Gene, Cell, & RNA Therapy Landscape Report

Q1 2025 Quarterly Data Report

About the authors

The American Society of Gene & Cell Therapy (ASGCT) is the primary professional membership organization for scientists, physicians, patient advocates, and other professionals with interest in gene and cell therapy.

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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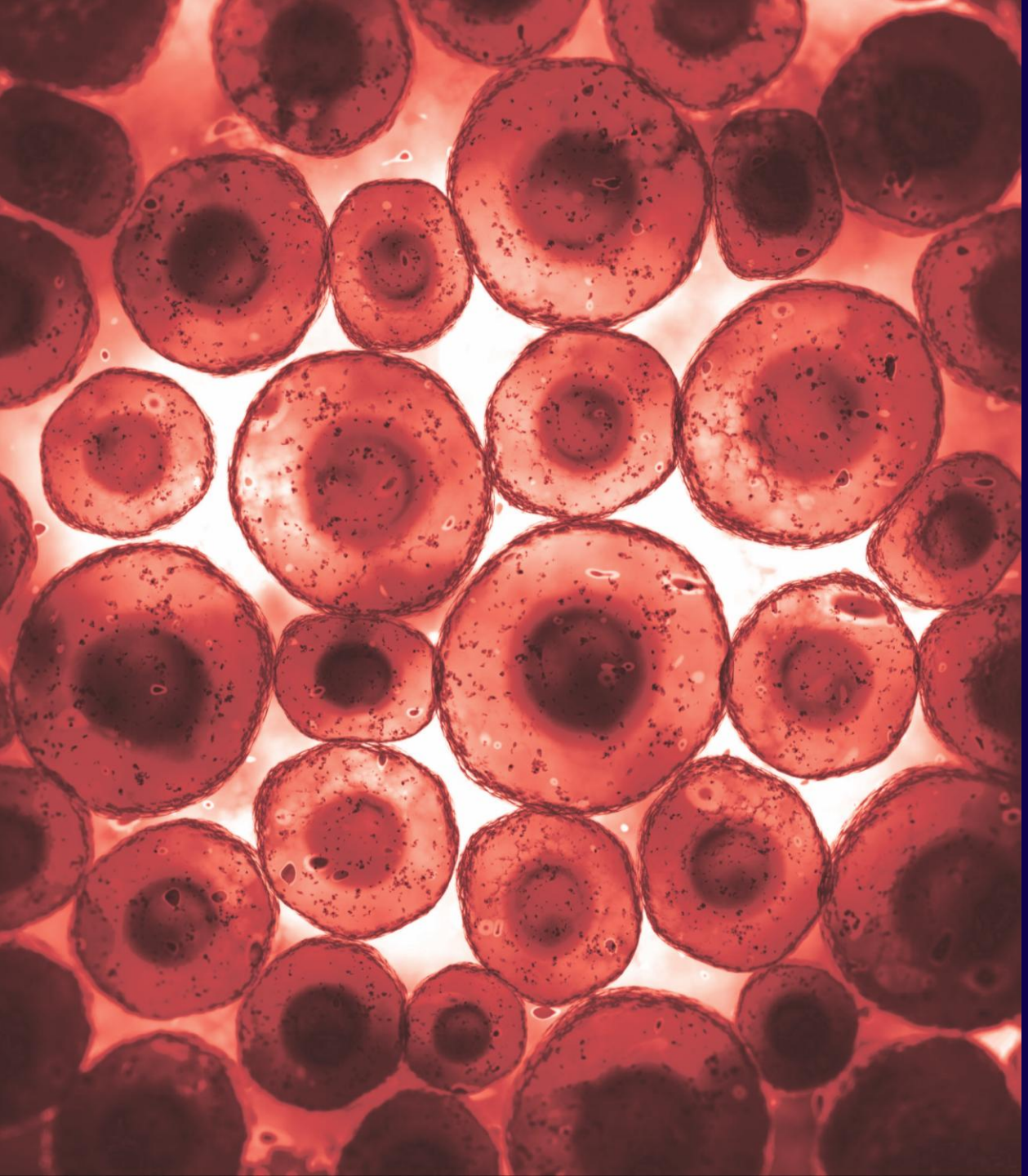

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Introduction

Welcome to the first quarterly report of 2025! During the past quarter, one new therapy was approved for each of the gene, cell, and RNA categories. In the U.S., the cell-based gene therapy Encelto became the first and only approved treatment for the degenerative eye disease macular telangiectasia type 2. An siRNA therapy, Qfitlia, was approved for hemophilia A or B. In China, the country's first mesenchymal stem cell therapy, Ruiboshen, was approved for steroid-refractory acute graft-versus-host disease.

During this past quarter, an increase continued in the number of non-genetically modified cell therapy trials initiated for non-oncology indications. Of the 27 trials initiated, 74 % were for non-oncology indications — 16% higher than the previous quarter. In the gene therapy pipeline, there were 79 trials initiated in Q1, of which 57% were for oncology indications — the highest oncology trial proportion for the past year. Among RNA therapies in the pipeline, mRNA and RNAi continued to be preferred research modalities.

Companies in Q1 announced 90 deals in financing, alliances, and acquisitions, a 20% decrease from 113 in the previous quarter. While alliances and financings were down, acquisitions saw a slight uptick from seven to nine. A total of 12 start-ups raised $304.3 million in Q1 seed and Series A funding, representing a 20% decrease in volume and 50% decrease in value compared with the previous quarter.

David Barrett, JD
CEO, ASGCT

Key takeaways from Q1 2025

One new approved advanced therapy for each of the gene, cell, and RNA categories occurred in Q1 2025

- In the US: Neurotech's Encelto, an intraocular implant of encapsulated engineered human cells, was approved for the treatment of macular telangiectasia type 2 (MacTel); Alnylam's siRNA therapy, Qfitlia was approved for the treatment of hemophilia A or B
- China's NMPA approves the country's first mesenchymal stem cell therapy, Platinum Life's Ruibosheng, indicated for steroid-refractory acute graft-versus-host disease

Non-genetically modified cell therapies continue a trend of increasing clinical trial proportions for non-oncology indications, while gene therapy trials lean to oncology

- 74% of non-genetically modified cell therapy trials in Q1 2025 were initiated for non-oncology indications, compared to 58% in Q4 2024 and 46% in Q3 2024; of pipeline therapies targeted at rare diseases, 62% were in development for non-oncology indications
- 79 gene therapy clinical trials were initiated in Q1 2025 and of those, 57% were for oncology indications — the highest oncology trial proportion for the past year

Dealmaking totals declined in Q1 2025 for advanced molecular therapy companies

- Q1's 90 deals represented a 20% decrease from the previous quarter due to lower financing and alliance activity, and were also 28% behind the 125 deals signed in 2024's opening quarter
- Acquisitions were slightly up in Q1 and featured one billion-dollar deal: AstraZeneca is paying $425 million up front and up to $575 million in earn-outs for in vivo cell therapy company EsoBiotec
- Series A and seed financings were down in volume and value in Q1, totaling $304.3 million from 12 rounds

Key highlights in Q1 2025

Q1 2025

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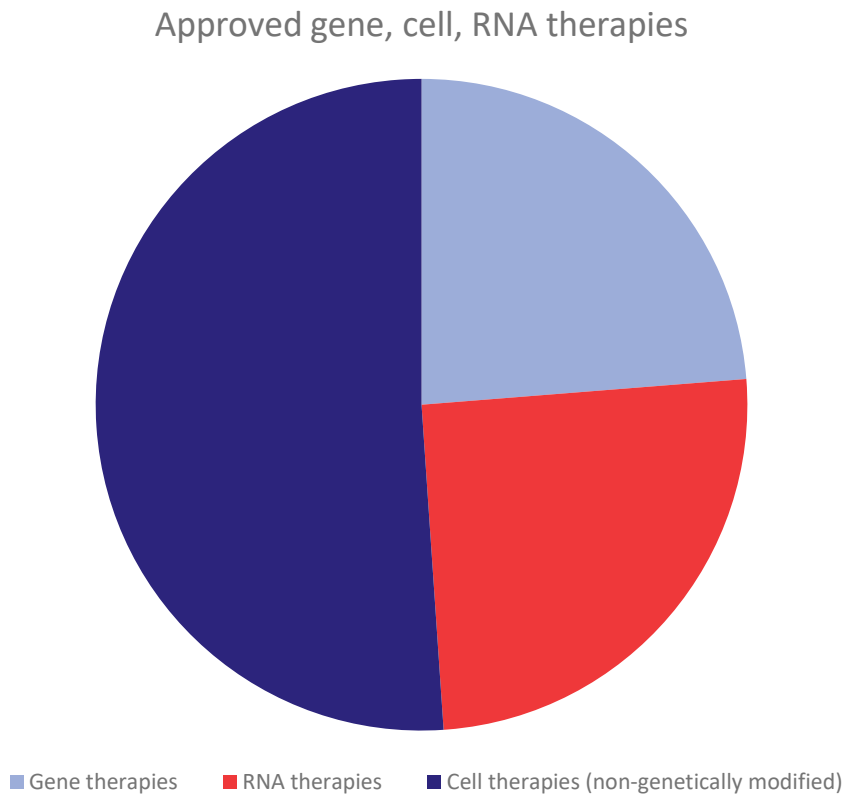
Approved gene, cell, and RNA therapies

