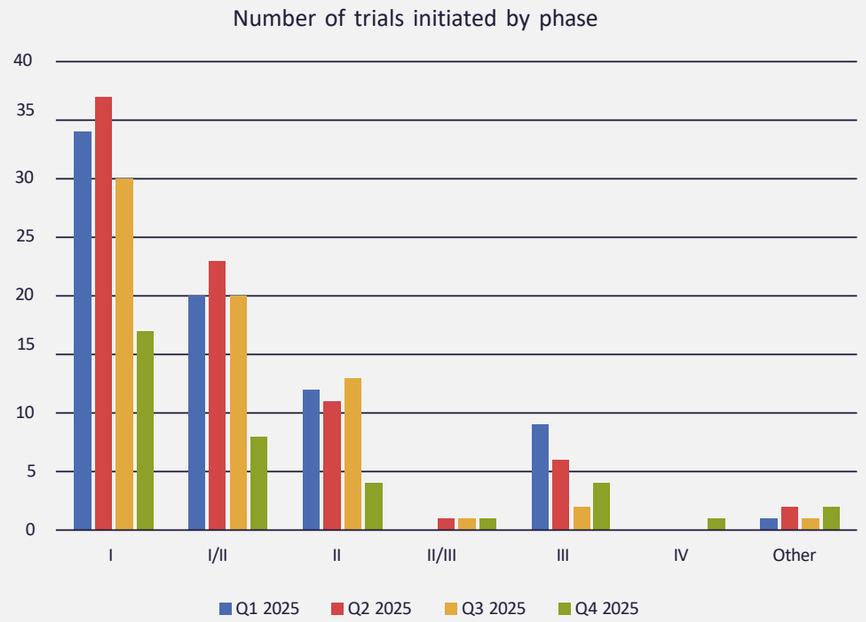
Gene therapy pipeline: most common targets *continued*

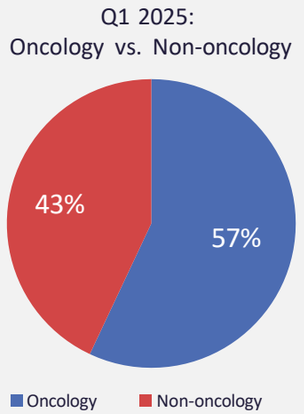


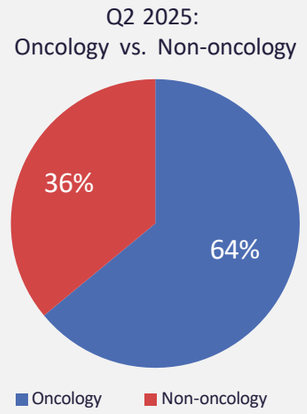
Source: Pharmaprojects | Citeline, December 2025

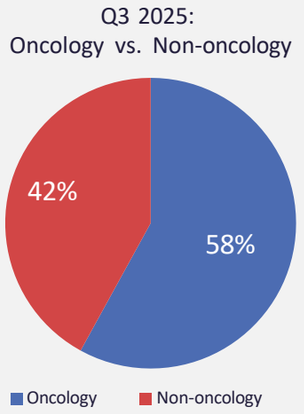
Gene therapy clinical trial activity

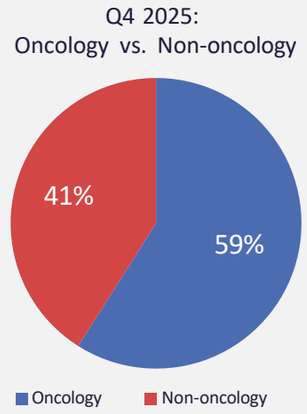
- The proportion of gene therapy trials for non-oncology indications decreased to 41%, one percentage point lower than last quarter
- 37 gene therapy trials were initiated in Q4 2025, 30 less than the previous quarter
- There are currently 1,888 open gene therapy clinical trials











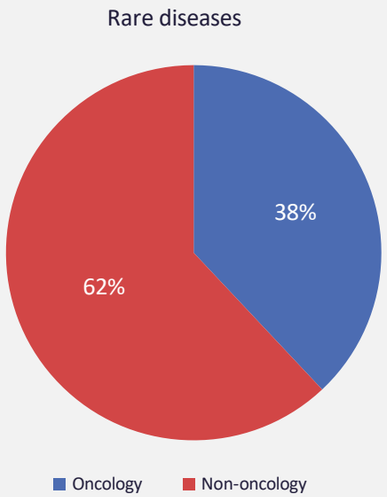
Source: Pharmaprojects | Citeline, December 2025

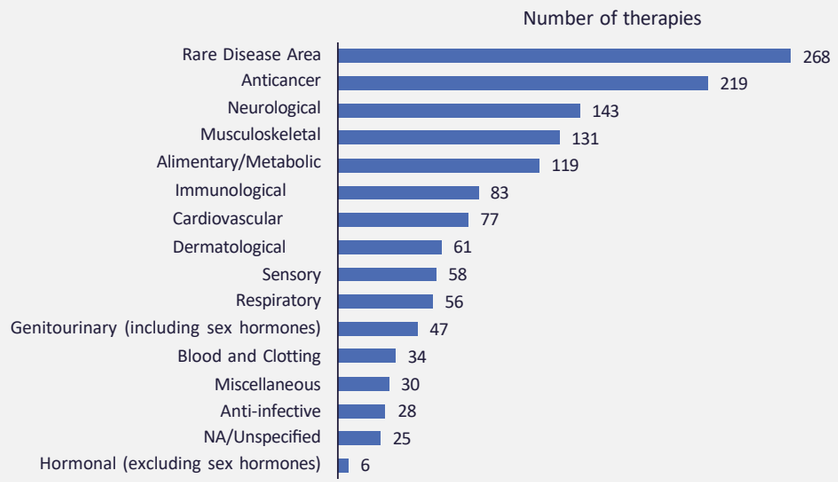
Non-genetically modified cell therapy pipeline

Non-genetically modified cell therapy pipeline: most commonly targeted therapeutic areas

Of the cell therapies in development (preclinical through pre-registration):

- Oncology and rare diseases remained the top areas of non-genetically modified cell therapy development
- Of the non-genetically modified cell therapies in preclinical to pre-registration stages for rare diseases, 62% were in development for non-oncology rare diseases, a one percentage point decrease from the previous quarter





