American Society of Gene + Cell Therapy

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Gene, Cell, & RNA Therapy
Landscape Report

Q3 2025 Quarterly Data Report



November 2025

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Introduction

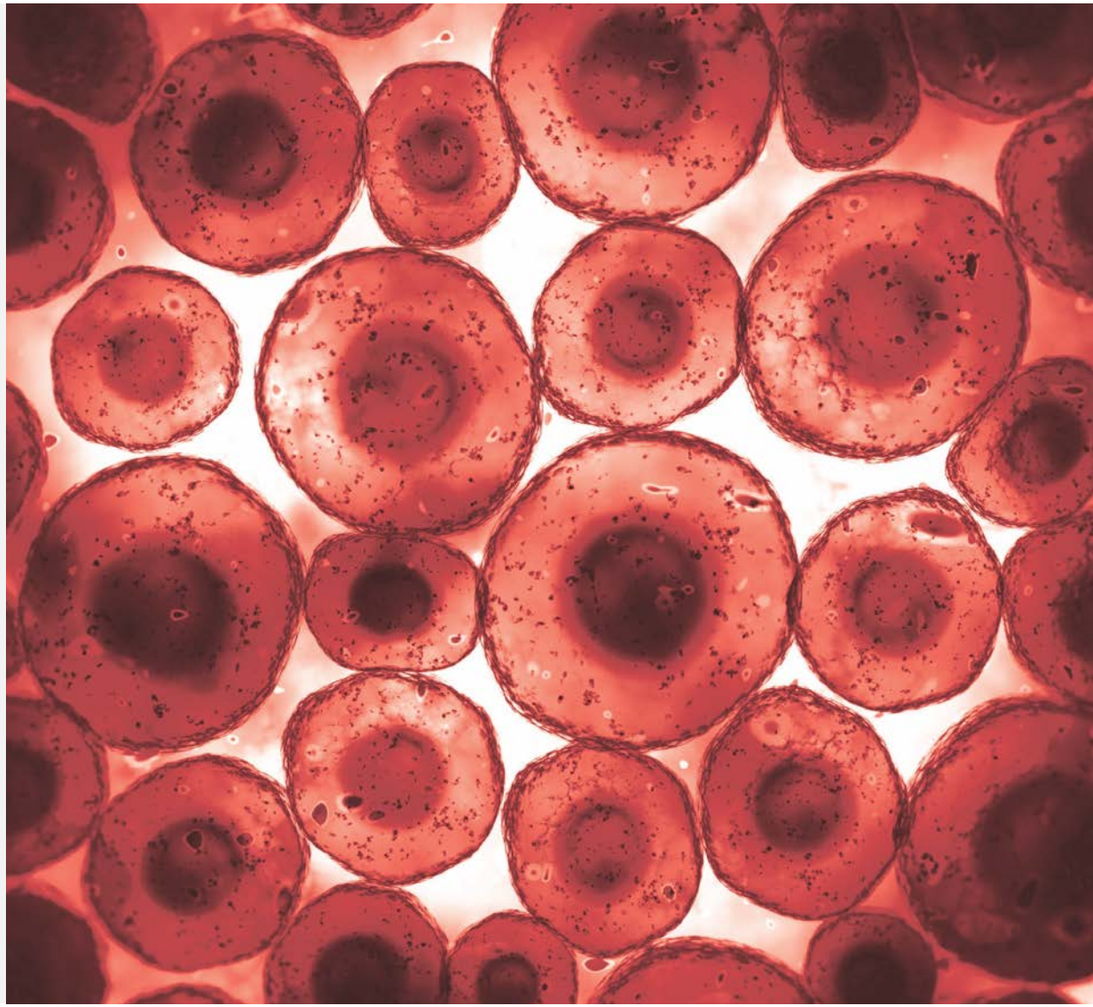
The third quarter of 2025 brought notable progress across the gene, cell, and RNA therapy landscape, marked by four new global approvals — one in each major therapeutic category. In gene therapy, China's NMPA approved Hrain Biotechnology's Hicara CAR-T therapy for large B-cell lymphoma, while in the US, the FDA approved Precigen's Papzimeos for recurrent respiratory papillomatosis. The FDA also granted approval for Ionis Pharmaceuticals' Dawnzera, an RNA therapy for hereditary angioedema. In Europe, ExCellThera's Zemcelpro, a non-genetically modified cell therapy for hematological malignancies, received authorization.

While regulatory progress continued, clinical development activity slowed, with 125 trials initiated across gene, cell, and RNA therapies. The global pipeline remains robust with more than 3,200 trials currently underway worldwide. Dealmaking activity increased in Q3, with 99 transactions representing a 9% rise from the previous quarter. This growth was supported by a rebound in start-up financing, totaling $230.9 million across 11 rounds. The quarter featured strategic takeovers by Kite and Biogen in areas of in vivo CAR therapies and ASO delivery, underscoring ongoing innovation and investment in next-generation modalities.

Pancreatic cancer joined the list of top five rare diseases targeted by CGTs. Additionally, the share of gene therapy trials focused on non-oncology indications rose to 42%, signaling a notable reversal of the downward trend observed since Q4 2024.

Across these indicators, the data tell a powerful story: progress in CGT is dynamic, but not linear. Each data point helps us better understand the pace and direction of the field, equipping the community to anticipate shifts and opportunities for impact. The data are critical to driving awareness, guiding research, and accelerating availability of cell and gene therapies for patients worldwide.

David Barrett, JD
JD CEO, ASGCT



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Key takeaways from Q3 2025

Four new approvals across each of the gene, cell, and RNA categories took place in Q3 2025

- Two gene therapy approvals: in China, the NMPA approved Hrain Biotechnology's CAR-T therapy, Hicara, for relapsed or refractory large B-cell lymphoma; while in the US, the FDA approved Precigen's Papzimeos, a gene therapy indicated for recurrent respiratory papillomatosis (RRP)
- One RNA therapy approval: the US FDA granted marketing approval for Ionis Pharmaceuticals' Dawnzera in hereditary angioedema
- One non-genetically modified cell therapy approval: ExCellThera's Zemcelpro was granted EU approval for the treatment of adults with hematological malignancies

Q3 sees the lowest number of total initiated clinical trials across gene, cell, and RNA therapies per quarter in over a year

- A total of 125 trials were initiated in Q3 2025 across each of the gene, cell, and RNA categories, 17% less vs. the previous quarter and a 13% downturn compared to the equivalent period in 2024 (Q3)
- There are a total of 3,243 trials investigating gene, cell, or RNA therapies that are currently ongoing

Dealmaking for advanced molecular therapy companies rebounded

- At 99 deals, total transaction volume for advanced molecular therapy companies represented a 9% increase over the previous quarter
- Acquisitions were 4x down in Q3 from Q2, but featured takeovers by Kite and Biogen in the areas of in vivo CAR therapies and ASO delivery, respectively
- Start-up financing rebounded from the low of last quarter to reach an aggregate $230.9 million from 11 rounds

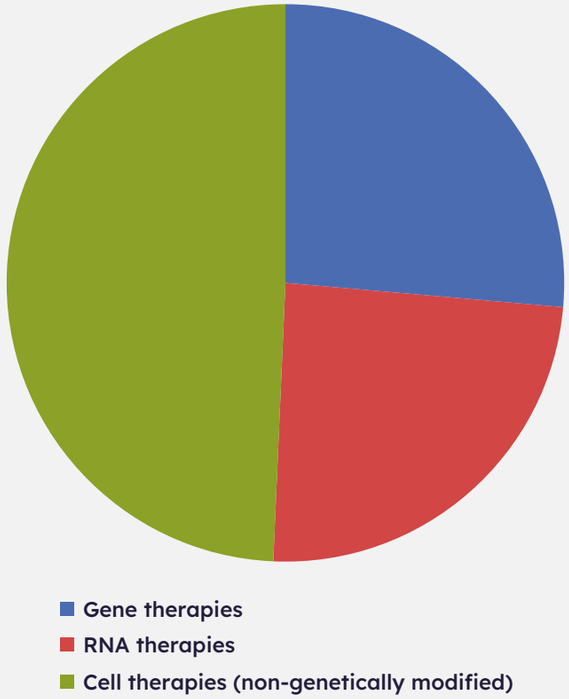
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Key highlights in Q3 2025

Approved gene, cell, and RNA therapies

Globally, for clinical use:

- **38 gene therapies have been approved** (including genetically modified cell therapies)
  - Hrain Biotechnology achieved NMPA approval in China for its CAR-T therapy, Hicara, indicated for relapsed or refractory large B-cell lymphoma; the FDA approved Precigen's Papzimeos, a gene therapy for recurrent RRP
- **35 RNA therapies have been approved**
  - Ionis Pharmaceuticals' Dawnzera was approved in the US for hereditary angioedema
- **71 non-genetically modified cell therapies have been approved**
  - ExCellThera's Zemcelpro was granted EU approval for the treatment of adults with hematological malignancies



Source: Pharmaprojects | Citeline, October 2025

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Approved gene therapies as of Q3 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	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	Novartis
Luxturna	voretigene neparvovec	2017	Leber’s congenital amaurosis; retinitis pigmentosa	US, EU, UK, Australia, Canada, South Korea, Japan	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, 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	bluebird bio
Tecartus	brexucabtagene autoleucel	2020	Mantle cell lymphoma; acute lymphocytic leukemia	US, EU, UK, Australia, Canada	Kite Pharma (Gilead)
Libmeldy	atidarsagene autotemcel	2020	Metachromatic leukodystrophy	EU, UK, Switzerland, US	Orchard Therapeutics
Breyanzi	lisocabtagene maraleucel	2021	Diffuse large B-cell lymphoma; follicular lymphoma; chronic lymphocytic leukemia; mantle cell lymphoma	US, Japan, EU, Switzerland, UK, Canada	Celgene (Bristol Myers Squibb)
Abecma	idecabtagene vicleucel	2021	Multiple myeloma	US, Canada, EU, UK, Japan, Israel, Switzerland	bluebird bio