Globally, for clinical use:

- 33 gene therapies have been approved (including genetically modified cell therapies)
  - ENCELTO, Neurotech's intraocular implant of encapsulated engineered human cells, was approved in the US for the treatment of macular telangiectasia type 2 (MacTel)
- 35 RNA therapies have been approved
  - Alnylam's siRNA therapy, QFITLIA was approved in the US for the treatment of hemophilia A or B
- 71 non-genetically modified cell therapies have been approved
  - Platinum Life's RUIBOSHENG, a human umbilical cord-derived mesenchymal stem cell (hUC-MSC) therapy, was approved in China for steroid-refractory acute graft-versus-host disease



Source: Pharmaprojects | Citeline, April 2025

Approved gene therapies as of Q1 2025 (1/3)

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	Kite Pharma (Gilead)
Zolgensma	onasemnogene abeparvovec	2019	Spinal muscular atrophy	US, EU, UK, Japan, Australia, Canada, Brazil, Israel, Taiwan, South Korea	Novartis
Zynteglo	betibeglogene autotemcel	2019	Transfusion-dependent beta thalassemia	US	bluebird bio

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

Approved gene therapies as of Q1 2025 (2/3)

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
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
Delytact	teserpaturev	2021	Malignant glioma	Japan	Daiichi Sankyo
Relma-cel	relmacabtagene autoleucel	2021	Diffuse large B-cell lymphoma; follicular lymphoma; mantle cell lymphoma	China	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	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	Sarepta Therapeutics
Vyjuvek	beremagene geperpavec	2023	Dystrophic epidermolysis bullosa	US	Krystal Biotech
Fucaso	equecabtagene autoleucel	2023	Multiple myeloma	China, Hong Kong	Nanjing IASO Biotechnology

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

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Approved gene therapies as of Q1 2025 (3/3)

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Casgevy	exagamglogene autotemcel	2023	Sickle cell anemia; thalassemia	US, UK, Bahrain, Saudi Arabia, EU, Canada, Switzerland	CRISPR Therapeutics
inaticabtagene autoleucel	inaticabtagene autoleucel	2023	Acute lymphocytic leukemia	China	Juventas Cell Therapy
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	Autolus
Encelto	revakinagene taroretcel	2025	Macular telangiectasia type 2 (MacTel)	US	Neurotech

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

Approved RNA therapies as of Q1 2025 (1/3)

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

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

Approved RNA therapies as of Q1 2025 (2/3)

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	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
Pfizer & BioNTech's Omicron BA.4/BA.5-adapted bivalent booster vaccine	Omicron BA.4/BA.5-adapted bivalent booster vaccine	2022	Infection, coronavirus, novel coronavirus prophylaxis	US, UK	BioNTech
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	Archemix

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

Approved RNA therapies as of Q1 2025 (3/3)

Product name	Generic name	Year first approved	Disease(s)	Locations approved*	Originator company
Arexvy	respiratory syncytial virus vaccine, GSK	2023	Respiratory syncytial virus prophylaxis	US, EU, Japan, UK, South Korea, Singapore, Canada, Australia	GSK
Qalsody	tofersen	2023	Amyotrophic lateral sclerosis	US, EU, Japan, China, Canada	Ionis Pharmaceuticals
ARCT-154	COVID-19 mRNA vaccine, Arcturus	2023	Infection, coronavirus, novel coronavirus prophylaxis	Japan, EU	Arcturus Therapeutics
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	Moderna Therapeutics
Tryngolza	olezarsen	2024	Lipoprotein lipase deficiency	US	Ionis Pharmaceuticals
Qfitlia	fitusiran	2025	Hemophilia A & B	US	Alnylam

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

Source: Pharmaprojects | Citeline, April 2025

Text highlighted in yellow represents new approvals during Q1 2025

Key highlights in Q1 2025 (1/2)

Noteworthy events that happened in Q1 2025

Drug	Event Type	Indication	Molecule	Event Date
CORDStrom	Orphan Drug Designation (U.S.)	Epidermolysis Bullosa	Cellular	01/06/2025
Restem-L	Fast Track Status	Polymyositis	Cellular	01/07/2025
Restem-L	Fast Track Status	Dermatomyositis	Cellular	01/07/2025
VG901	Rare Pediatric Disease (RPD) Designation	Retinitis Pigmentosa (RP) (Ophthalmology)	Viral Gene Therapy	01/08/2025
LOAd703	Fast Track Status	Pancreatic Cancer	Viral Gene Therapy	01/10/2025
CB-012	Orphan Drug Designation (U.S.)	Acute Myelogenous Leukemia (AML)	Cellular	01/12/2025
Rese-cel	Fast Track Status	Multiple Sclerosis (MS)	Cellular	01/13/2025
Tabelecleucel	Complete Response Letter (CRL)	Hematologic Cancer	Cellular	01/16/2025
VP-001	Rare Pediatric Disease (RPD) Designation	Retinitis Pigmentosa (RP) (Ophthalmology)	Antisense	01/20/2025
DYNE-101	Fast Track Status	Myotonic Muscular Dystrophy	Antisense	01/21/2025
SGT-212	Fast Track Status	Friedreich's Ataxia	Viral Gene Therapy	01/21/2025
NS-051	Rare Pediatric Disease (RPD) Designation	Duchenne Muscular Dystrophy (DMD)	Antisense	01/23/2025
Elevidys	Orphan Drug Designation (U.S.)	Adult T-cell Leukemia/Lymphoma (ATL)	Cellular	01/28/2025
Elevidys	Regenerative Medicine Advanced Therapy (RMAT) Designation	Retinitis Pigmentosa (RP) (Ophthalmology)	Viral Gene Therapy	01/28/2025
MB-105 (March Bio)	Innovative Licensing and Access Pathway (ILAP) (U.K.)	Eclampsia/Pre-Eclampsia	Other Nucleic Acid	01/30/2025
laruparetigene zovaparvovec	Orphan Drug Designation (U.S.)	Hyperoxaluria	Cellular	02/02/2025
CBP-4888	Orphan Drug Designation (U.S.)	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	siRNA/RNAi	02/04/2025
ABO-101 (Arbor)	J-NDA Filing (Japan)	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Oligonucleotide	02/05/2025
nucresiran	Fast Track Status	Systemic Lupus Erythematosus (SLE)	Cellular	02/05/2025
Izervay	Rare Pediatric Disease (RPD) Designation	Hyperoxaluria	Cellular	02/05/2025
ADI-001	Regenerative Medicine Advanced Therapy (RMAT) Designation	Ovarian Cancer	Cellular	02/05/2025
ABO-101 (Arbor)	Regenerative Medicine Advanced Therapy (RMAT) Designation	Amyloid light-chain (AL) Amyloidosis	Cellular	02/10/2025
Vigil EWS	Orphan Drug Designation (U.S.)	Cystic Fibrosis (CF)	Cellular	02/10/2025
NXC-201	Fast Track Status	Alzheimer's Disease (AD)	Cellular	01/06/2025
CF-PT101	Approval for sNDA/sBLA	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Cellular	01/07/2025

Source: Biomedtracker | Citeline, April 2025

Key highlights in Q1 2025 (2/2)