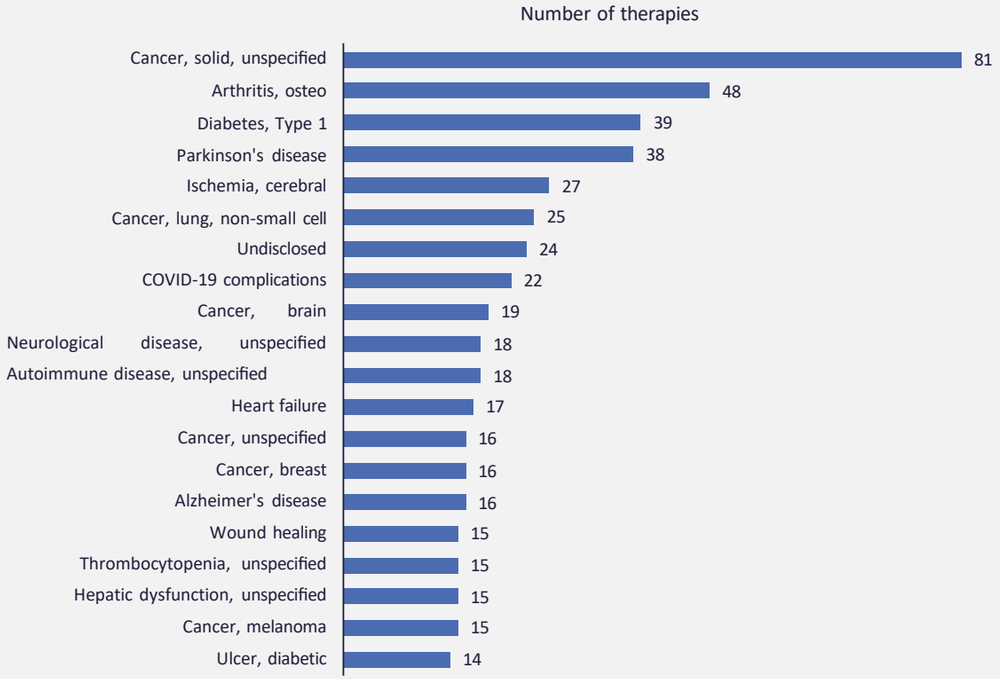
Note: Figures based on indications in pipeline developent only for each therapy

Source: Pharmaprojects | Citeline, December 2025

Non-genetically modified cell therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q4 2025 were:

- Osteoarthritis
- Type 1 diabetes
- Parkinson’s disease



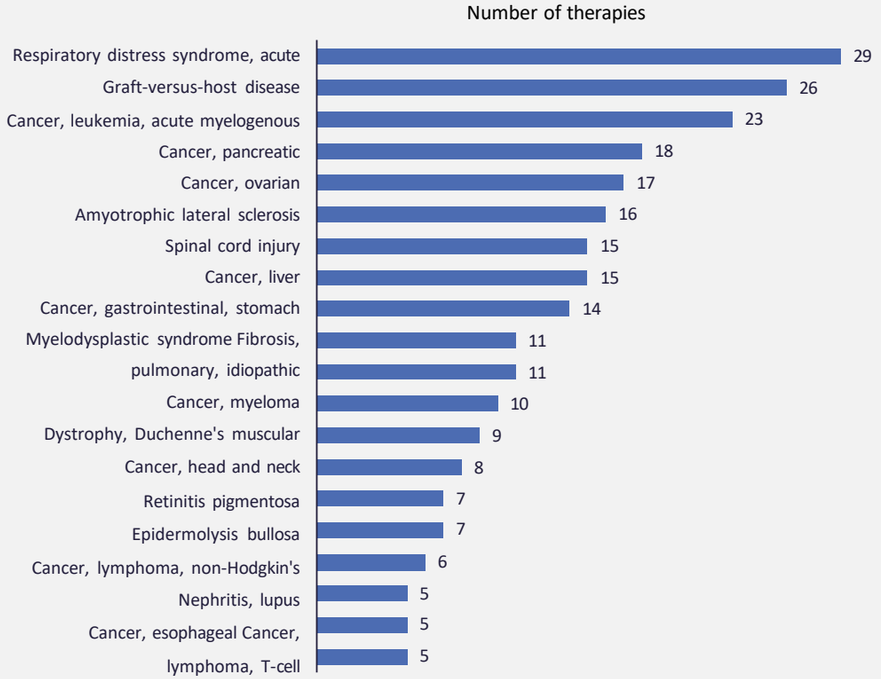
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

Non-genetically modified cell therapy pipeline: most common rare diseases targeted

Of the therapies in development (preclinical through pre-registration) for rare diseases:

- The top three oncology indications were acute myelogenous leukemia, pancreatic cancer, and ovarian cancer
- The top three non-oncology indications were acute respiratory distress syndrome, graft-versus-host disease, and amyotrophic lateral sclerosis

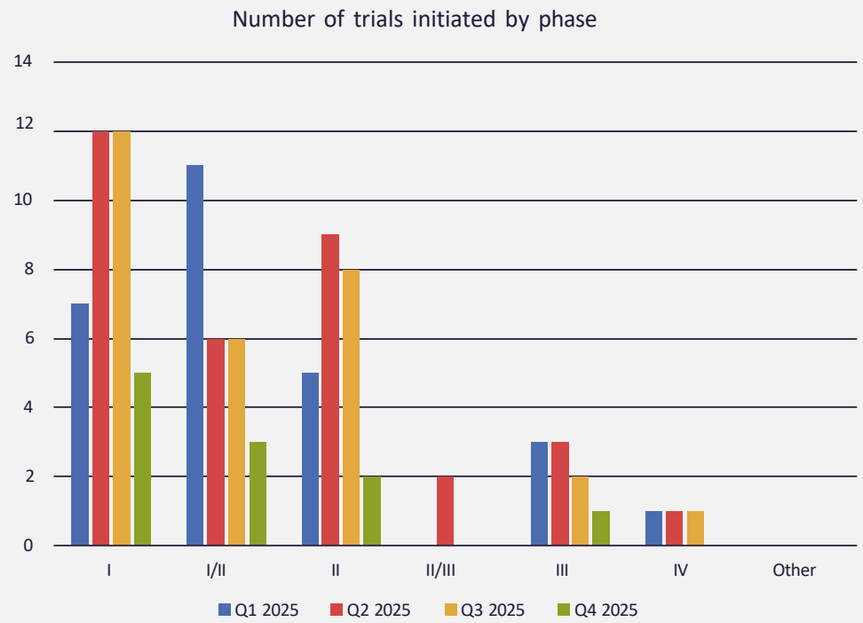


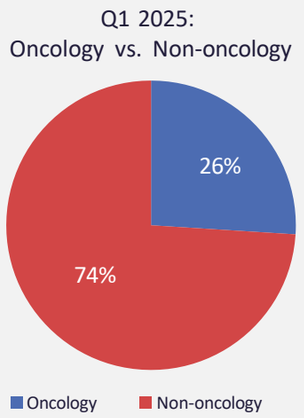
Note: Figures based on indications in pipeline development only for each therapy