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Approved gene therapies as of Q3 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
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	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	PTC Therapeutics
Roctavian	valoctocogene roxaparvovec	2022	Hemophilia A	EU, US	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, United Arab Emirates, Qatar, Kuwait, Bahrain, Oman, Israel, Japan	Sarepta Therapeutics
Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Vyjuvek	beremagene geperpavec	2023	Dystrophic epidermolysis bullosa	US, EU, Japan	Krystal Biotech
Fucaso	equecabtagene autoleucel	2023	Multiple myeloma	China, Hong Kong	Nanjing IASO Biotechnology
Casgevy	exagamglogene autotemcel	2023	Sickle cell anemia; thalassemia	US, UK, Bahrain, Saudi Arabia, EU, Canada, Switzerland; Qatar	CRISPR Therapeutics
inaticabtagene autoleucel	inaticabtagene autoleucel	2023	Acute lymphocytic leukemia	China	Juventas Cell Therapy

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Approved gene therapies as of Q3 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Lyfgenia	lovotibeglogene autotemcel	2023	Sickle cell anemia	US	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	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

Source: Pharmaprojects | Citeline, October 2025

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Approved RNA therapies as of Q3 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, 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	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
Libmeldy	atidarsagene autotemcel	2020	Metachromatic leukodystrophy	EU, UK, Switzerland, US	Orchard Therapeutics

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Approved RNA therapies as of Q3 2025 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Givlaari	givosiran	2020	Porphyria	US, EU, UK, Canada, Switzerland, Brazil, Israel, Japan, Australia	Alnylam
Oxlumo	lumasiran	2020	Hyperoxaluria	EU, UK, US, Brazil	Alnylam
Viltepso	viltolarsen	2020	Dystrophy, Duchenne muscular	US, Japan	NS Pharma
Leqvio	inclisiran	2020	Atherosclerosis; heterozygous familial hypercholesterolemia; hypercholesterolemia	EU, UK, Australia, Canada, Israel, US, Saudi Arabia, Japan, China	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
Amvuttra	vutrisiran	2022	Amyloidosis, transthyretin-related hereditary	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

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Approved RNA therapies as of Q3 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
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

*For COVID-19 vaccines, this includes emergency use authorization and full approvals.

Source: Pharmaprojects | Citeline, October2025

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Key highlights in Q3 2025 (Noteworthy events that happened in Q3 2025)

Drug	Event Type	Indication	Molecule	Event Date
CRD-003	Regulatory - Orphan Drug Designation (U.S.)	Limb-Girdle Muscular Dystrophy (LGMD)	Viral Gene Therapy	09/24/2025
SAR446268	Regulatory - Fast Track Status	Myotonic Muscular Dystrophy	Viral Gene Therapy	09/23/2025
MVX-220	Regulatory - Fast Track Status	Angelman Syndrome	Viral Gene Therapy	09/22/2025
Tryngolza	Regulatory - Approval (Europe)	Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	Antisense	09/19/2025
Izervay	Regulatory - Approval (Japan)	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Oligonucleotide	09/19/2025
MNV-201	Regulatory - Fast Track Status	Myelodysplastic Syndrome (MDS)	Cellular	09/18/2025
SAR402663	Regulatory - Fast Track Status	Wet Age-Related Macular Degeneration (Wet AMD) (Ophthalmology)	Viral Gene Therapy	09/11/2025
ION-581	Regulatory - Breakthrough Therapy Designation (U.S.)	Angelman Syndrome	Antisense	09/09/2025
RGX-121	Regulatory - Advanced Therapy Medicinal Product (ATMP) Classification	Mucopolysaccharidosis II (MPS II; Hunter Syndrome)	Viral Gene Therapy	09/05/2025
NS-051	Regulatory - Orphan Drug Designation (U.S.)	Duchenne Muscular Dystrophy (DMD)	Antisense	09/05/2025
sonpiretigene isteparvovec	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Stargardt Disease (Ophthalmology)	Viral Gene Therapy	09/02/2025
ETX101	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Dravet Syndrome (Epilepsy)	Viral Gene Therapy	09/02/2025
HB-adMSC	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Multiple Sclerosis (MS)	Cellular	08/27/2025
Adstiladrin	Regulatory - J-NDA Filing (Japan)	Bladder Cancer	Viral Gene Therapy	08/27/2025
VGT-1849B	Regulatory - Orphan Drug Designation (U.S.)	Polycythemia Vera (PV)	Antisense	08/26/2025
Zemcelpro	Regulatory - Conditional Marketing Authorisation (Europe)	Bone Marrow and Stem Cell Transplant - Graft vs. Host Disease (GVHD) Prophylaxis	Cellular	08/25/2025

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Key highlights in Q3 2025 (Noteworthy events that happened in Q3 2025) *continued*

Drug	Event Type	Indication	Molecule	Event Date
Dawnzera	Regulatory - Approval (U.S.)	Hereditary Angioedema (HAE)	Antisense	08/21/2025
VG801	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Stargardt Disease (Ophthalmology)	Viral Gene Therapy	08/21/2025
VG801	Regulatory - Orphan Drug Designation (U.S.)	Stargardt Disease (Ophthalmology)	Viral Gene Therapy	08/21/2025
RPA-501	Regulatory - Rare Pediatric Disease (RPD) Designation	Danon Disease	Viral Gene Therapy	08/20/2025
RPA-501	Regulatory - Advanced Therapy Medicinal Product (ATMP) Classification	Danon Disease	Viral Gene Therapy	08/20/2025
NouvNeu001	Regulatory - Fast Track Status	Parkinson's Disease (PD)	Cellular	08/15/2025
AlloStemSpine	Regulatory - Fast Track Status	Chronic Low Back Pain (CLBP)	Cellular	08/13/2025
GLPG5101	Regulatory - Regenerative Medicine Advanced Therapy (RMAT)	Mantle Cell Lymphoma - NHL	Cellular	08/06/2025
DYNE-251	Regulatory - Breakthrough Therapy Designation (U.S.)	Duchenne Muscular Dystrophy (DMD)	Antisense	08/04/2025
zimislecel	Regulatory - Innovative Licensing and Access Pathway (ILAP) (U.K.)	Diabetes Mellitus, Type I	Cellular	08/04/2025
nucresiran	Regulatory - Fast Track Status	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	siRNA/RNAi	07/31/2025
CLD-201	Regulatory - Fast Track Status	Sarcoma - Unspecified	Cellular	07/29/2025
Tabelecleucel	Regulatory - Priority Review	Hematologic Cancer	Cellular	07/24/2025
Vyjuvek	Regulatory - Approval (Japan)	Epidermolysis Bullosa	Viral Gene Therapy	07/24/2025
CAN-2409	Regulatory - Orphan Drug Designation (Europe)	Pancreatic Cancer	Viral Gene Therapy	07/24/2025
PBGENE-DMD	Regulatory - Orphan Drug Designation (U.S.)	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	07/23/2025
SGT-501	Regulatory - Fast Track Status	Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)	Viral Gene Therapy	07/23/2025

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Key highlights in Q3 2025 (Noteworthy events that happened in Q3 2025) *continued*

Drug	Event Type	Indication	Molecule	Event Date
vusolimogene oderparepvec	Regulatory - Complete Response Letter (CRL)	Melanoma	Viral Gene Therapy	07/22/2025
KRRO-110	Regulatory - Orphan Drug Designation (Europe)	Alpha-1 Antitrypsin Deficiency (A1AD or AATD)	Oligonucleotide	07/21/2025
Rese-cel	Regulatory - Orphan Drug Designation (Europe)	Systemic Lupus Erythematosus (SLE)	Cellular	07/18/2025
Aucatzyl	Regulatory - Approval (Europe)	Acute Lymphoblastic Leukemia (ALL)	Cellular	07/18/2025
RP-A601	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Cardiovascular Disease	Viral Gene Therapy	07/17/2025
GNSC-001	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Osteoarthritis	Viral Gene Therapy	07/16/2025
SAR446597	Regulatory - Fast Track Status	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Viral Gene Therapy	07/16/2025
stem cell therapy, HeXem Bio	Regulatory - Orphan Drug Designation (U.S.)	Bone Marrow Transplant and Stem Cell Transplant	Cellular	07/11/2025
Deramiocel	Regulatory - Complete Response Letter (CRL)	Duchenne Muscular Dystrophy (DMD)	Cellular	07/11/2025
UX111	Regulatory - Complete Response Letter (CRL)	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A Syndrome)	Viral Gene Therapy	07/11/2025
SGT-501	Regulatory - Rare Pediatric Disease (RPD) Designation	Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)	Viral Gene Therapy	07/08/2025
MB-101	Regulatory - Orphan Drug Designation (U.S.)	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	Cellular	07/07/2025
LX2006	Regulatory - Breakthrough Therapy Designation (U.S.)	Friedreich's Ataxia	Viral Gene Therapy	07/07/2025

Source: Biomedtracker | Evaluate, October 2025

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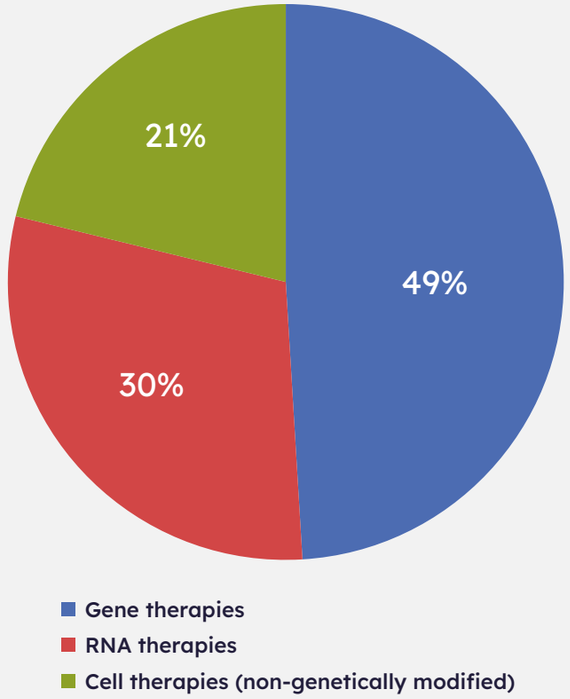
Pipeline overview

Pipeline of gene, cell, and RNA therapies

4,341 therapies are in development, ranging from preclinical through pre-registration

- 2,129 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
- 920 non-genetically modified cell therapies are in development, accounting for 21% of gene, cell, and RNA therapies

Pipeline therapies by category



Source: Pharmaprojects | Citeline, October 2025

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Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Gene therapy pipeline: quarterly comparison