Noteworthy events that happened in Q1 2025

Drug	Event Type	Indication	Molecule	Event Date
Troculeucel	Fast Track Status	Alzheimer's Disease (AD)	Cellular	02/12/2025
EXG34217	Regenerative Medicine Advanced Therapy (RMAT) Designation	Undisclosed	Cellular	02/13/2025
EN-374	Orphan Drug Designation (U.S.)	Chronic Granulomatous Disease	Cellular	02/13/2025
ETX101	Rare Pediatric Disease (RPD) Designation	Dravet Syndrome (Epilepsy)	Viral Gene Therapy	02/13/2025
RZ001	Fast Track Status	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	Viral Gene Therapy	02/14/2025
Tegsedi	Withdrawal from Market	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	Antisense	02/14/2025
RPESC-RPE-4W	Regenerative Medicine Advanced Therapy (RMAT) Designation	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Cellular	02/18/2025
BS01	Regenerative Medicine Advanced Therapy (RMAT) Designation	Retinitis Pigmentosa (RP) (Ophthalmology)	Viral Gene Therapy	02/18/2025
PTP-001	Regenerative Medicine Advanced Therapy (RMAT) Designation	Osteoarthritis	Cellular	02/19/2025
AB-1005	Regenerative Medicine Advanced Therapy (RMAT) Designation	Parkinson's Disease (PD)	Viral Gene Therapy	02/19/2025
BRTX-100	Fast Track Status	Disc and Spine Repair	Cellular	02/20/2025
SkinTE	Breakthrough Therapy Designation (U.S.)	Diabetic Foot and Other Ulcers	Cellular	02/20/2025
IN-013	Rare Pediatric Disease (RPD) Designation	Wilson's Disease	mRNA (messenger RNA)	02/21/2025
EN001	Orphan Drug Designation (U.S.)	Charcot-Marie-Tooth Disease	Cellular	02/27/2025
OCU410	Advanced Therapy Medicinal Product (ATMP) Classification	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Viral Gene Therapy	03/03/2025
OCU410ST	Advanced Therapy Medicinal Product (ATMP) Classification	Stargardt Disease (Ophthalmology)	Viral Gene Therapy	03/03/2025
NEU-001	Orphan Drug Designation (U.S.)	Undisclosed	Cellular	03/03/2025
NEU-001	Rare Pediatric Disease (RPD) Designation	Undisclosed	Cellular	03/04/2025
Encelto	Approval (U.S.)	Macular Telangiectasia	Cellular	03/05/2025
Wainua	Approval (Europe)	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	Antisense	03/06/2025
Rytelo	Approval (Europe)	Myelodysplastic Syndrome (MDS)	Oligonucleotide	03/07/2025
ATSN-201	Fast Track Status	X-Linked Retinoschisis	Viral Gene Therapy	03/12/2025
KRRO-110	Orphan Drug Designation (U.S.)	Alpha-1 Antitrypsin Deficiency (A1AD or AATD)	Oligonucleotide	03/12/2025
Nex-z	Regenerative Medicine Advanced Therapy (RMAT) Designation	Transthyretin Amyloid Cardiomyopathy (ATTR-CM, Wild Type Or Hereditary)	Cellular	03/21/2025
Fitusiran	Approval (U.S.)	Hemophilia A and B - General Clotting Products	Non-Viral Gene Therapy	03/25/2025

Source: Biomedtracker | Citeline, April 2025

Pipeline overview

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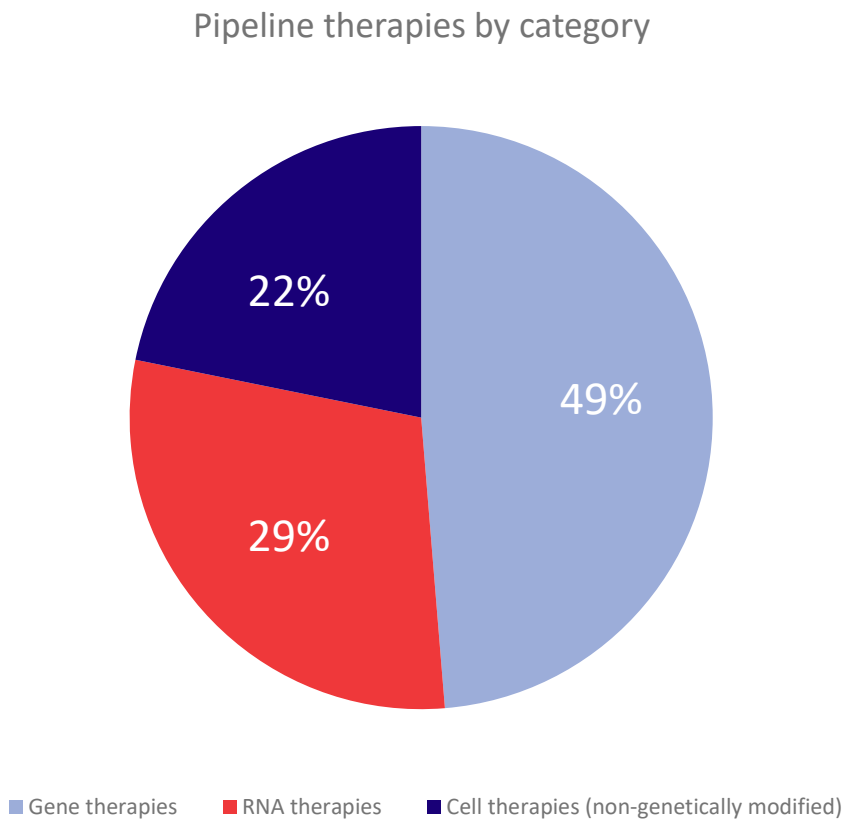
Pipeline of gene, cell, and RNA therapies

4,418 therapies are in development, ranging from preclinical through pre-registration

- 2,154 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
- 966 non-genetically modified cell therapies are in development, accounting for 22% of gene, cell, and RNA therapies



Source: Pharmaprojects | Citeline, April 2025

Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Q1 2025

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Gene therapy pipeline: quarterly comparison

- An increase in the number of gene therapy programs was seen at all stages of pipeline development
- There are two more gene therapies currently in pre-registration compared to Q4 2024; the total number has more than doubled since Q3 2024
- Therapies currently in pre-registration:
  - In the US
    - RP-L201 (Rocket Pharmaceuticals)
    - RGX-121 (Regenxbio)
    - SEL-212 (3SBio)
    - Pz-cel (Abeona)
    - UX111 (Ultragenyx)
    - vusolimogene oderparepvec (Replimune)
    - PRGN-2012 (Precigen)
  - In the EU
    - RP-L102 (Rocket Pharmaceuticals)
  - In China
    - BBM-H901 (Belief BioMed)*
    - donaperminogene seltoplasmid (Helixmith)
    - pulkilumab (pCAR-19B) cells (Chongqing Precision Biotech)
    - IM-19 (Imunopharm)a
  - In South Korea
    - Anbal-cel (Curocell)

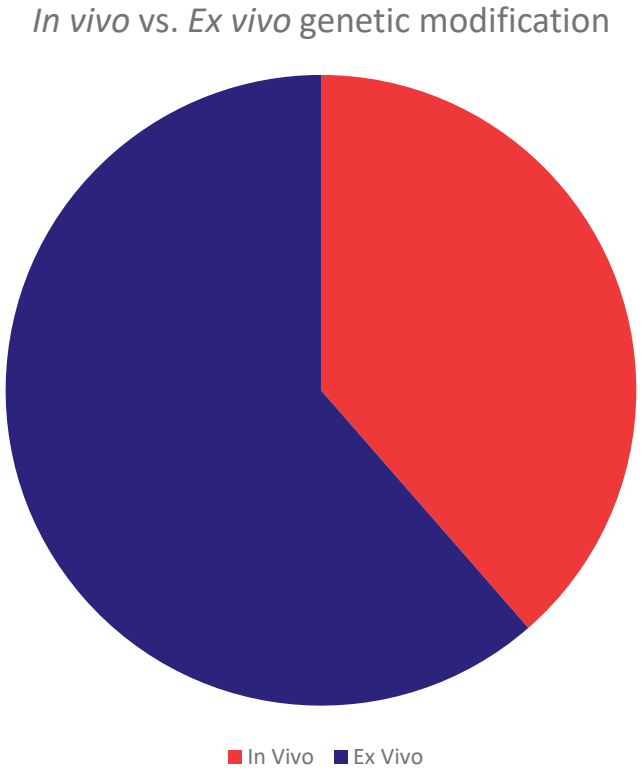
**Approved as of mid-April 2025*

Global Status	Q1 2024	Q2 2024	Q3 2024	Q4 2024	Q1 2025
Preclinical	1,471	1,436	1,393	1,424	1,432
Phase I	301	314	318	341	350
Phase II	282	279	289	306	319
Phase III	35	34	35	35	41
Pre-registration	4	5	6	11	13
Total	**2,093**	**2,068**	**2,041**	**2,117**	**2,155**