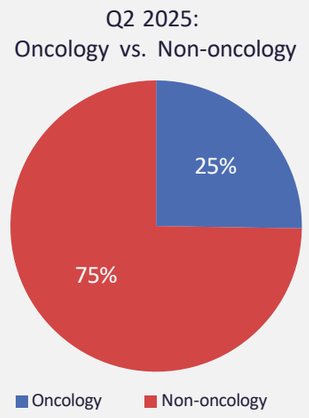
Source: Pharmaprojects | Citeline, December 2025

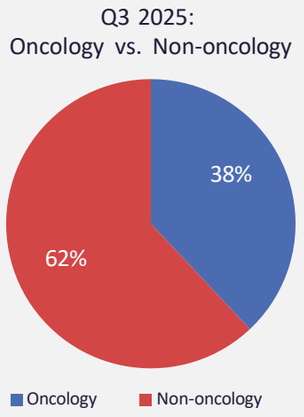
Non-genetically modified cell therapy trial activity

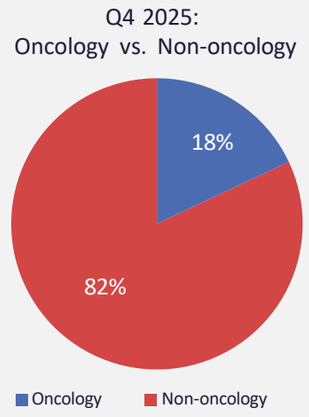
- 11 trials were initiated for non-genetically modified cell therapies in Q4 2025, 18 less than in Q3 2025
- Of these 11, 82% were for non-oncology indications, 20 percentage points higher than the previous quarter
- There are currently 842 open non-genetically modified cell therapy clinical trials









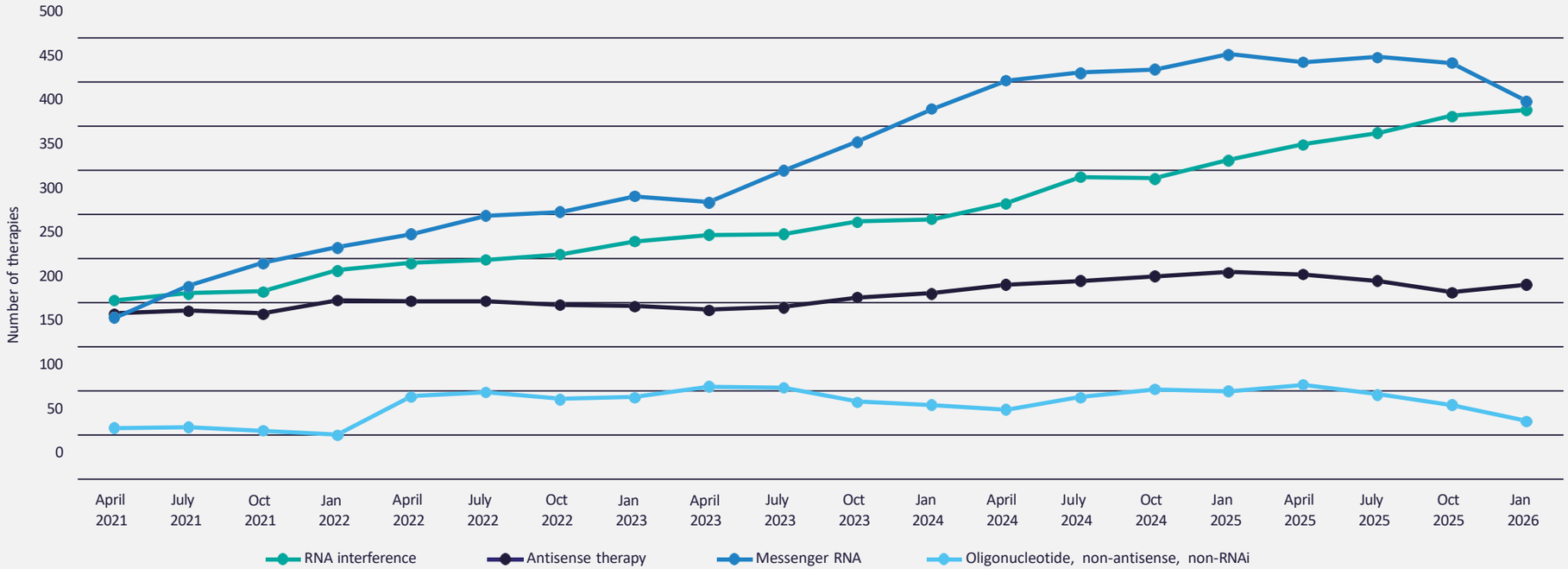


Source: Pharmaprojects | Citeline, December 2025

RNA therapy pipeline

RNA therapy pipeline: most common modalities

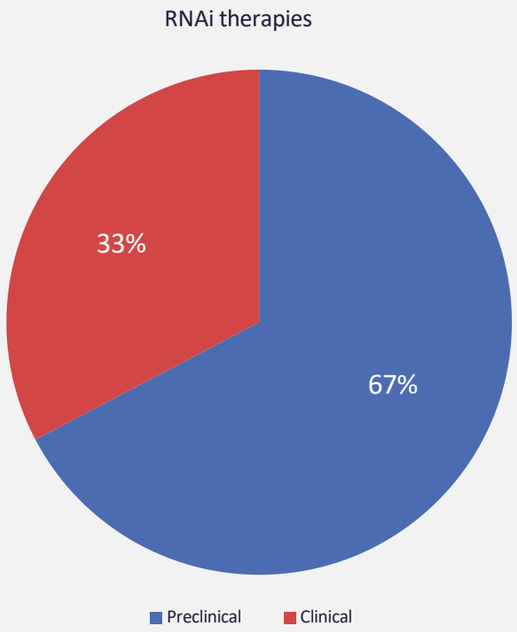
- Of RNA therapies in the pipeline, messenger RNA (mRNA) and RNA interference (RNAi) continued to be the preferred RNA modalities for research; RNAi is trending to overtake mRNA as the most common RNA modality