- An increase in the number of gene therapy programs was seen at all stages of clinical development
- The number of gene therapies currently in pre-registration has dropped slightly from Q2 2025; notably, Rocket Pharmaceuticals decided to withdraw marketing applications in the US and EU for its Fanconi anemia gene therapy, RP-L102
- Therapies currently in pre-registration:

In the US

- RP-L201 (Rocket Pharmaceuticals)
- RGX-121 (Regenxbio)
- UX111 (Ultragenyx)
- vusolimogene oderparepvec (Replimune)
- OTL-103 (Fondazione Telethon/ Orchard Therapeutics)
- sonpiretigene isteparvove (Nanoscope Therapeutics)

In the EU

- OTL-103 (Fondazione Telethon/ Orchard Therapeutics)

In China

- satricabtagene autoleucel (CARsgen Therapeutics)
- donaperminogene seltoplasmid (Helixmith)
- pulkilumab (pCAR-19B) cells (Chongqing Precision Biotech)
- IM-19 (Imunopharm)

In South Korea

- Anbal-cel (Curocell)

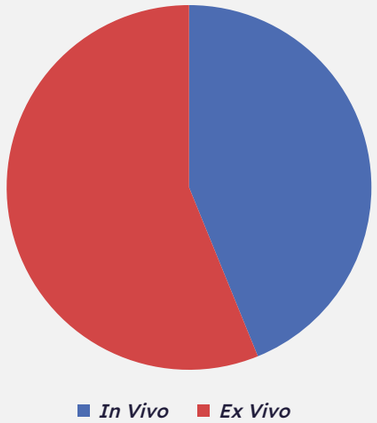
Global Status	Q3 2024	Q4 2024	Q1 2025	Q2 2025	Q3 2025
Preclinical	1,393	1,424	1,432	1,461	1,346
Phase I	318	341	350	361	377
Phase II	289	306	319	330	347
Phase III	35	35	41	45	47
Pre-registration	6	11	13	13	11
Total	**2,068**	**2,041**	**2,117**	**2,155**	**2,129**

Source: Pharmaprojects | Citeline, October 2025

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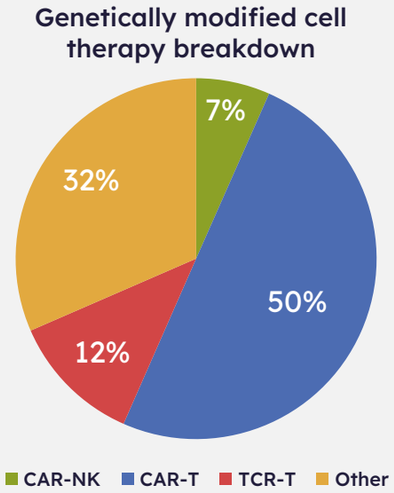
Genetic modification: *In vivo* vs. *Ex vivo*

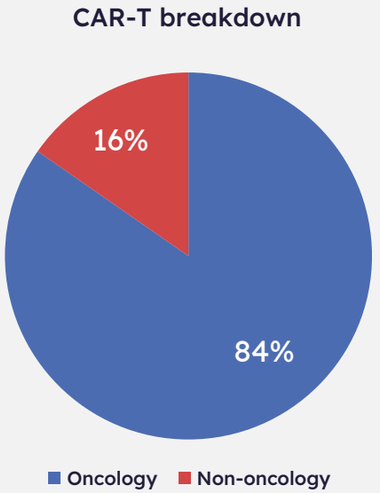
- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q3 2025, *In vivo* delivery techniques were used in 44% of gene therapies



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 50%, followed by the “other” category at 32%, which includes a list of less commonly used technologies such as TCR-NK, CAR-M, and TAC-T
- 84% 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, multiple sclerosis, and HIV/AIDS





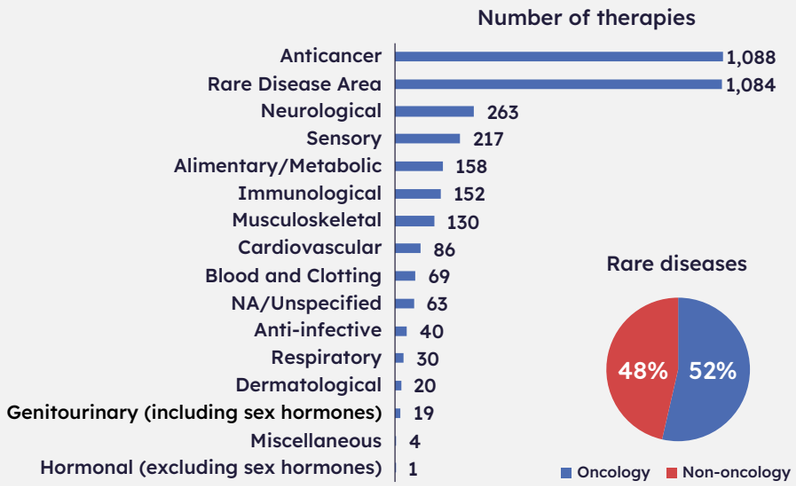
Source: Cell and Gene Therapy dashboard | Citeline, October 2025

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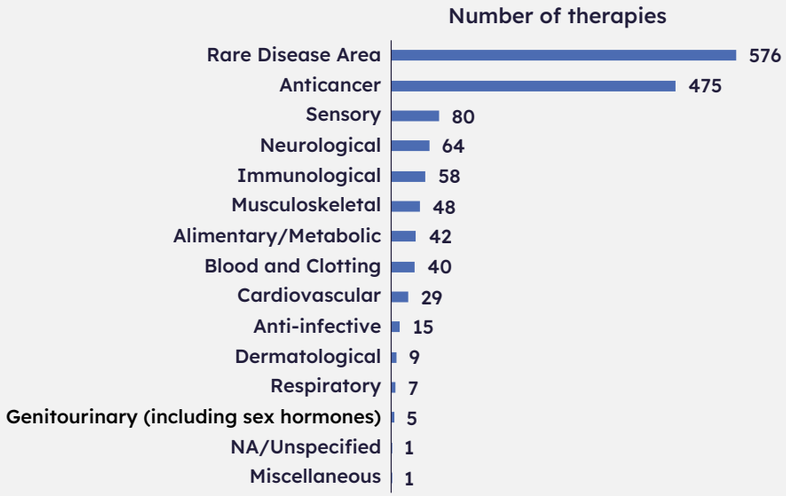
Gene therapy pipeline: most commonly targeted therapeutic areas

- 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 most commonly occurred in oncology, representing a majority of 52% compared to non-oncology rare disease gene therapy pipeline development, the same proportion as 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

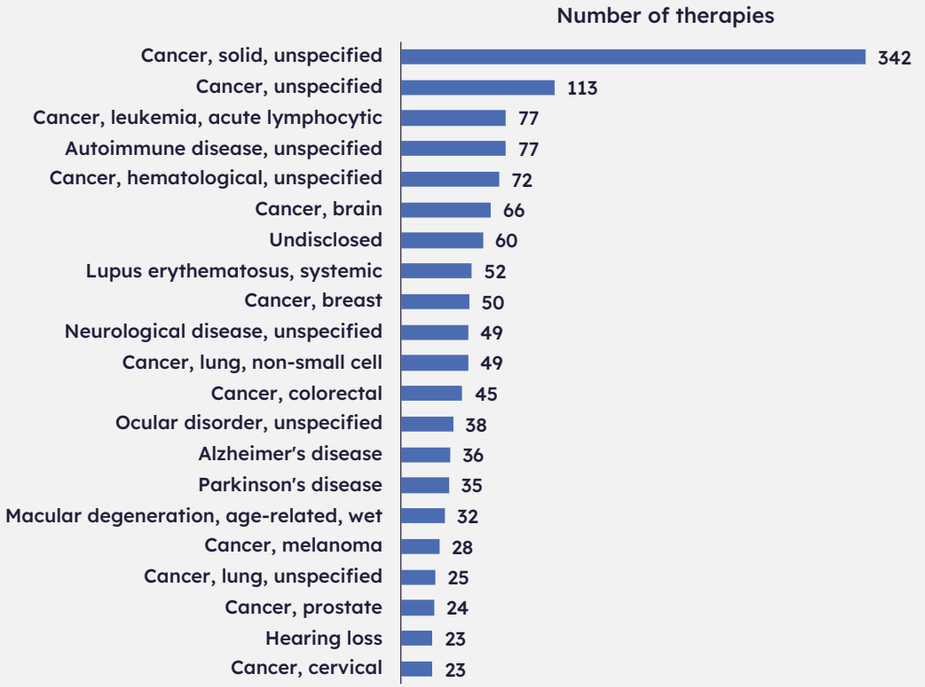
Source: Pharmaprojects | Citeline, October 2025

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Gene therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q3 2025 were:

- Acute lymphocytic leukemia
- Brain cancer
- Systemic lupus erythematosus



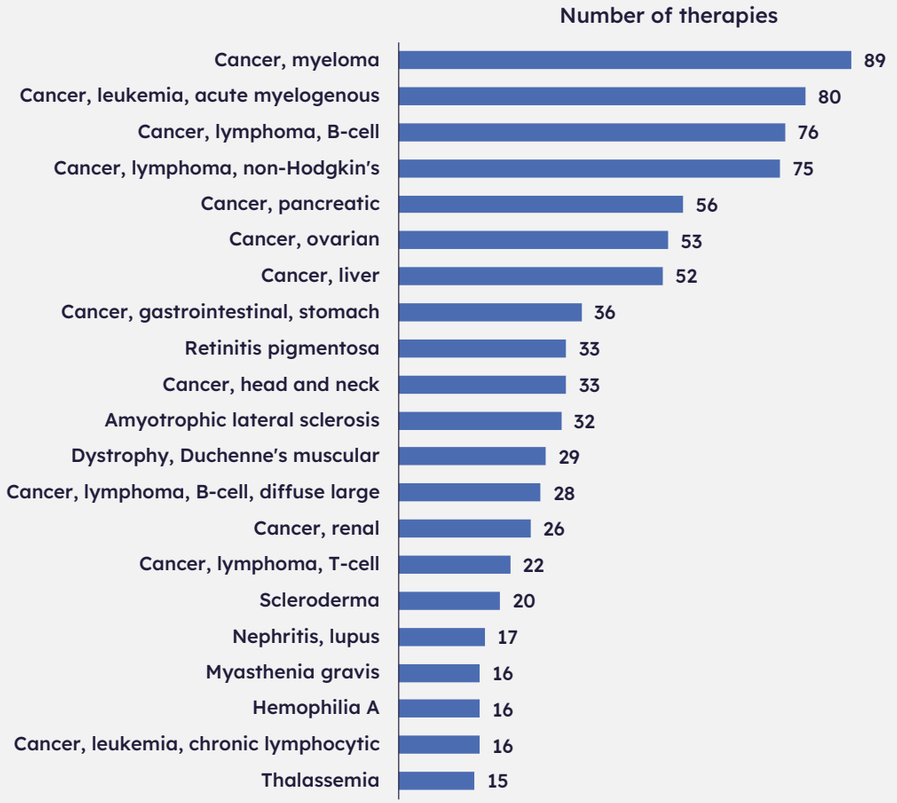
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, October 2025