Source: Pharmaprojects | Citeline, April 2025

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

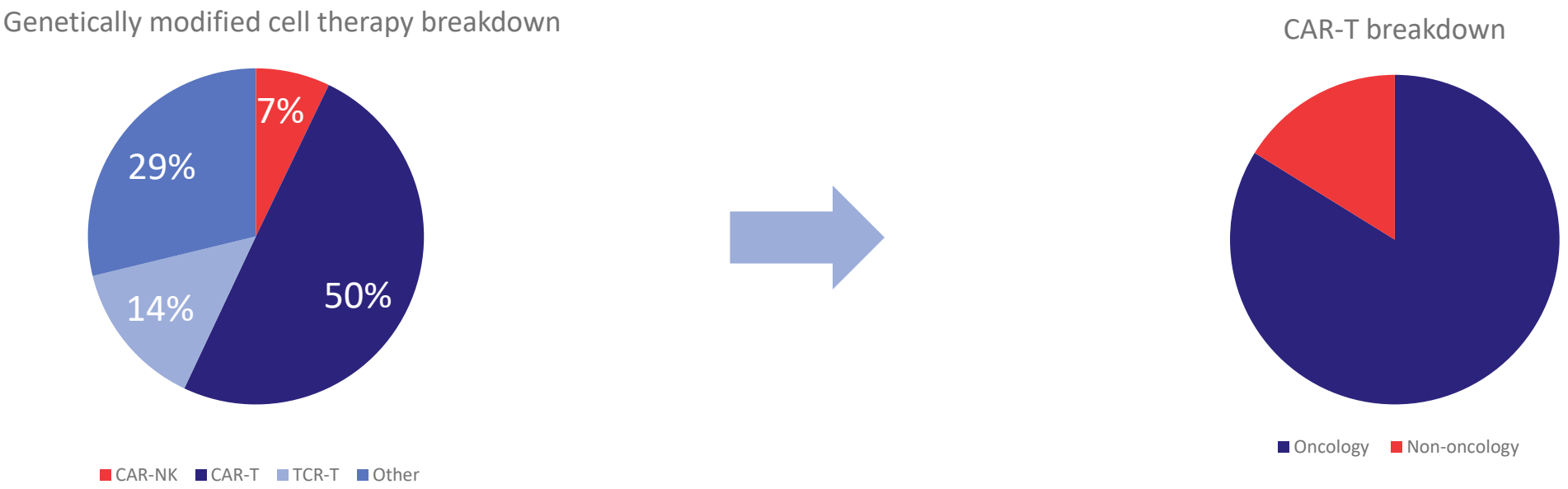
- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q1 2025, *in vivo* delivery techniques were used in 39% of gene therapies



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

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 29%, which includes a list of less commonly used technologies such as TCR-NK, CAR-M, and TAC-T
- 97% 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

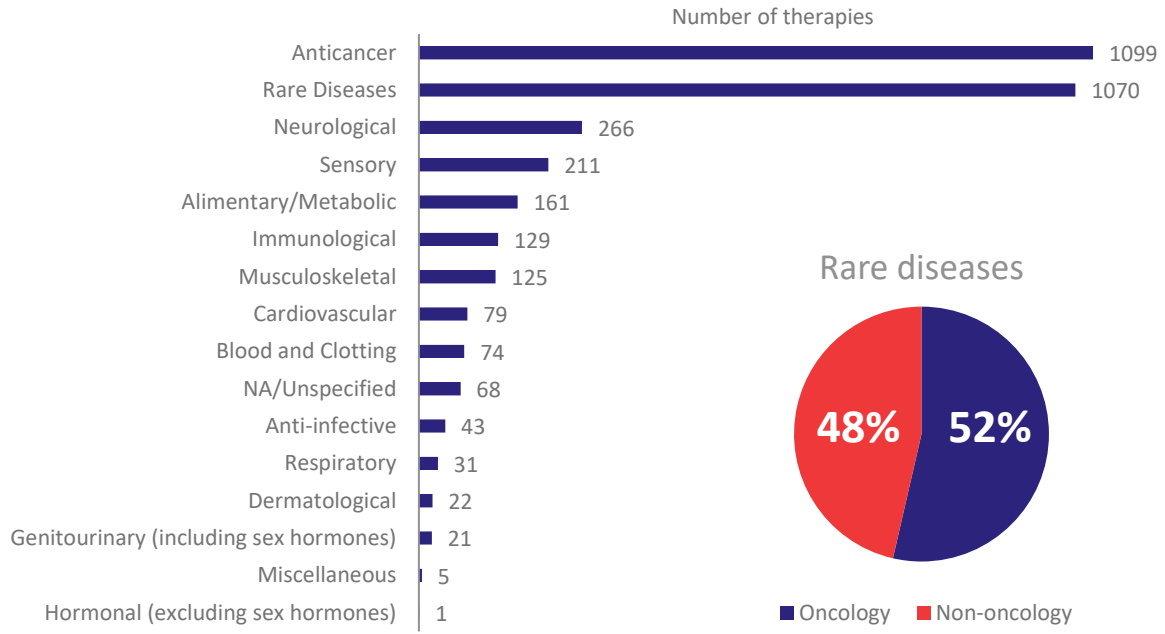


Source: Cell and Gene Therapy dashboard | Citeline, April 2025

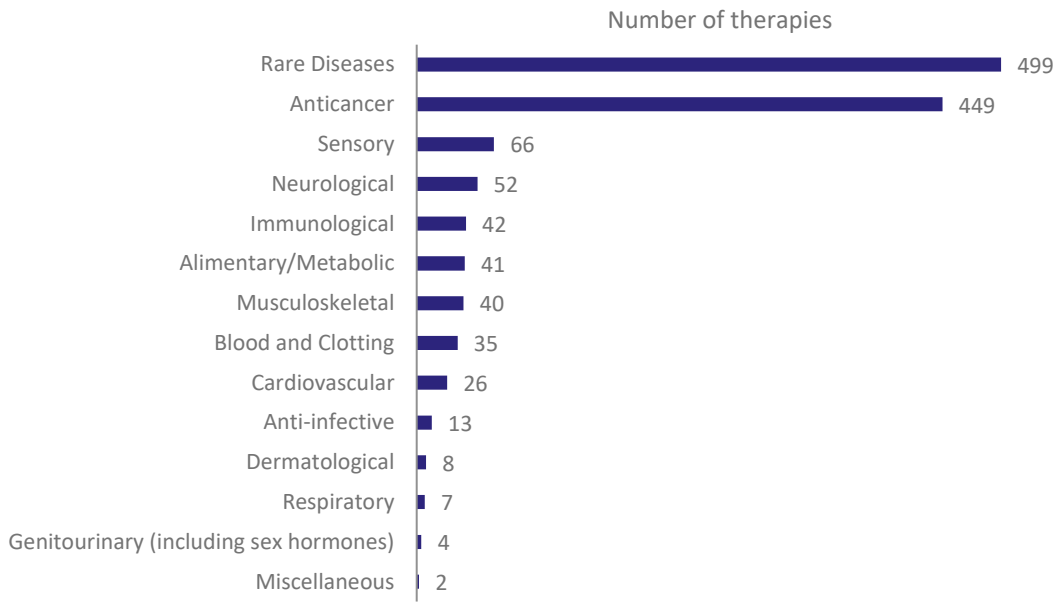
Gene therapy pipeline: most commonly targeted therapeutic areas

- 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 most commonly occurred in oncology, representing a majority of 52% compared to non-oncology rare disease gene therapy pipeline development, two percentage points lower than the previous quarter

Number of therapies form preclinical through pre-registration



Therapies in the clinic (excludes preclinical development)



Source: Pharmaprojects | Citeline, April 2025

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

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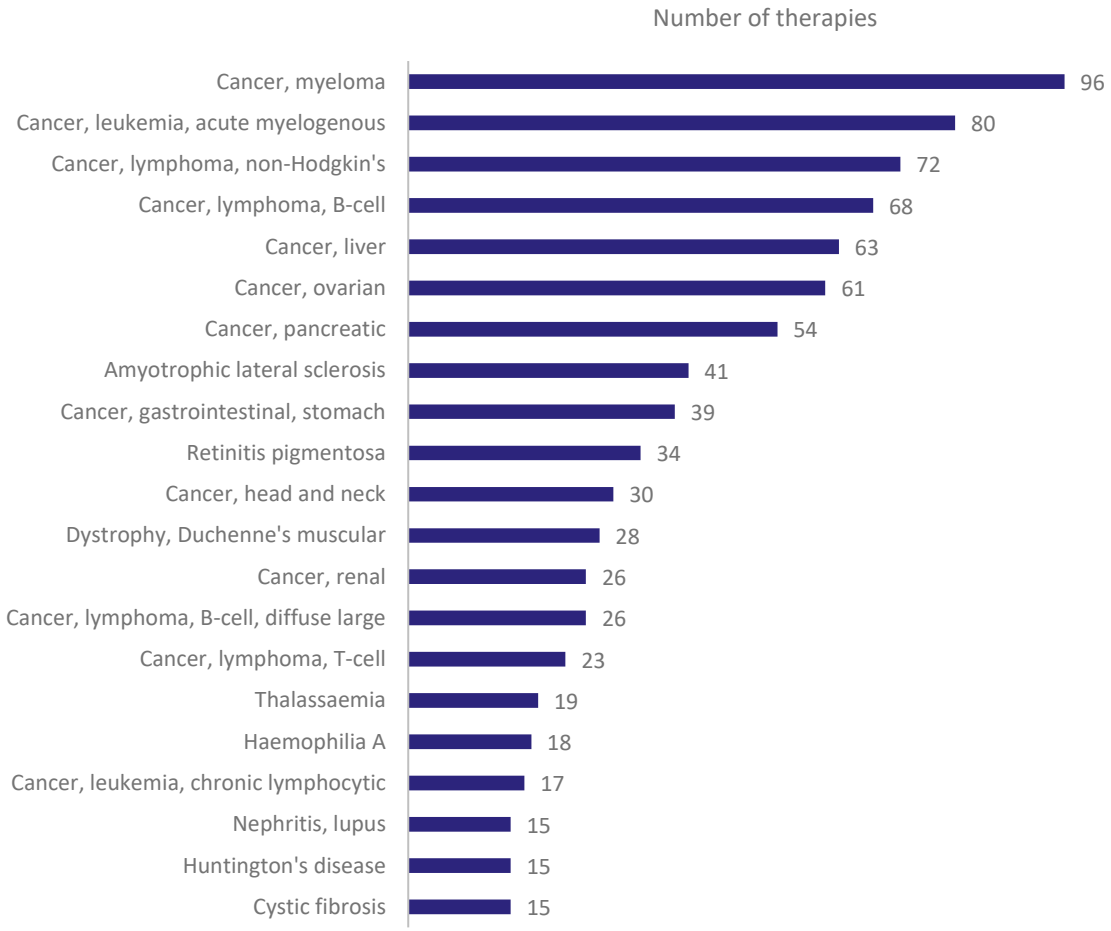
Gene therapy pipeline: most common rare diseases targeted