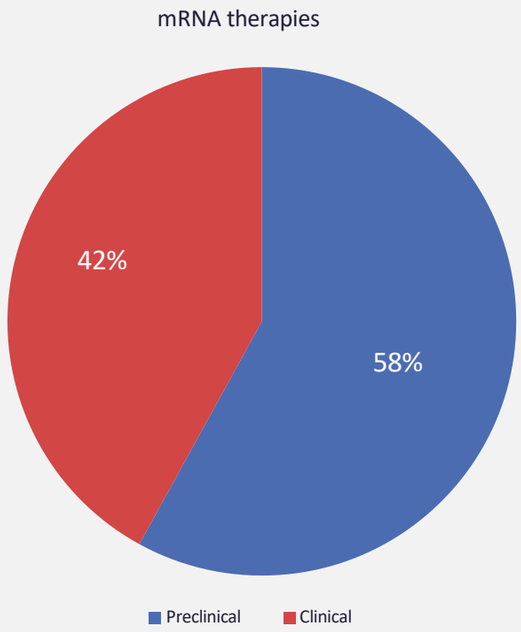


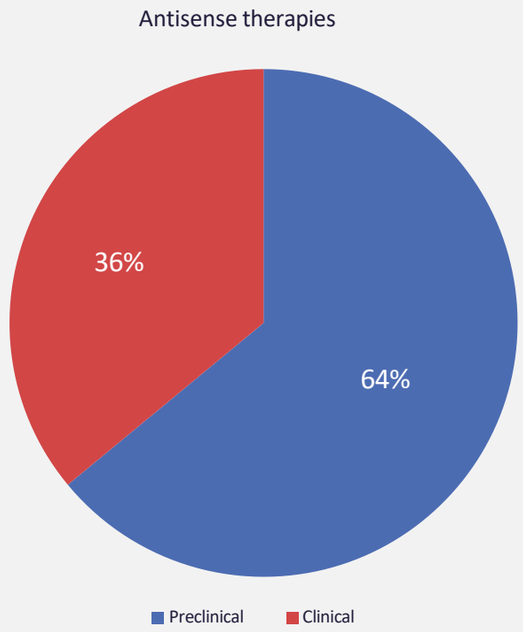
Source: Pharmaprojects | Citeline, December 2025

RNAi, mRNA, and antisense oligonucleotides: preclinical vs. clinical

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 67%, 58%, and 64% of their respective pipelines





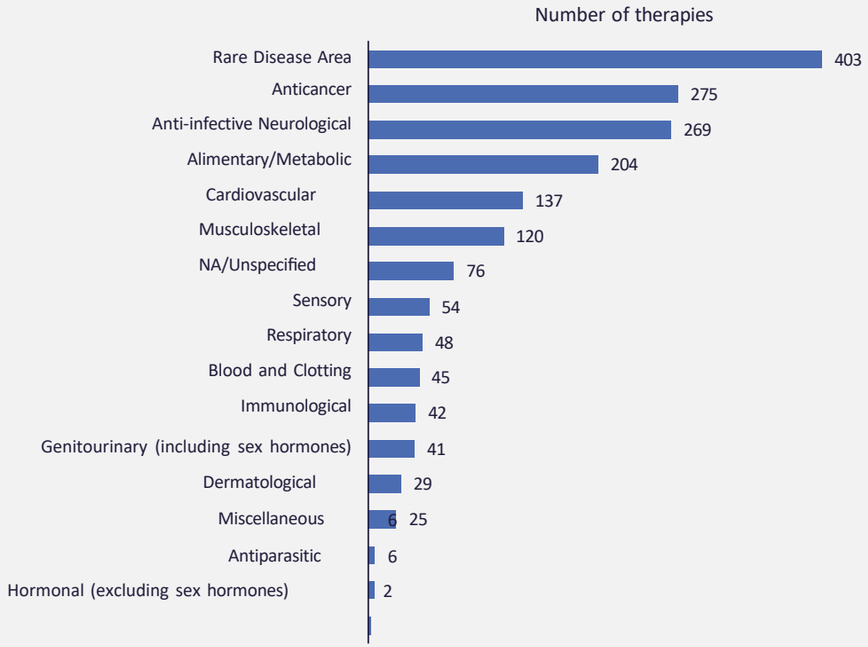


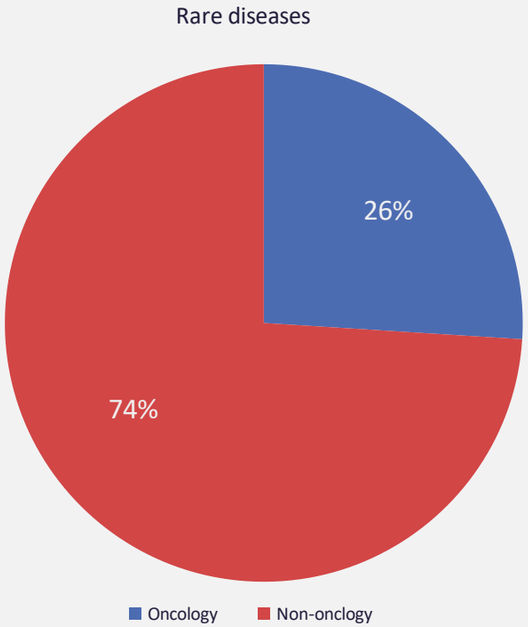
Source: Pharmaprojects | Citeline, December 2025

RNA therapies: most commonly targeted therapeutic areas

Of the 1,248 RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Rare diseases remained the top targeted therapeutic area by RNA therapies, and oncology surpassed anti-infective as the second most targeted therapeutic area in Q4 2025
- Non-oncology indications continued to be the most targeted rare diseases by RNA therapies, representing a majority of 74%





Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

RNA therapies: most common rare diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q4 2025 were:

- Coronavirus prophylaxis
- Influenza prophylaxis
- Obesity

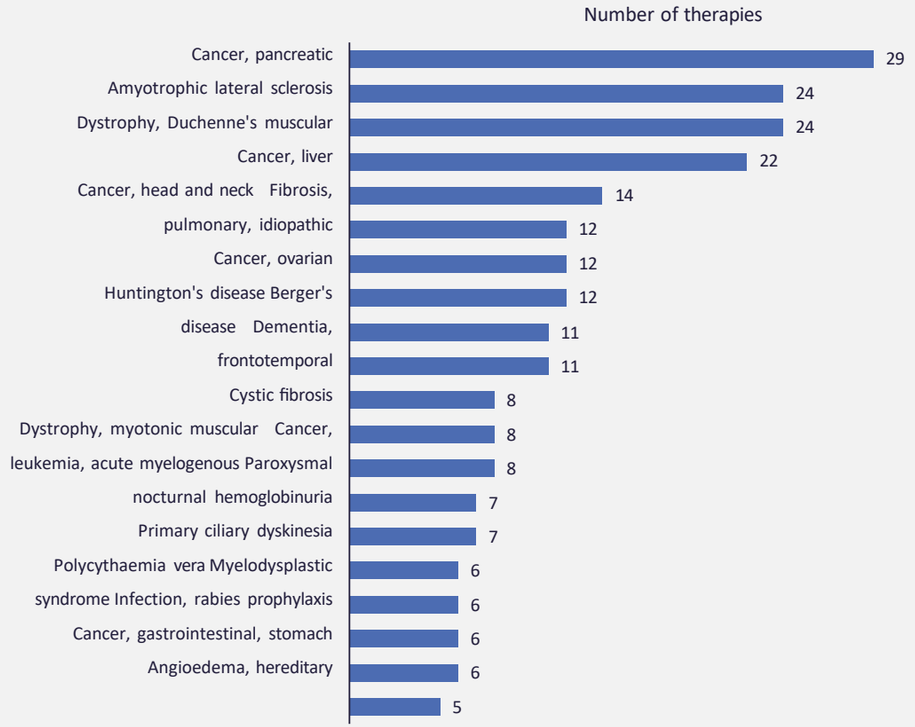
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

RNA therapies: most common rare diseases targeted

Of the RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Top specified rare oncology indications were pancreatic, liver, and head and neck cancer
- For non-oncology rare diseases, amyotrophic lateral sclerosis, Duchenne muscular dystrophy, and idiopathic pulmonary fibrosis were the most targeted indications

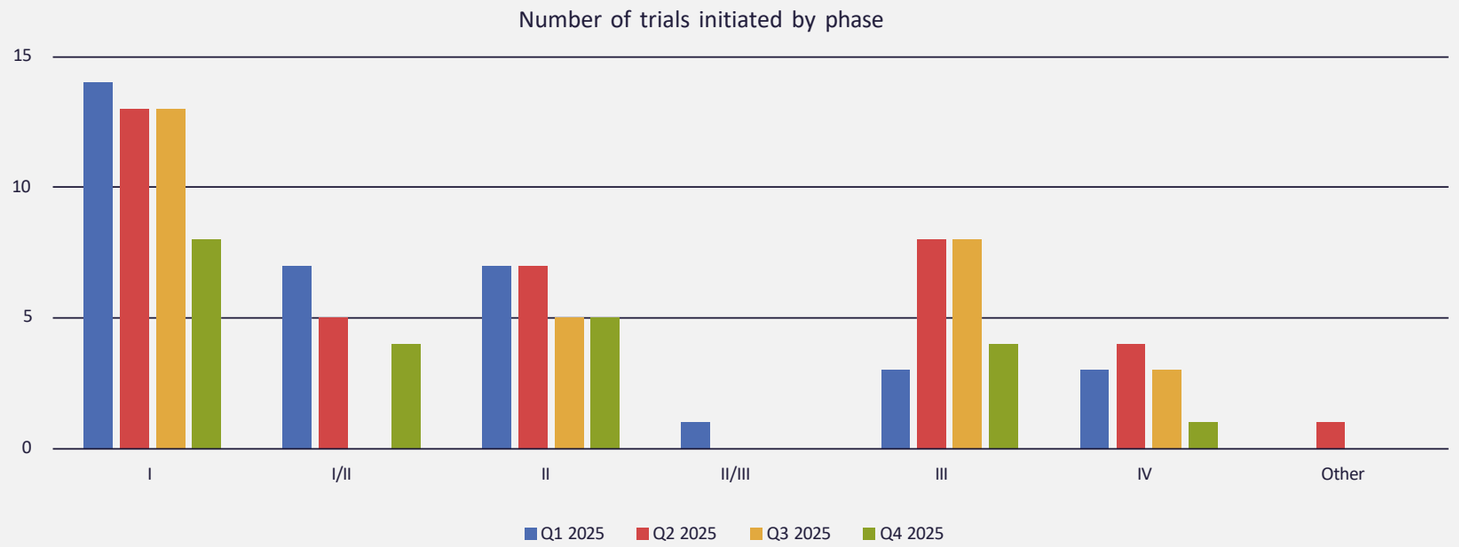


Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, December 2025

RNA therapy pipeline: clinical trial activity

- 22 RNA trials were initiated in Q4 2025, compared to 29 in Q2 2025, 77% of which were for non-oncology indications
- There are currently 480 open RNA therapy clinical trials