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Gene therapy pipeline: most common rare diseases targeted

- For the 1,419 pipeline (preclinical to pre-registration) gene therapies being developed for rare diseases, nine out of the top 10 rare diseases were oncological, one more rare oncology indication than the previous quarter
- Pancreatic cancer marks a new addition to the top five rare diseases for which gene therapies are being developed:
  - Myeloma
  - Acute myelogenous leukemia
  - B-cell lymphoma
  - Non-Hodgkin’s lymphoma
  - Pancreatic cancer



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

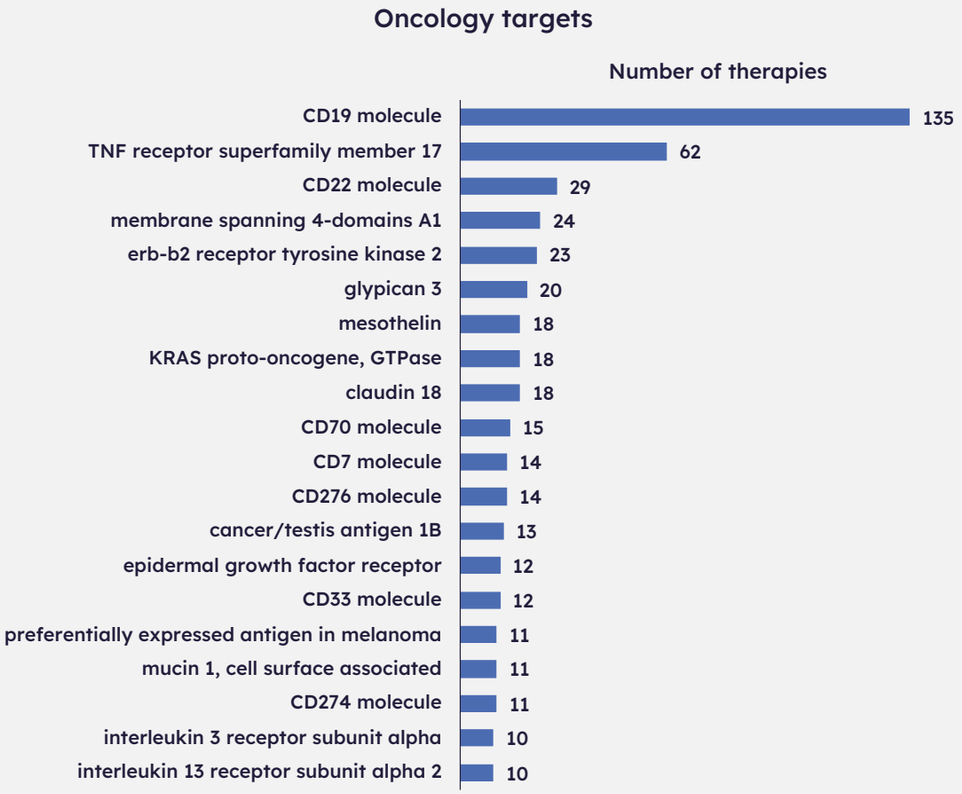
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Gene therapy pipeline: most common targets

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

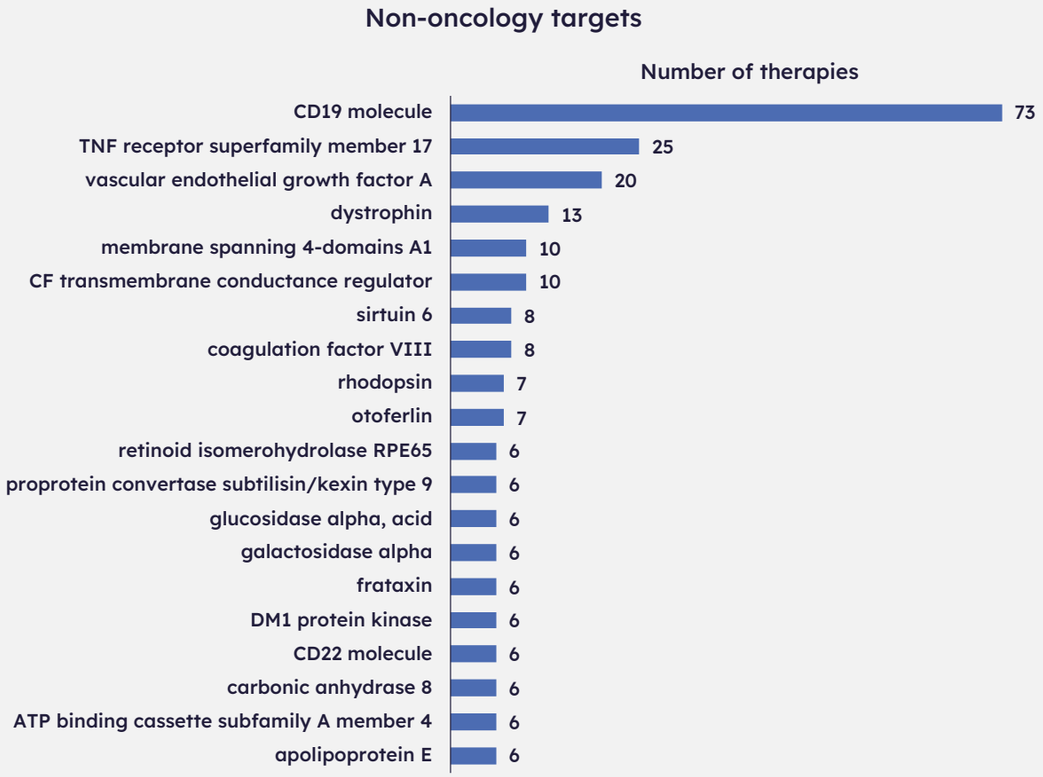
- CD19 molecule and B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17, remained the top two most common targets for oncology indications
- CD19 molecule, TNF receptor superfamily member 17, and vascular endothelial growth factor A continued to be the top three most common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



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Gene therapy pipeline: most common targets *continued*

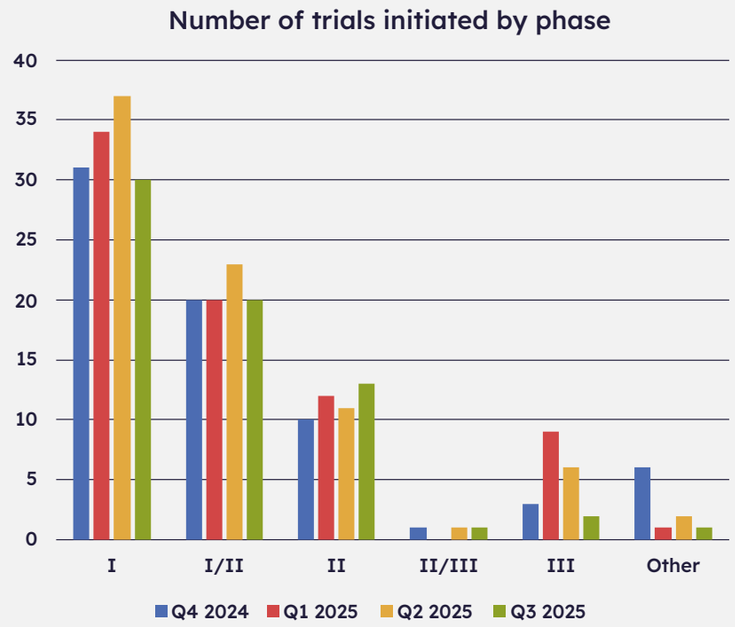


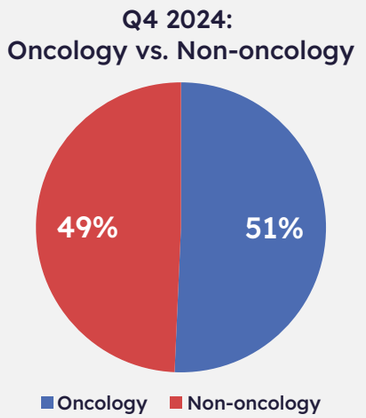
Source: Pharmaprojects | Citeline, October 2025

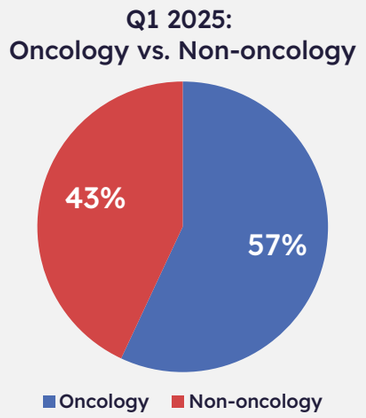
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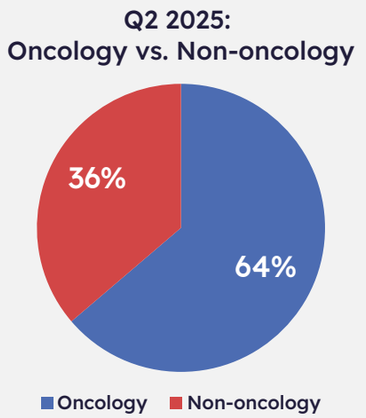
Gene therapy clinical trial activity