- For the 1,070 pipeline (preclinical to pre-registration) gene therapies being developed for rare diseases, eight out of the top 10 rare diseases were oncological, a trend seen throughout the past three years
- Liver cancer marks a new addition to the top five rare diseases for which gene therapies are being developed:

1. Myeloma
2. Acute myelogenous leukemia
3. Non-Hodgkin’s lymphoma
4. B-cell lymphoma
5. Liver cancer



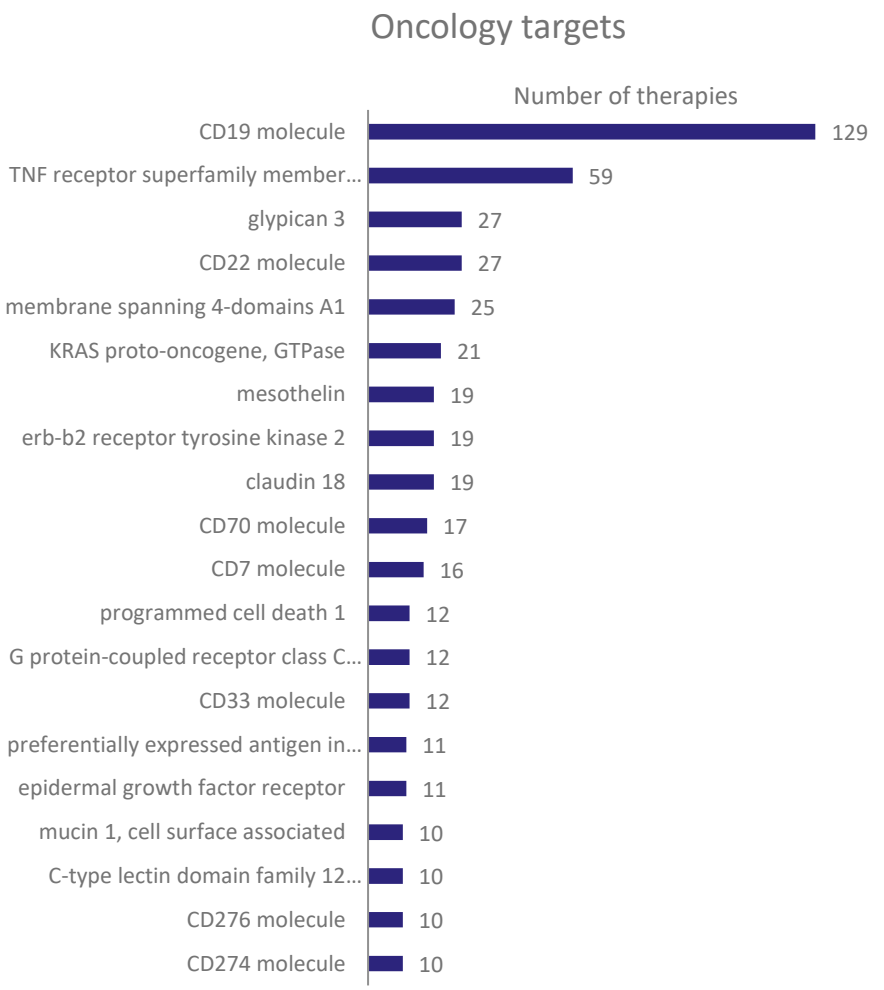
Source: Pharmaprojects | Citeline, April 2025

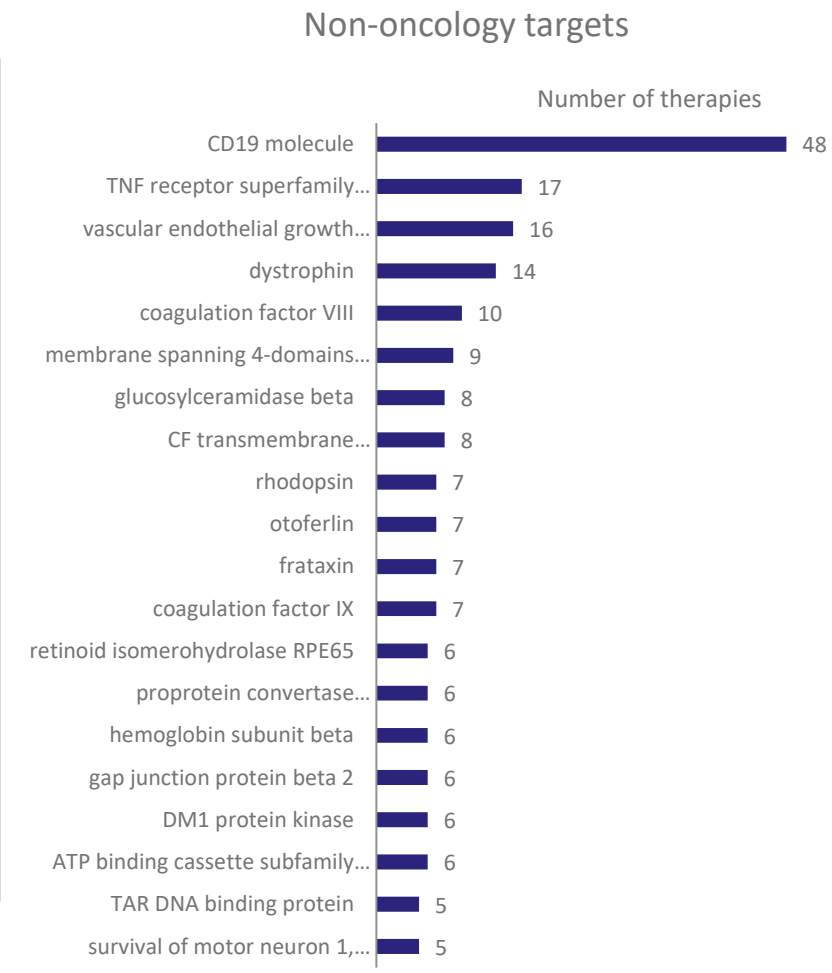
Note: Figures based on indications in pipeline development only for each therapy

Gene therapy pipeline: most common targets

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

- CD19 molecule and B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17, remain the top two most common target for oncology indications; glypican 3 climbs to the third most common
- CD19 molecule, vascular endothelial growth factor A, and TNF receptor superfamily member 17 continued to be the top three most common targets for non-oncology indications, though TNF receptor superfamily member 17 has risen to the second most common since Q4 2024