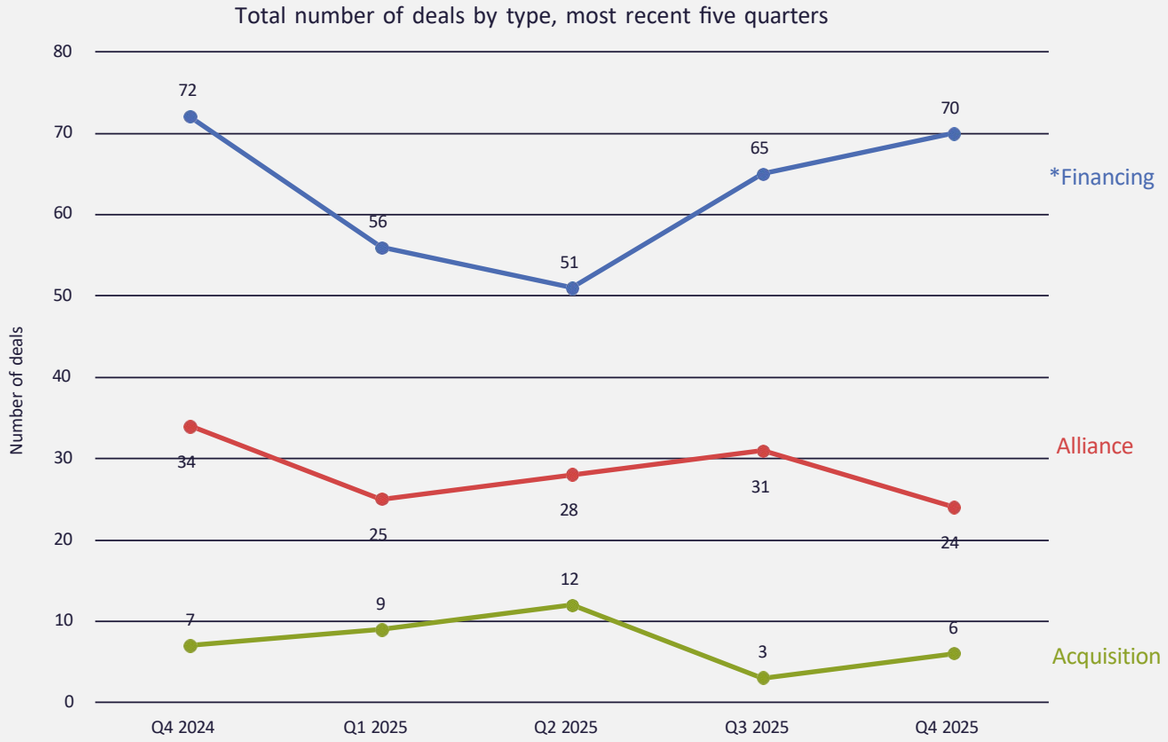


Source: Trialtrove | Citeline, December 2025

Overview of dealmaking for gene, cell, and RNA therapy companies

Alliance, acquisition, and financing in gene, cell, and RNA therapy

- In Q4 2025, advanced molecular therapy companies reached a total of 100 deals, virtually flat from the previous quarter's 99 deals
- Year over year, dealmaking experienced a decline, with a 12% decrease in Q4 2025 compared to the 113 total from the same quarter last year
- Financing volume continues to grow quarterly, and there was a jump in acquisition activity in Q4 2025



*Financings include public financings (IPOs and follow-ons) plus privately raised funding through venture rounds, debt offerings, or private investment in public equity

Sources: Biomedtracker, BioSciDB | Evaluate, December 2025

Q4 2025 acquisitions in gene, cell, and RNA therapy

- The volume of advanced molecular therapy acquisitions doubled in Q4 2025 to six transactions, up from only three in the previous quarter
- The quarter featured three multi-billion-dollar RNA technology acquisitions: in the largest, Novartis will spend up to $12 billion on Avidity Biosciences and its antibody oligonucleotide conjugates in the neuroscience area; Sanofi paid $2.2 billion for Dynavax, whose pipeline includes synthetic oligonucleotides targeting toll-like receptors; and for $1.5 billion, Bristol Myers Squibb now owns Orbital Therapeutics and its lead RNA encoding CD19-targeted CAR therapy OTX-201

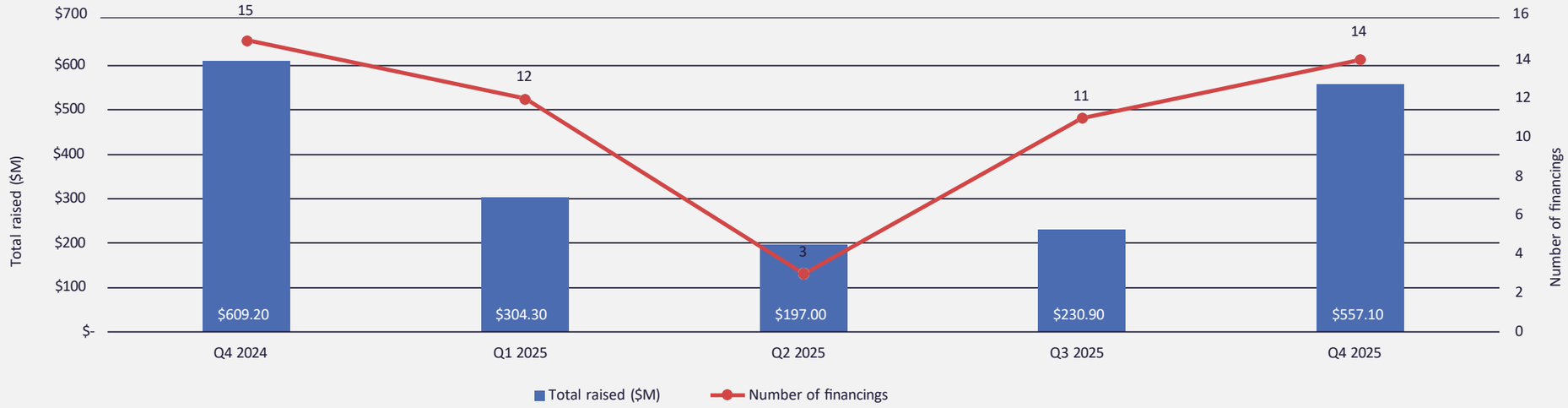
Deal date	Deal title	Potential deal value (USD $)
10 October 2025	Bristol Myers Squibb to Acquire Orbital Therapeutics	1,500,000,000
24 October 2025	Lilly to Acquire Adverum Biotechnologies; Acquisition Completed	Undisclosed
26 October 2025	Novartis to Acquire Avidity Biosciences for $12B	12,000,000,000
14 November 2025	Repare Therapeutics Enters into Definitive Agreement to be Acquired by XenoTherapeutics	Undisclosed
15 December 2025	XOMA Royalty Enters into Agreement to Acquire Generation Bio	Undisclosed
24 December 2025	Sanofi to Acquire Dynavax for $2.2B	2,200,000,000