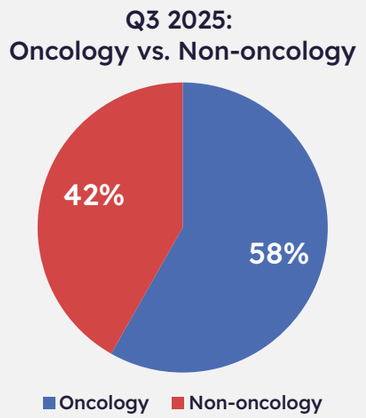
- The proportion of gene therapy trials for non-oncology indications increased to 42%, marking a reversal of previous declining trends for non-oncology trials since Q4 2024
- 67 gene therapy trials were initiated in Q3 2025, 13 less than the previous quarter
- There are currently 1,877 open gene therapy clinical trials











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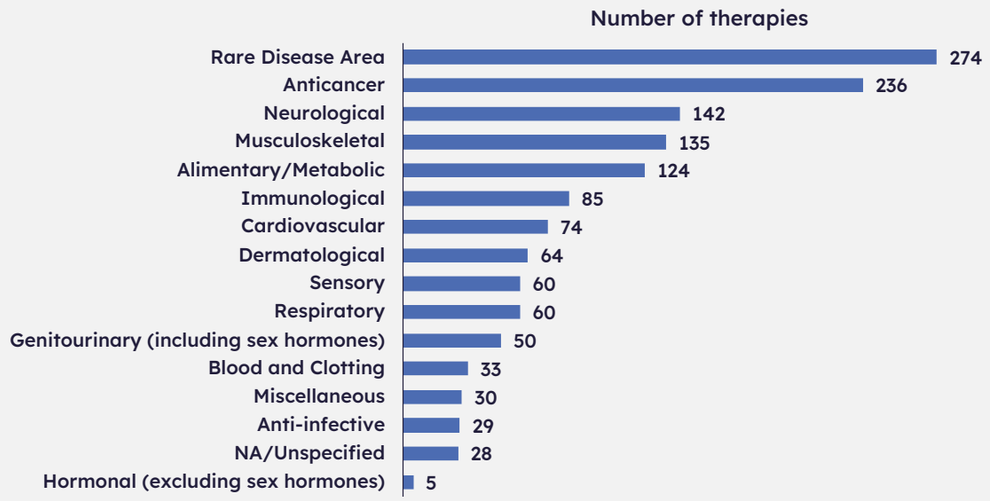
Non-genetically modified cell therapy pipeline

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, 63% 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

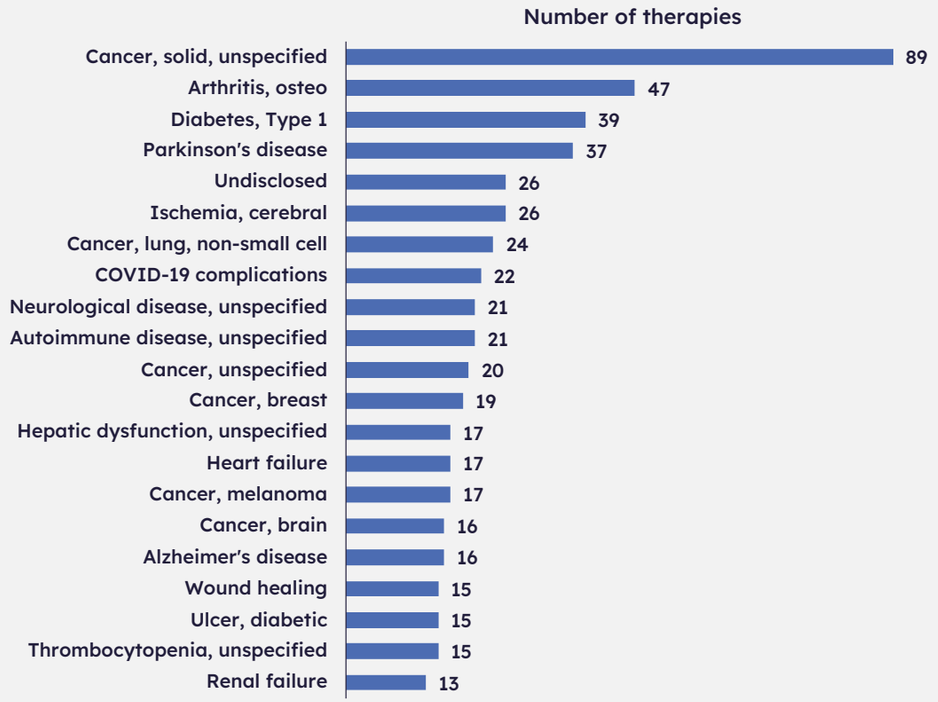
Source: Pharmaprojects | Citeline, October 2025

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

Of the therapies for which indications are specified, the most targeted indications in Q3 2025 were:

- Osteoarthritis
- Type 1 diabetes
- Parkinson’s disease



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

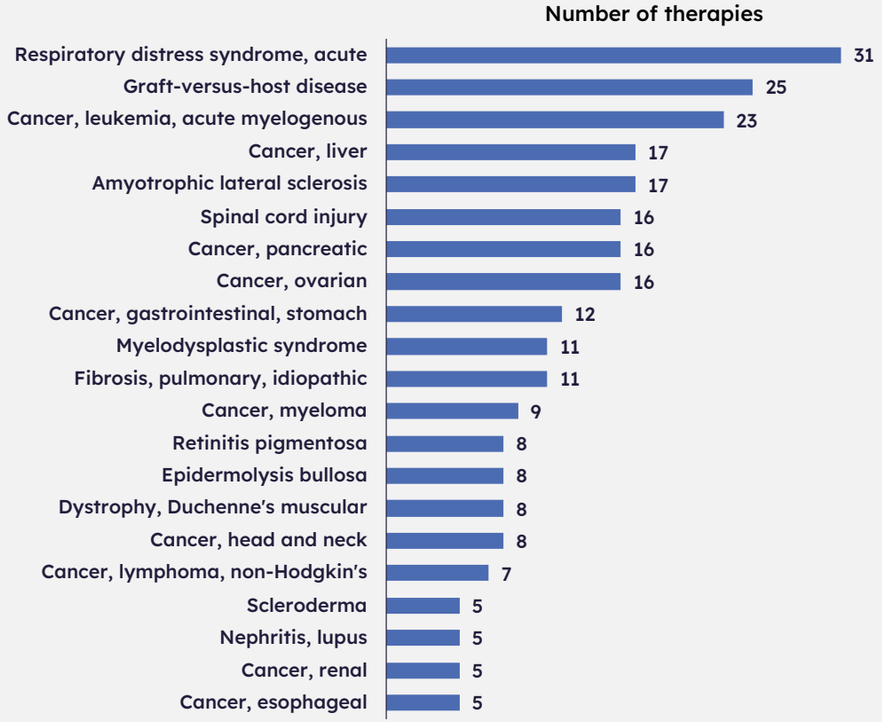
Source: Pharmaprojects | Citeline, October 2025

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



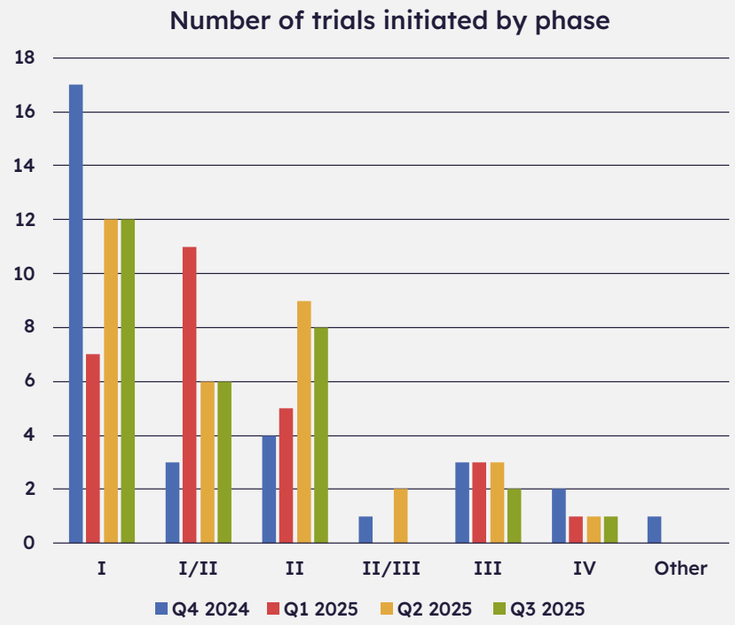
Note: Figures based on indications in pipeline development only for each therapy

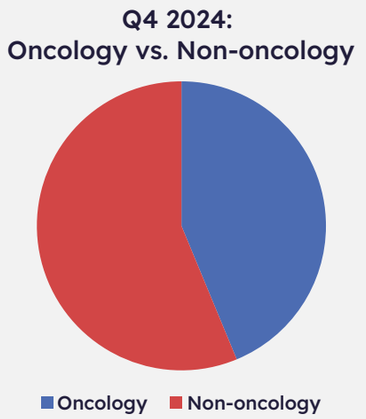
Source: Pharmaprojects | Citeline, October 2025

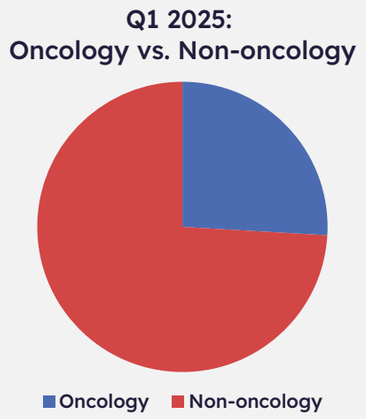
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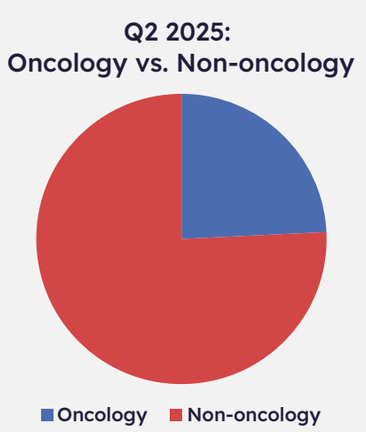
Non-genetically modified cell therapy trial activity