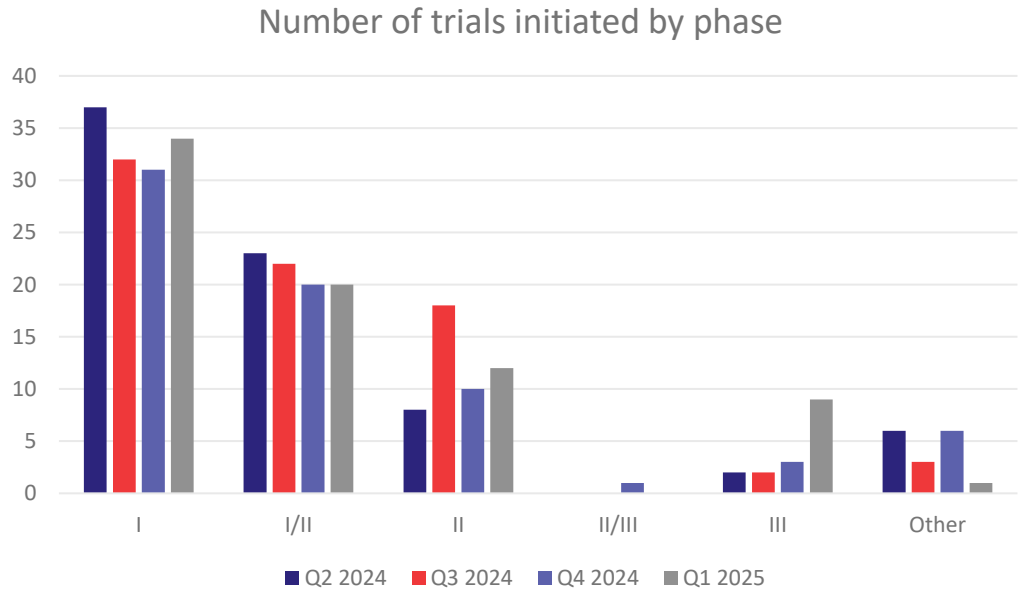


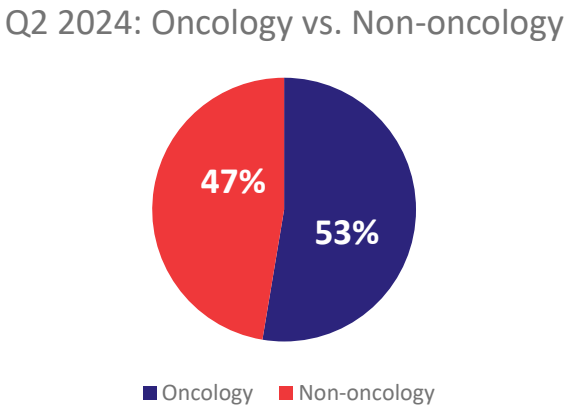


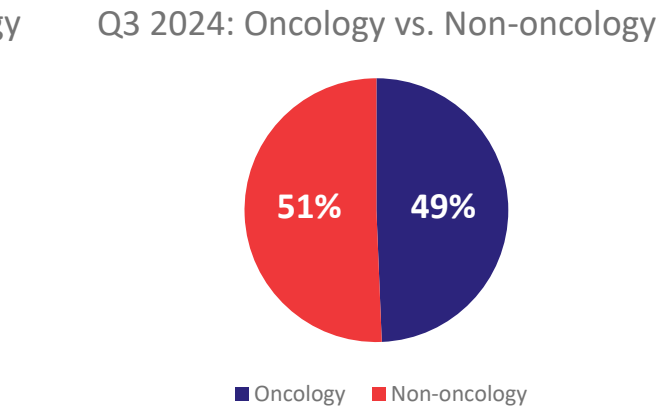
Source: Pharmaprojects | Citeline, April 2025

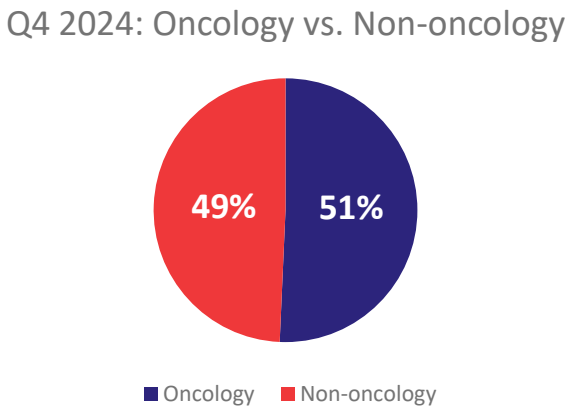
Gene therapy clinical trial activity

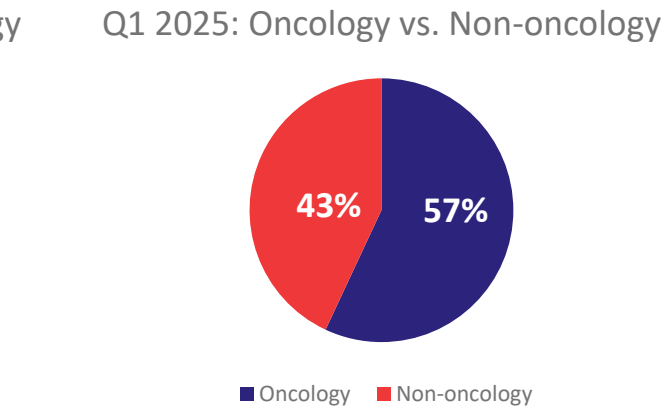
- The proportion of gene therapy trials for non-oncology indications decreased for the second quarter in a row to 43%, while the proportion of gene therapy trials for oncology indications is the highest for the past year
- 79 gene therapy trials were initiated in Q1 2025, eight more than the previous quarter











Source: Trialtrove | Citeline, April 2025

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Non-genetically modified cell therapy pipeline

Q1 2025

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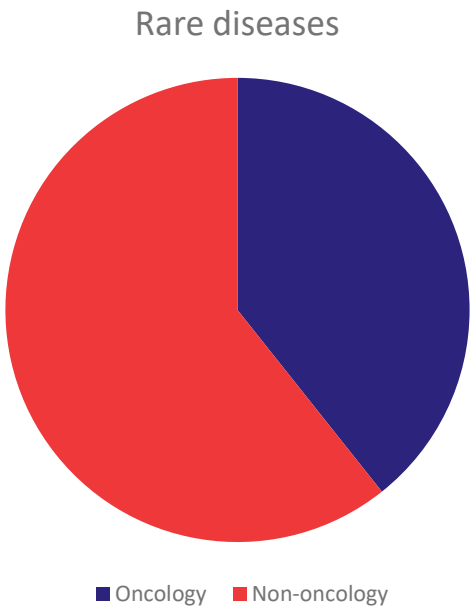
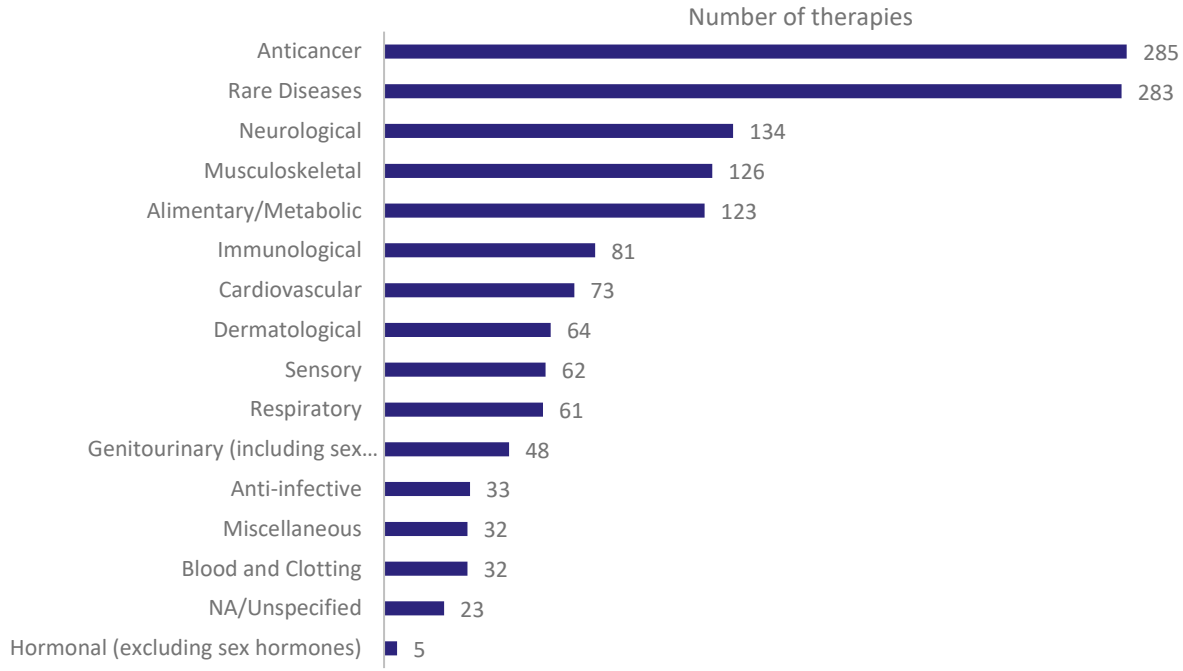
Non-genetically modified cell therapy pipeline: most commonly targeted therapeutic areas

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 increase from the previous quarter