Sources: Biomedtracker, BioSciDB | Evaluate, December 2025

Start-up funding for gene, cell, and RNA therapy companies

- Start-up advanced molecular companies completed 14 seed and Series A financings in Q4 2025, together valued at $557.1 million
- Q4 2025's total represented a 27% increase in volume and 141% increase in value compared with Q3 2025, but still slightly behind the 15 financings worth $609.2 million in 2024's final quarter
- Leading the group in Q4 2025 was Soufflé Therapeutics, which raised $200 million to support work on siRNA therapies

Volume and dollar value of Series A and seed financings for gene, cell, & RNA therapy companies, most recent five quarters



Source: Biomedtracker | Evaluate, December 2025

Q4 2025 start-up financing for gene, cell, and RNA therapy companies

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
01 October 2025	Aerska Gets $21M in Seed Round	Antibody-oligonucleotide conjugates	Ireland/Dublin	Founded by former scientists from Ochre Bio and Alnylam	21
02 October 2025	Repairon Closes Series A Financing to Expand the Clinical Development of Its Regenerative Heart Therapy	Cell therapy	Germany/Gottingen	University Medical Center Goettingen's Institute of Pharmacology and Toxicology	Undisclosed
02 October 2025	Cirrus Therapeutics $11M Seed Financing	Gene therapy	United States/Massachusetts/ Cambridge	University of Bristol; UCL Institute of Ophthalmology	11
06 October 2025	Trogenix Closes $95M Series A Financing	Gene therapy	United Kingdom/Edinburgh	University of Edinburgh's UK Centre for Mammalian Synthetic Biology and Institute for Regeneration and Repair; Cancer Research UK Scotland Centre	95
07 October 2025	Soufflé Therapeutics Raises $200M in Series A Financing	siRNA	United States/Massachusetts/Boston	MIT	200
29 October 2025	Rarity PBC Gets $4.6M in Seed Financing	Autologous genetically modified cell therapy	United States/California/Los Angeles	UCLA; University College London	4.6
30 October 2025	Helex Raises $3.5M in Seed Round to Support Gene Therapy Development	Gene editing	United States/New York/New York	King’s College London	3.5
04 November 2025	Azalea Launches with $82M in Seed and Series A Funding	In vivo CAR-T	United States/California/Berkeley	University of California	82
13 November 2025	RAGE Biotech Completes $A29M Series A Financing	Precision splice-switching oligonucleotides	Australia/Melbourne	Monash University	19
20 November 2025	Cassidy Bio Raises $8M in Seed Financing	Gene editing	Israel/Tel Aviv	Founded by biotech entrepreneurs, CRISPR scientists, and AI specialists	8
10 December 2025	EpilepsyGTx Raises $33M Series A Round to Develop Single-Dose Gene Therapy for FRE	Gene therapy	United Kingdom/Cambridgeshire	UCL's Queen Square Institute of Neurology	33
15 December 2025	Link Cell Therapies Completes $60M Series A Financing	CAR-T	United States/California/South San Francisco	Stanford University	60
17 December 2025	T-CURX Gets $20M in Series A Financing	CAR-T	Germany/Würzburg	University of Würzburg	20
22 December 2025	RheumaGen Gets Series A-1 Investment from Beyond Celiac	HLA editing	United States/Colorado/Aurora	University of Colorado	Undisclosed

Source: Biomedtracker | Evaluate, December 2025

Notable Q4 2025 start-up gene, cell, and RNA therapy companies

Company details	Academic source	Financing type/ amount raised	Lead investor(s)	Therapy areas of interest
Precision targeting of nucleic acids via cell-specific ligands to deliver siRNA-based medicines	MIT	Series A/$200M	Bessemer Venture Partners	Musculoskeletal (facioscapulohumeral muscular dystrophy) and cardiovascular (heart failure)
Synthetic Super Enhancer technology: AAV vector delivery of enzyme converting oral prodrug into cytotoxic agent, and a payload expressing IL-12	University of Edinburgh's UK Centre for Mammalian Synthetic Biology and Institute for Regeneration and Repair; Cancer Research UK Scotland Centre	Series A/$95M	IQ Capital	Oncology (treatment resistant solid tumors, including glioblastoma)
Genome editing, delivered via Enveloped Delivery Vehicles, to engineer T cells in vivo	University of California	Combined seed and Series A/$82M	Third Rock Ventures	Oncology and autoimmune diseases

Source: Biomedtracker | Evaluate, December 2025

Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q1 2026

Therapy	Generic name	Disease	Catalyst	Catalyst date
Tabelecleucel	tabelecleucel	Hematologic Cancer	PDUFA for BLA - 2nd Review	10 January 2026 - 10 January 2026
Waskyra	etuvetidigene autotemcel	Wiskott-Aldrich Syndrome	Approval Decision (EU)	13 November 2025 - 19 January 2026
Kresladi	marnetegragene autotemcel	Autoimmune Disorders	PDUFA Decision	28 March 2026 - 28 March 2026
DSP-1083		Parkinson's Disease (PD)	Approval Decision (Japan)	01 April 2025 - 31 March 2026
Kygevvi	doxecitine, doxribtimine	Metabolic - General	CHMP Opinion	01 September 2025 - 31 March 2026
Dawnzera	donidalorsen	Hereditary Angioedema (HAE)	Approval Decision (Europe)	01 January 2026 - 31 March 2026
UX111	rebisufligene etisparvovec	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A Syndrome)	BLA Filing Resubmission (FDA)	01 January 2026 - 30 April 2026
Kygevvi	doxecitine, doxribtimine	Metabolic - General	Approval Decision (Europe)	01 November 2025 - 31 May 2026
Telomelysin	suratadenoturev	Esophageal Cancer	J-NDA Approval	15 December 2025 - 30 June 2026
BBM-H901	dalnacogene ponparvovec	Hemophilia B	CHMP (European Panel) Results	01 January 2026 - 31 Jul 2026
FF-31501		Cartilage and Joint Repair	J-NDA Approval (Japan)	01 February 2026 - 31 Aug 2026
BBM-H901	dalnacogene ponparvovec	Hemophilia B	Approval Decision (Europe)	01 March 2026 - 30 September 2026
Beqvez	fidanacogene elaparvovec	Hemophilia B	Approval Decision (Japan)	25 November 2025 - 31 March 2027
Zemcelpro	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	25 November 2025 - 31 March 2027

Source: Biomedtracker | Evaluate, December 2025

Appendix

Methodology, sources, and glossary of key terms

Methodology: sources and scope of therapies (Sources for all data come from Citeline and from Evaluate Ltd.)