- 29 trials were initiated for non-genetically modified cell therapies in Q3 2025, four less than in Q2 2025
- Of these 29, 62% were for non-oncology indications, 14 percentage points lower than the previous quarter
- There are currently 886 open non-genetically modified cell therapy clinical trials











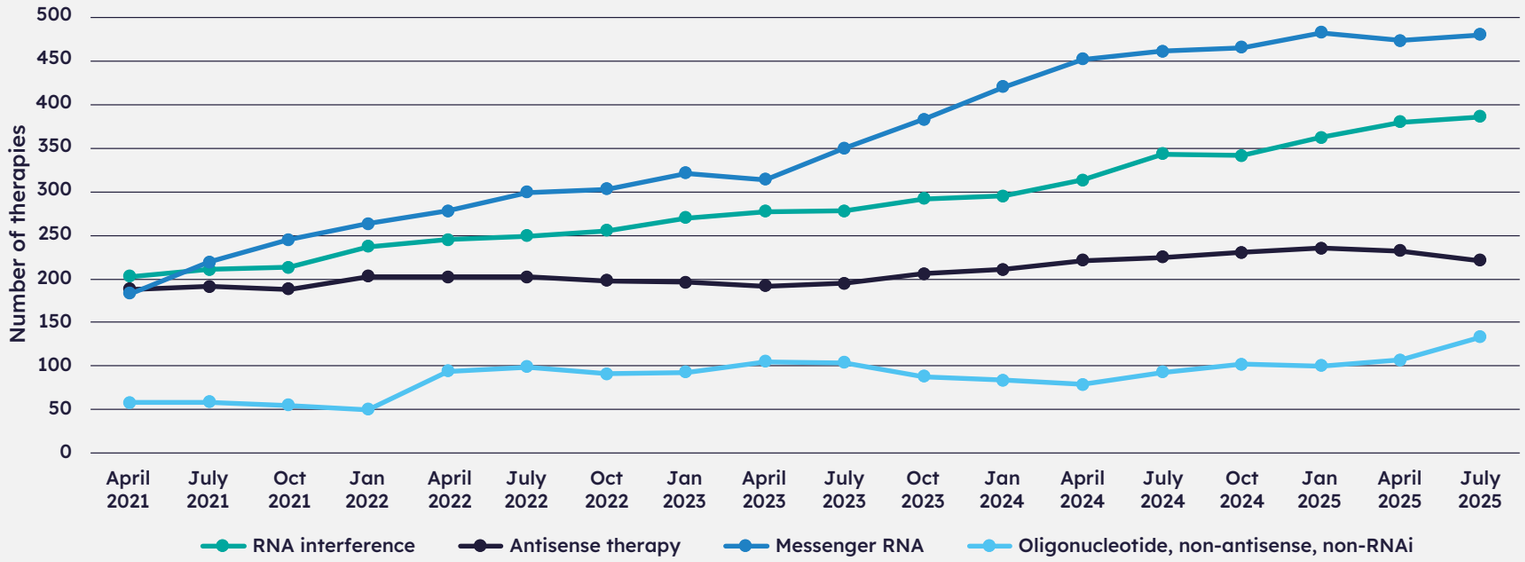
Source: Pharmaprojects | Citeline, October 2025

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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

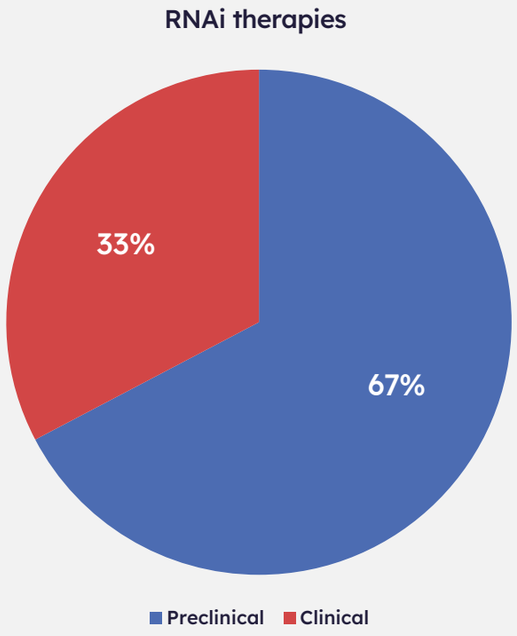


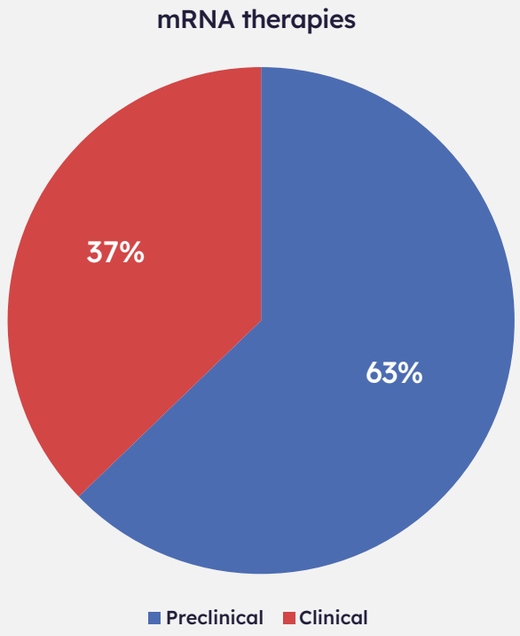
Source: Pharmaprojects | Citeline, October 2025

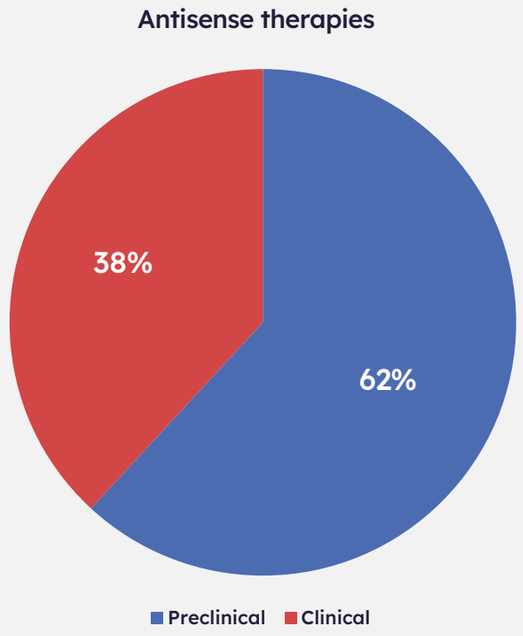
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RNAi, mRNA, and antisense oligonucleotides: preclinical vs. clinical

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 67%, 63%, and 62% of their respective pipelines







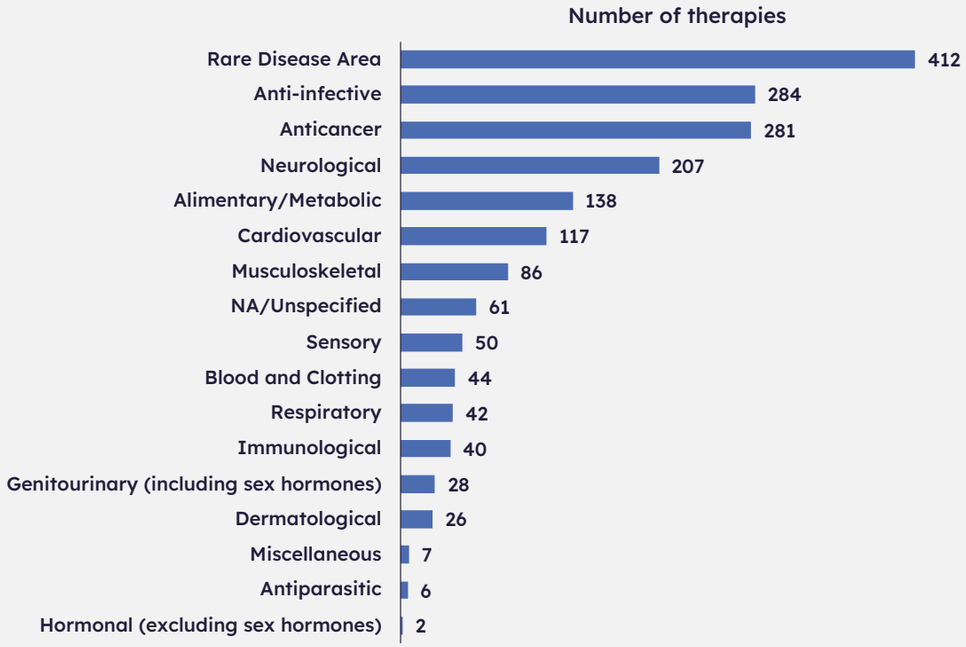
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RNA therapies: most commonly targeted therapeutic areas

Of the 1,297 RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Rare diseases remained the top targeted therapeutic area by RNA therapies, while anticancer indications climbed to the second most commonly targeted
- Non-oncology indications continued to be the most targeted rare diseases by RNA therapies, representing a majority of 75%





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

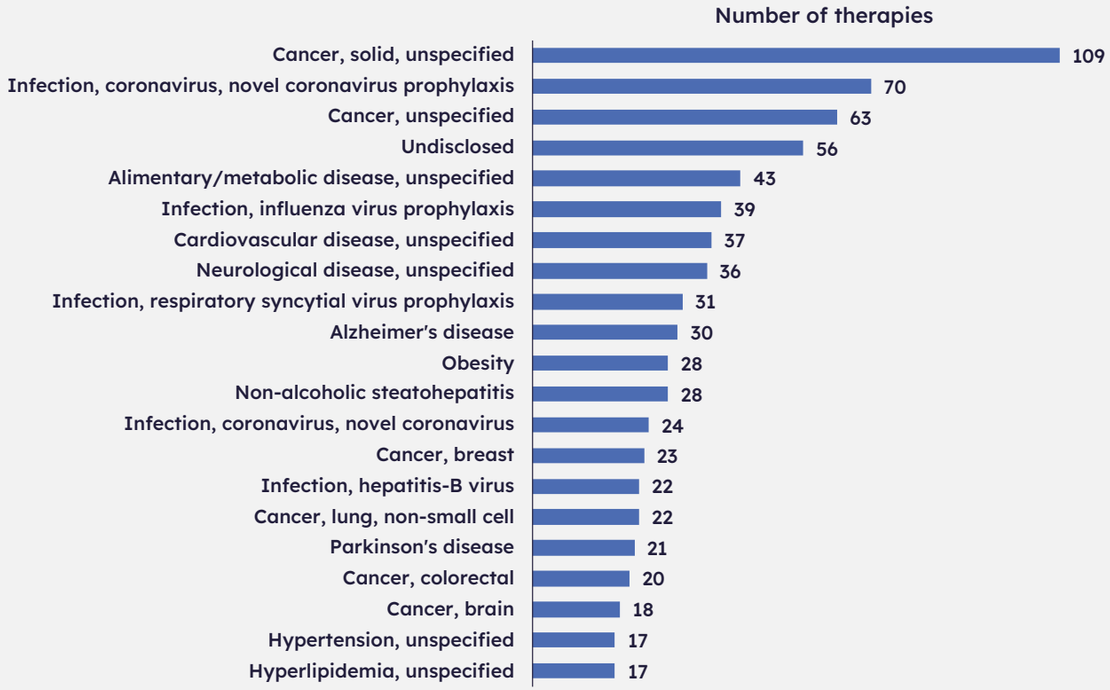
Source: Pharmaprojects | Citeline, October 2025