Source: Pharmaprojects | Citeline, April 2025

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

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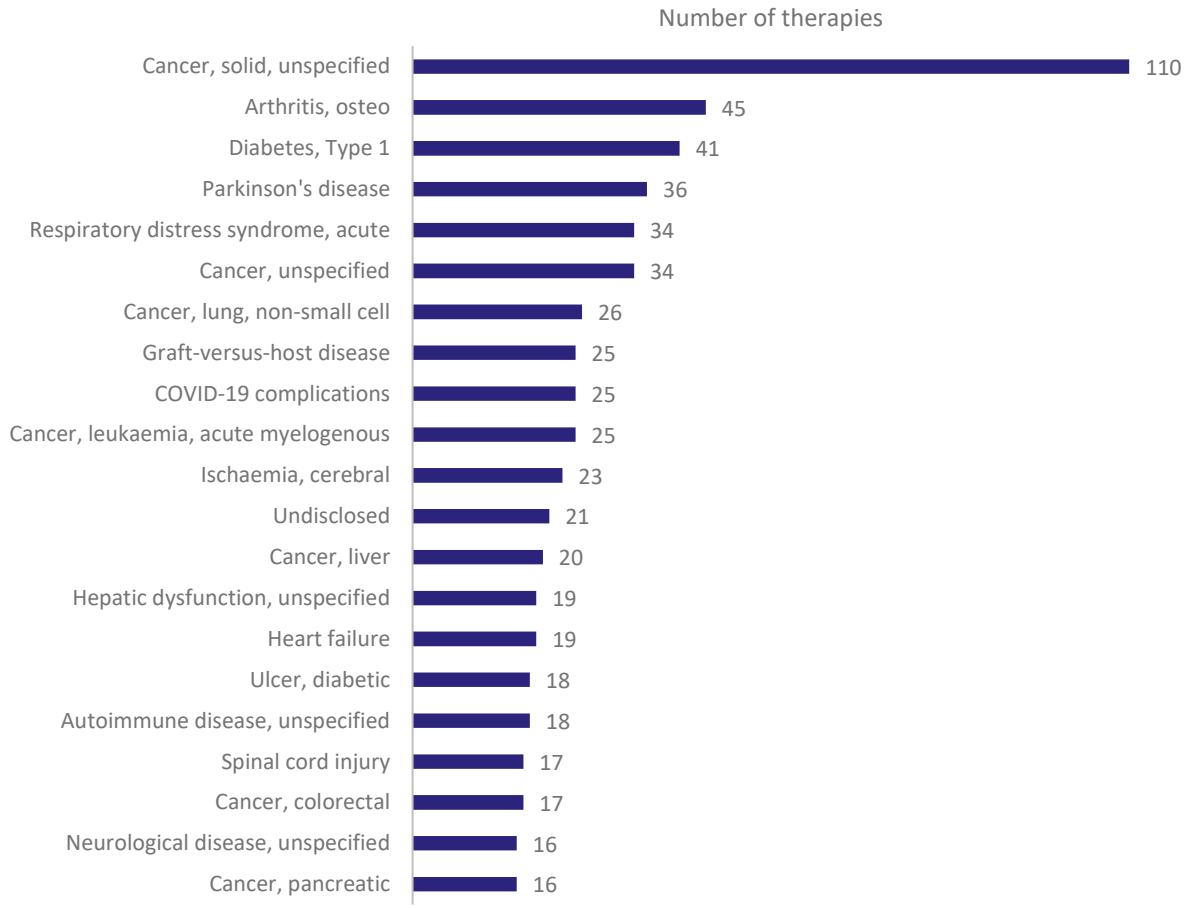
Non-genetically modified cell therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, in the same order as found in the previous quarter, the most targeted indications in Q1 2025 were:

1. Osteoarthritis
2. Type 1 diabetes
3. Parkinson’s disease



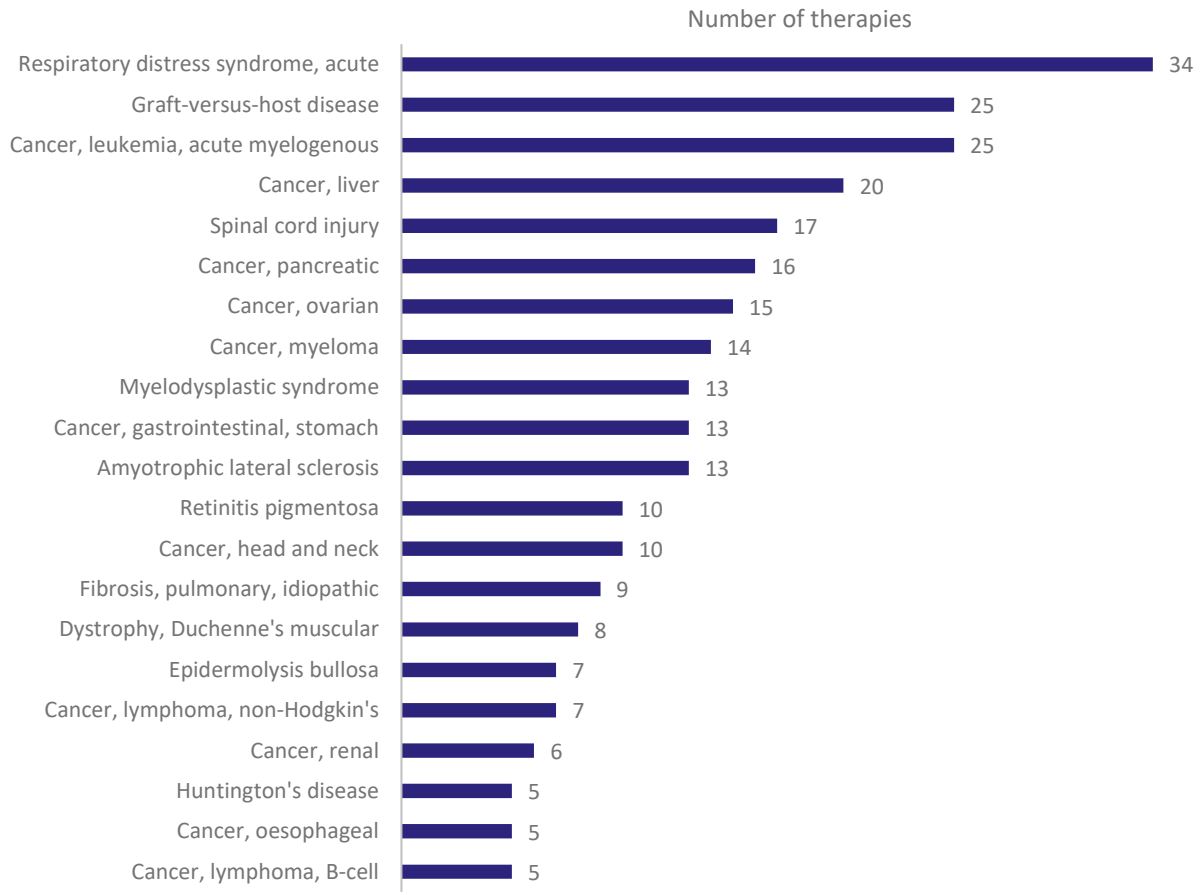
Source: Pharmaprojects | Citeline, April 2025

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

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 spinal cord injury



Source: Pharmaprojects | Citeline, April 2025

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

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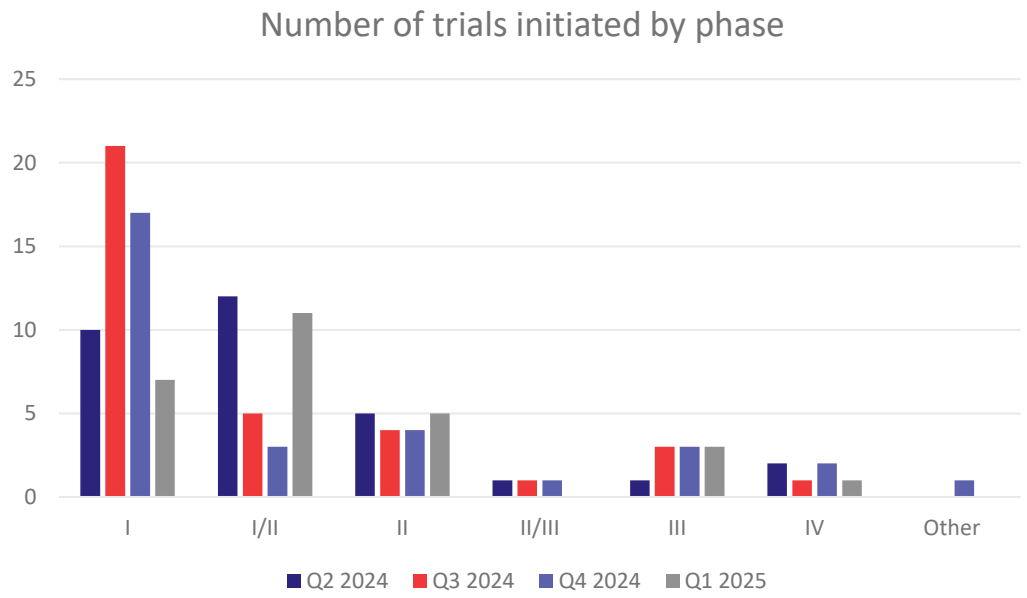
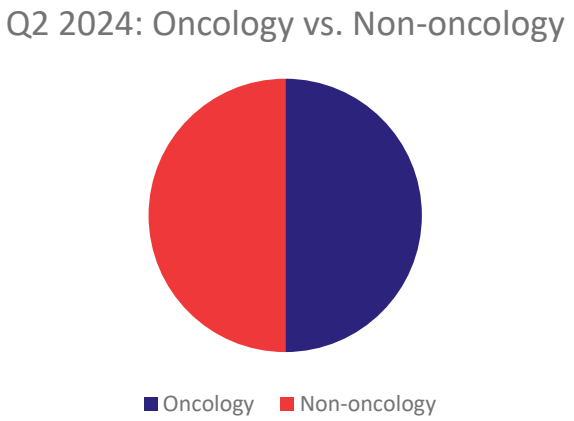
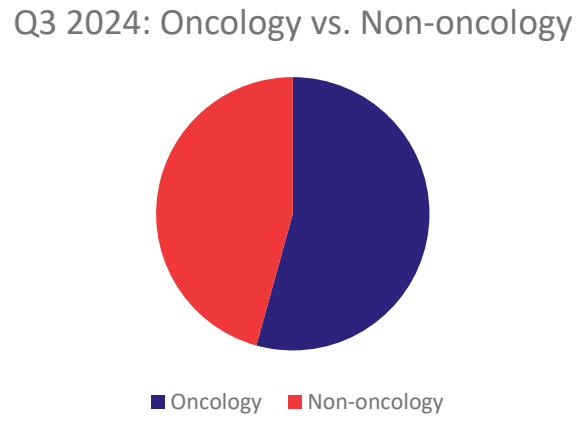
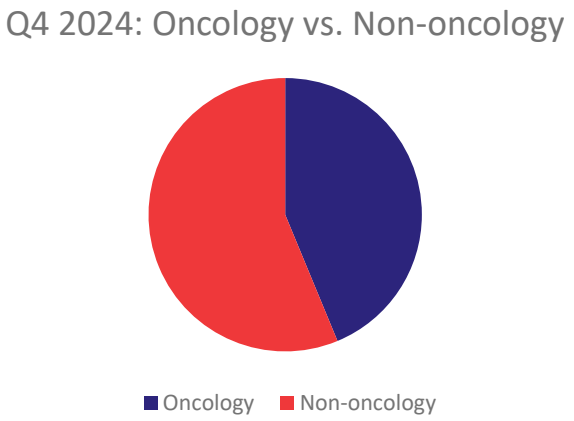
Non-genetically modified cell therapy trial activity