Pipeline and trial data

- Data derived from Pharmaprojects and Trialtrove, part of Citeline
- Therapeutic classes included in report categorizations:
  - Gene therapies: gene therapy; cellular therapy, chimeric antigen receptor; cellular therapy, T cell receptor; lytic virus
  - Cell therapies: cellular therapy, other; cellular therapy, stem cell; cellular therapy, tumor-infiltrating lymphocyte
  - RNA therapies: messenger RNA; oligonucleotide, non-antisense, non-RNAi; RNA interference; antisense therapy

Deal, financing, and catalyst data

- Data derived from Biomedtracker, part of Evaluate Ltd. The following industry categorizations of deals are included: gene therapy, cell therapy; antisense, oligonucleotides
- Additional alliance and acquisition deals data from BioSciDB, part of Evaluate Ltd. The following industry categorizations of deals are included: cell therapy – stem cells/factors, oligonucleotides, antisense/triple helix, gene therapy, RNAi

Glossary of Key Terms

Therapy type definitions

For the purpose of this report, the following terms shall mean the following:

Cell therapy includes the following therapeutic classes:	
Cellular therapy, other	Cellular therapies that do not fall under the categories of cellular therapy, stem cell; cellular therapy, CAR; cellular therapy, TIL; cellular therapy, TCR; or the specific cellular therapy are unspecified
Cellular therapy, stem cell	Regenerative therapy which promotes the repair response of injured tissue using stem cells (cells from which all other specialized cells would originate)
Cellular therapy, tumor-infiltrating lymphocyte	Adoptive cellular transfer of tumor-resident T cells from tumor material, their expansion ex vivo, and transfer back into the same patient after a lymphodepleting preparative regimen

Gene therapy is the use of genetic material to treat or prevent disease.	
Cellular therapy, chimeric antigen receptor (falls under gene	Cellular therapy consisting of T cells that have been modified to express a chimeric antigen receptor (CAR) – this is a cell surface receptor that gives the T cells the therapy in this report) ability to target a specific protein and fight the targeted cells
Cellular therapy, T cell receptor (falls under gene therapy in this report)	Cellular therapies whereby natural T cells collected for the patient are engineered to express artificial receptors (usually through viral transfections) that would target specific intracellular antigens (as peptides bound to proteins encoded by the major histocompatibility complex, MHC)
Gene therapy	Therapies containing an active ingredient synthesized following vector-mediated introduction of a genetic sequence into target cells in- or ex-vivo. Used to replace defective or missing genes (as in cystic fibrosis) as well as to introduce broadly acting genetic sequences for the treatment of multifactorial diseases (e.g., cancer). Direct administration of oligonucleotides without using vectors is covered separately in the antisense therapy class; RNA interference class; or oligonucleotide, non-antisense, non-RNAi class. Platform technologies for gene delivery are covered separately in the gene delivery vector class
Lytic virus (falls under gene therapy in this report)	Therapies that have a replication-competent virus, that lyse pathogenic cells directly. These are normally genetically modified to render them harmless to normal tissues. Examples include oncolytic viruses that specifically attack cancer cells

RNA therapy includes the following therapeutic classes:	
Antisense therapy	Antisense compounds under development as potential therapeutics. These may be synthetic oligonucleotides, or antisense RNA may be expressed from a vector as a form of gene therapy. They may prevent the expression of a specific protein in vivo by binding to and inhibiting the action of mRNA, since they have a specific oligonucleotide sequence which is complementary to the DNA or RNA sequence that codes for the protein
Messenger RNA	Therapies that carry the desired mRNA code to overcome genetic mutations. The mRNA sequence will replace the defective mRNA in a patient and start producing the desired protein
Oligonucleotide, non-antisense, non-RNAi	Synthetic therapeutic oligonucleotides which operate by a mechanism other than antisense or RNA interference (RNAi). This includes ribozymes, aptamers, decoys, CpGs, and mismatched and immunostimulant oligonucleotides. Sequences delivered using vectors (gene therapy) are covered separately in “gene therapy.” Antisense and RNAi oligonucleotides are covered separately in “antisense therapy” and “RNA interference,” respectively
RNA interference	Includes products which act therapeutically via an RNA interference (RNAi) mechanism, including small interfering RNAs (siRNAs). These may be synthetic oligonucleotides, or RNAi sequences may be expressed from a vector as a form of gene therapy (see “gene therapy” therapeutic class). In vivo, these sequences block the expression of a specific protein by forming an RNA-induced silencing complex, which then specifically binds to and degrades a complementary mRNA encoding the target protein. The use of RNAi purely as a drug discovery tool (e.g., in transgenic animal model production or in target validation) is not covered in this section

Deal type categories	
Alliances	Co-marketing, co-promotion, disease management, joint venture, manufacturing or supply, marketing-licensing, product or technology swap, product purchase, R&D and marketing-licensing, reverse licensing, trial collaborations
Financing	Convertible debt, FOPO, IPO, nonconvertible debt, financing/other, private investment in public equity, private placement, royalty sale, special-purpose financing vehicle, spin-off
Acquisitions	Buy-out, divestiture, spin-out, full acquisition, partial acquisition, reverse acquisition

Development status definitions	
Pipeline	Drugs that are in active development
Preclinical	Not yet tested in humans
Phase I	Early trials, usually in volunteers, safety, PK, PD
Phase II	First efficacy trials in small numbers of patients
Phase III	Large-scale trials for registrational data
Pre-registration	Filing for approval made to regulatory authorities
Approved	Approval from relevant regulatory authorities for human use

Unspecified indications	
Cancer, unspecified	Indications for which the specific tumor type is not specified
Cancer, hematological, unspecified	Indications for which the specific hematological cancer is not specified
Cancer, solid, unspecified	Indications for which the specific solid tumor is not specified

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Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, biotechnology, and pharmaceutical companies. ASGCT advances knowledge, awareness, and education leading to the discovery and clinical application of gene and cell therapies to alleviate human disease to benefit patients and society.

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