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RNA therapies: most common rare diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q3 2025 were:

- Coronavirus prophylaxis
- Influenza prophylaxis
- Respiratory syncytial virus prophylaxis



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

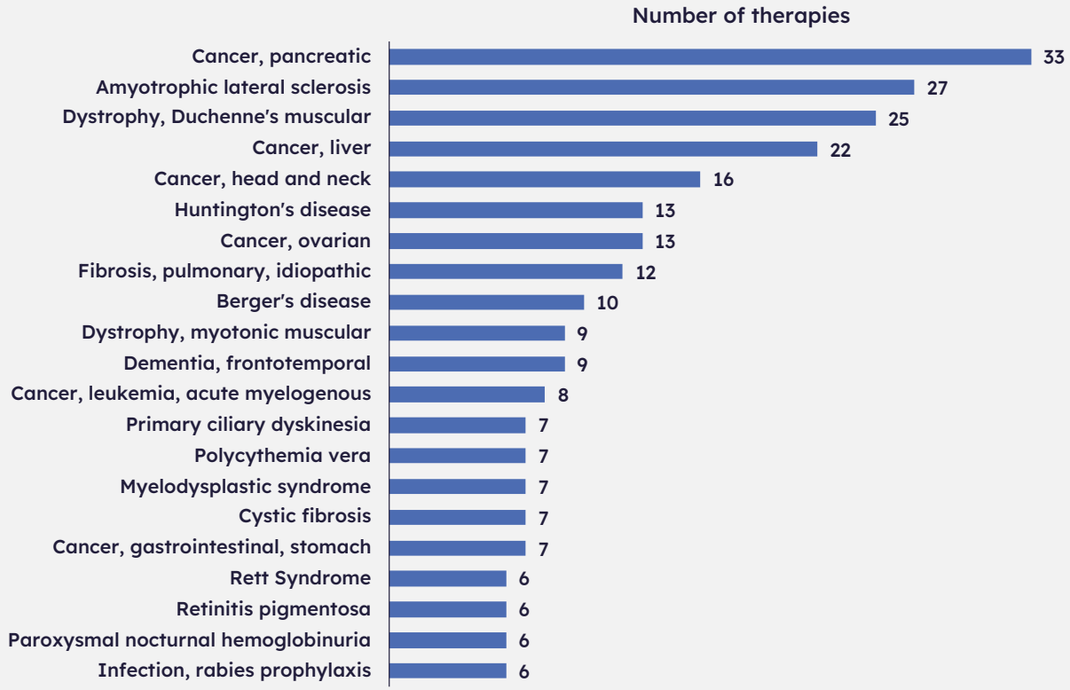
Source: Pharmaprojects | Citeline, October 2025

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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 Huntington's disease were the most targeted indications



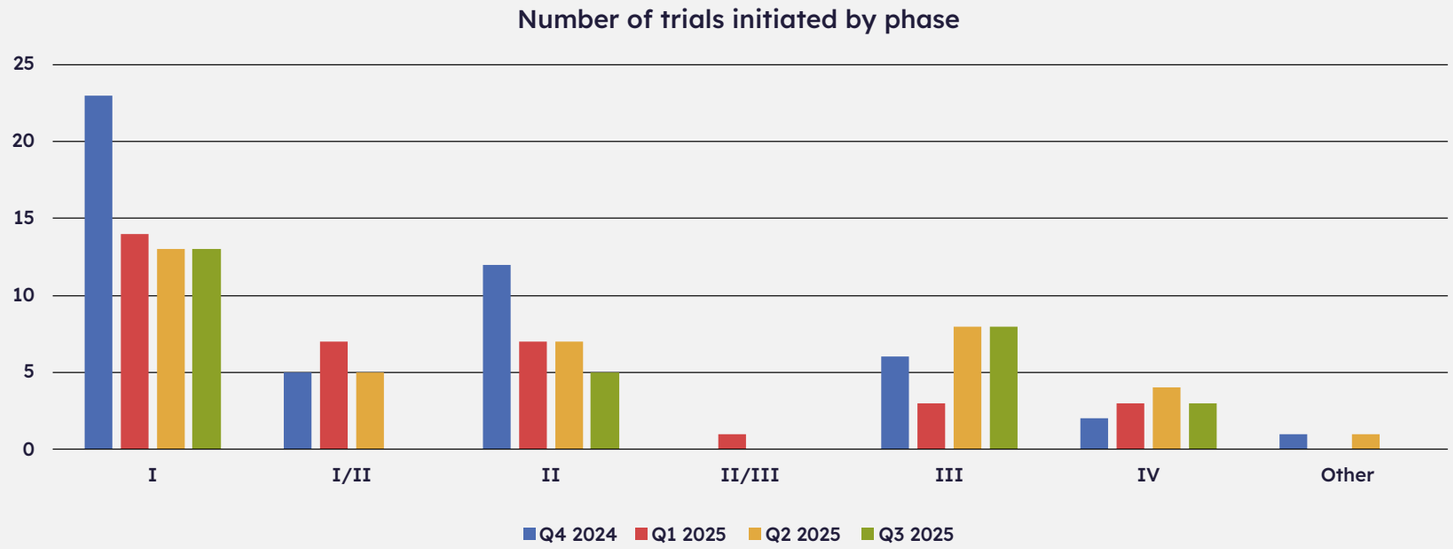
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, October 2025

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RNA therapy pipeline: clinical trial activity

- 29 RNA trials were initiated in Q3 2025, compared to 38 in Q2 2025, 90% of which were for non-oncology indications
- There are currently 480 open RNA therapy clinical trials



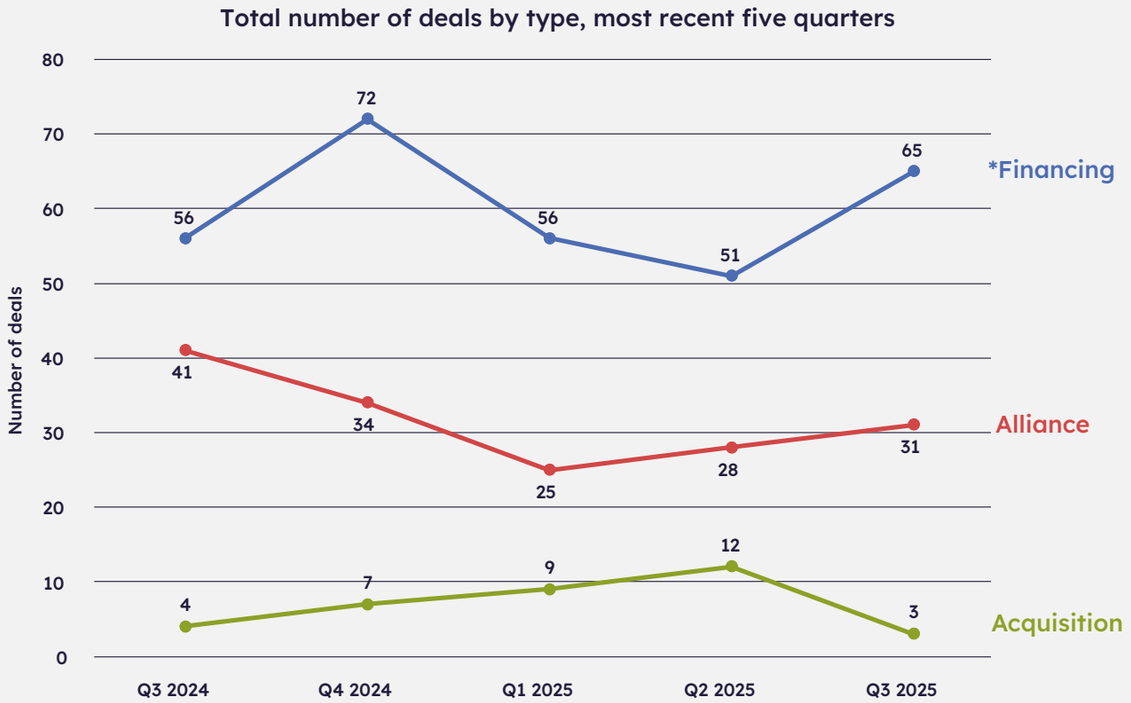
Source: Trialtrove | Citeline, October 2025

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

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

- Advanced molecular therapy companies signed 91 deals in Q2 2025, virtually flat from the previous quarter's 90 deals
- Gains seen in acquisition and alliance activity vs. Q1 2025 were offset by a 9% decrease in financing volume, lowering from 56 to 51 transactions
- Q2 2025's total was 9% behind the 100 deals signed in the second quarter of 2024



*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, October 2025

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Q3 2024 acquisitions in gene, cell, and RNA therapy

- Q3 2025 featured 3 acquisitions, a 4x decrease from Q2 and a quarterly low within the last year
- Two top biotech companies purchased novel assets: for $350 million, Kite acquired Interius BioTherapeutics, focusing on *in vivo* CAR therapeutics; and Biogen paid $85 million plus milestones for Alcyone Therapeutics, with which it has been developing ThecaFlex DRx, an implantable subcutaneous port and catheter device that intrathecally delivers antisense oligonucleotides

Deal date	Deal title	Potential deal value (USD $)
08 July 2025	Concentra Biosciences to Acquire CARGO Therapeutics; Acquisition Completed	Undisclosed
21 August 2025	Kite to Acquire Interius BioTherapeutics	350,000,000
18 September 2025	Biogen to Acquire Alcyone Therapeutics for its ThecaFlex System and Divest Rest of Pipeline as a New Company, Neela Therapeutics	85,000,000

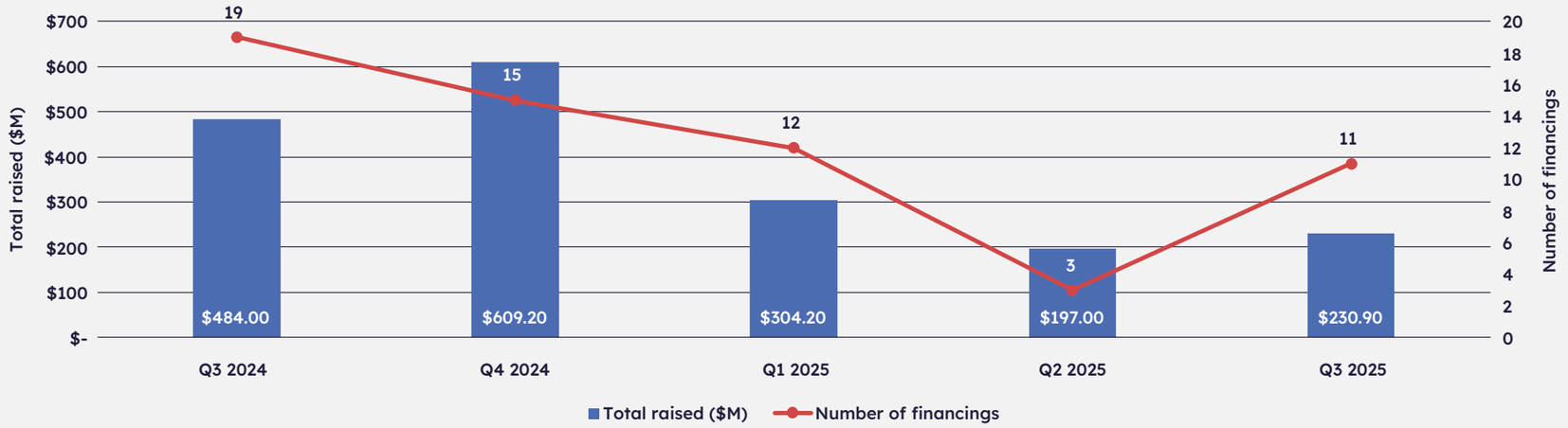
Sources: Biomedtracker, BioSciDB | Evaluate, October 2025

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

- 11 start-up advanced molecular therapy companies raised a total of $230.9 million in seed and Series A funding in Q3 2025
- The volume of start-ups in Q3 2025 represents a rebound from the three seen in the previous quarter, but is still relatively low compared with other quarters in the last year
- APAC-based companies accounted for almost half of the start-up financings in Q3 2025, led by AccurEdit Therapeutics' $75 million Series A round

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



Source: Biomedtracker | Evaluate, October 2025

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Q2 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)
25 August 2024	Arnatar Therapeutics Raises $52M in Series A Round*	siRNA therapy	United States/California/ San Diego	n/a - founded by former Ionis Pharmaceuticals scientists	52
07 July 2025	A-Seeds Closes $15M Series A Round	CAR-T therapy	Japan/Nagano	Shinshu University	15
23 July 2025	Dispatch Bio Completes Series A Round	CAR-T therapy	United States/ Pennsylvania/ Philadelphia	Memorial Sloan Kettering Cancer Center; University of Pennsylvania; Stanford University; UCSF	Undisclosed
19 August 2025	Axelyf Closes Seed Financing to Advance Next-Generation RNA-LNP Therapeutics	LNP delivery technology	United States/ Massachusetts/ Boston	n/a - founded by former Moderna scientist and assets acquired from 76Bio	3
27 August 2025	Leal Therapeutics Raises $30M in Series A Financing	Antisense oligonucleotides	United States/ Massachusetts/ Worcester	n/a - founded by former CEO of Prevail Therapeutics	30
08 September 2025	bYoRNA Raises €1.5M in Pre-Seed Round	mRNA manufacturing	France/Bordeaux	Charité Berlin	2
08 September 2025	Integra Therapeutics Raises €10.7M in Pre-Series A Round	Gene writing tools and CAR-T therapy	Spain/Barcelona	Pompeu Fabra University	13
29 August 2025	Shenzhen MagicRNA Biotech Completes Pre-Series A+ Round	In vivo CAR-T delivered via LNPs	China/Shenzhen	n/a - founded by Zha Gaofeng	14
12 September 2025	StarryGene Raises Series A2 Financing Worth Tens of Millions CNY	Gene therapy	China/Hefei	Chinese Academy of Sciences' Center for Brain and Intelligence Excellence	Undisclosed
13 September 2025	AccurEdit Therapeutics Raises $75M Series A Financing	Gene editing	China/Suzhou	Undisclosed; has JV with Stanford University spin-off	75
13 September 2025	Rui Therapeutics Closes Series A Round	CAR natural killer cell therapy	China/Nanjing City	n/a - co-founded by returned overseas scientists and executives of multinational pharmaceutical companies	28

*Arnatar's financing is included since it was announced in August 2025 when the company emerged from stealth mode; Arnatar closed the Series A in March 2024

Source: Biomedtracker | Evaluate, October 2025

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Notable Q2 2025 start-up gene, cell, and RNA therapy companies

Company details	Academic source	Financing type/ amount raised	Lead investor(s)	Therapy areas of interest
In vivo gene editing using LNP delivery	Undisclosed; has JV with Stanford University	$75M/Series A	Tibet Rhodiola Pharmaceutical Holding; Shenzhen Kangzhe Pharmaceutical; China Medical System	ATTR amyloidosis and heterozygous familial hypercholesterolemia
DARGER (Dual-functional Antisense RNA for Gene Expression Regulation) platform to produce siRNA and upregulating antisense oligonucleotides	n/a - founded by former Ionis Pharmaceuticals scientists	$52M/Series A*	Eight Roads; 3E Bioventures	Cardiometabolic, hepatic, neurological, and renal
Antisense oligonucleotide LXT-002 targeting SPTLC1 to inhibit ceramide/sphingolipid synthesis in ALS	n/a - founded by former CEO of Prevail Therapeutics	$30M/Series A	V Health Investors' Dementia Discovery Fund	Neurological

*Arnatar's financing is included since it was announced in August 2025 when the company emerged from stealth mode; Arnatar closed the Series A in March 2024

Source: Biomedtracker | Evaluate, October 2025

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Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q3 2025.

Therapy	Generic name	Disease	Catalyst	Catalyst date
plozasiran	plozasiran	Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	PDUFA for NDA - First Review	18 November 2025 - 18 November 2025
Zemcelpro	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	29 August 2025 - 30 November 2025
Breyanzi	lisocabtagene maraleucel	Marginal Zone Lymphoma - NHL	PDUFA for sBLA - First Review	05 December 2025 - 05 December 2025
Omisirge		Aplastic Anemia	Approval Decision (U.S)	10 December 2025 - 10 December 2025
Kresladi	marnetegragene autotemcel	Autoimmune Disorders	PDUFA Decision	01 January 2025 - 31 December 2025
MT1621	doxecitine, doxribtimine	Metabolic - General	PDUFA for NDA - First Review	24 September 2025 - 31 December 2025
Beqvez	fidanacogene elaparvovec	Hemophilia B	Approval Decision (Japan)	12 September 2025 - 31 December 2025
VN-0102	measles, mumps & rubella vaccine	Measles, Mumps and Rubella (MMR) Vaccines	Approval Decision (Japan)	11 September 2025 - 31 December 2025
DSP-1083		Parkinson's Disease (PD)	Approval Decision (Japan)	01 April 2025 - 31 March 2026
MT1621	doxecitine, doxribtimine	Metabolic - General	CHMP Opinion	01 September 2025 - 31 March 2026
Dawnzera	donidalorsen	Hereditary Angioedema (HAE)	CHMP Opinion	01 October 2025 - 30 April 2026
OTL-103	etuvetidigene autotemcel	Wiskott-Aldrich Syndrome	CHMP Opinion	01 November 2025 - 31 May 2026
MT1621	doxecitine, doxribtimine	Metabolic - General	Approval Decision (Europe)	01 November 2025 - 31 May 2026

Source: Biomedtracker | Evaluate, October 2025

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Appendix

Methodology, sources, and glossary of key terms

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

Pipeline and trial data

- Data derived from Pharmaprojects and Trialtrove
- 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. 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

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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 therapy in this report)	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 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

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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

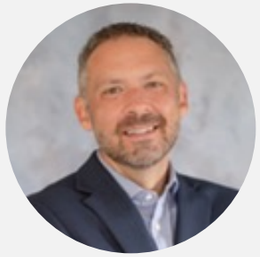
Deal type categories	
Alliances	Co-marketing, co-promotion, disease management, joint venture, manufacturing or supply, marketing-licensing, product or technology swap, product purchase, R&D and marketing-licensing, reverse licensing, trial collaborations
Financing	Convertible debt, FOPO, IPO, nonconvertible 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

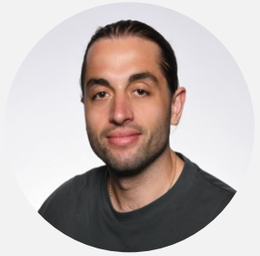
Unspecified indications	
Cancer, unspecified	Indications for which the specific tumor type is not specified
Cancer, hematological, unspecified	Indications for which the specific hematological cancer is not specified
Cancer, solid, unspecified	Indications for which the specific solid tumor is not specified

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Report Contributors



David Barrett, JD
CEO
American Society of Gene + Cell Therapy



Daniel Di Gaudio, MSc
Senior Associate, Consulting & Analytics
Citeline



Sarah Kikkert
Director of Communications
American Society of Gene + Cell Therapy



Amanda Micklus, MSc
Senior Manager, Consulting & Analytics
Citeline

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American Society of Gene + Cell Therapy

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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.

Contact: David Barrett, JD at info@asgct.org

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