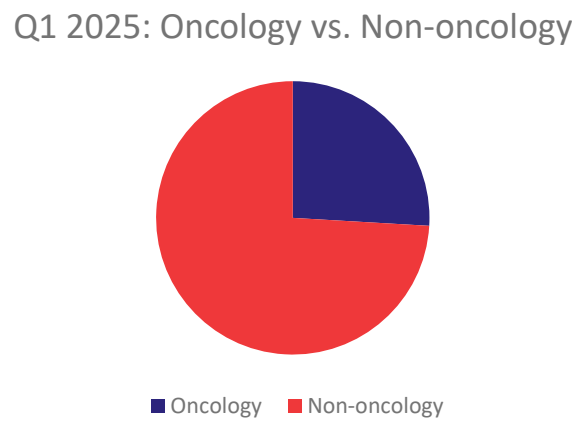
- 27 trials were initiated for non-genetically modified cell therapies in Q1 2025, four fewer than in Q4 2024
- Of these 27, 74% were for non-oncology indications, 16 percentage points higher than the previous quarter











Source: Trialtrove | Citeline, April 2025

RNA therapy pipeline

Q1 2025

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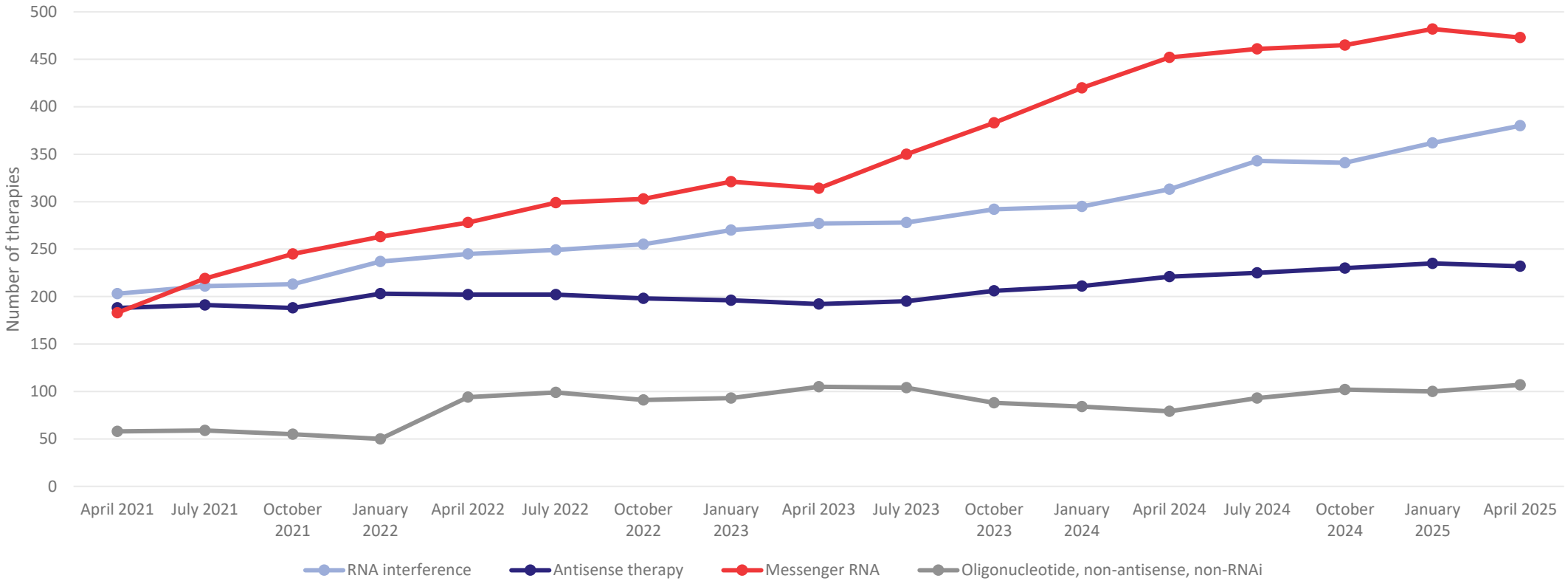
RNA therapy pipeline: most common modalities

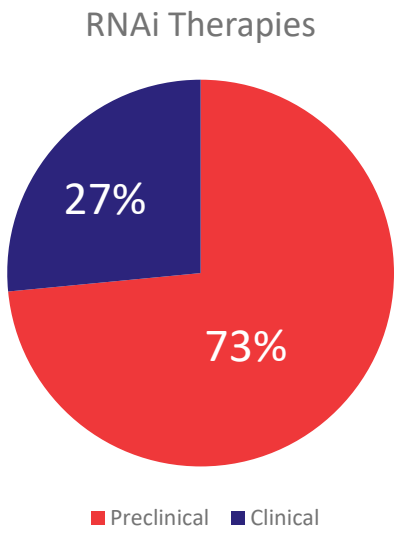
- Of RNA therapies in the pipeline, messenger RNA (mRNA) and RNA interference (RNAi) continued to be the preferred RNA modalities for research

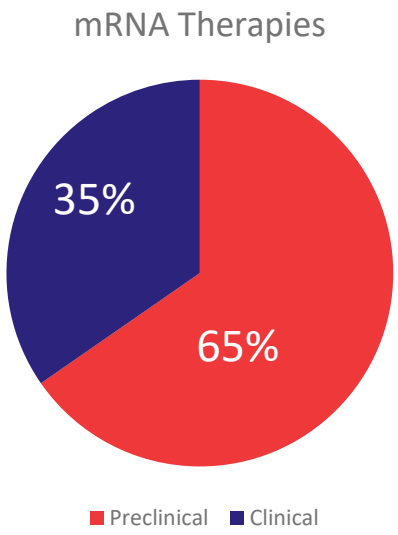


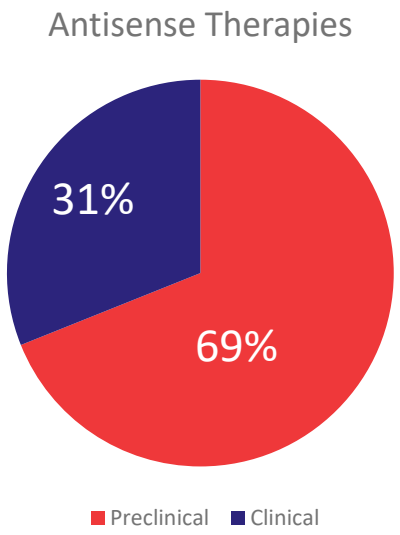
Source: Pharmaprojects | Citeline, April 2025

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

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 73%, 65%, and 69% of their respective pipelines





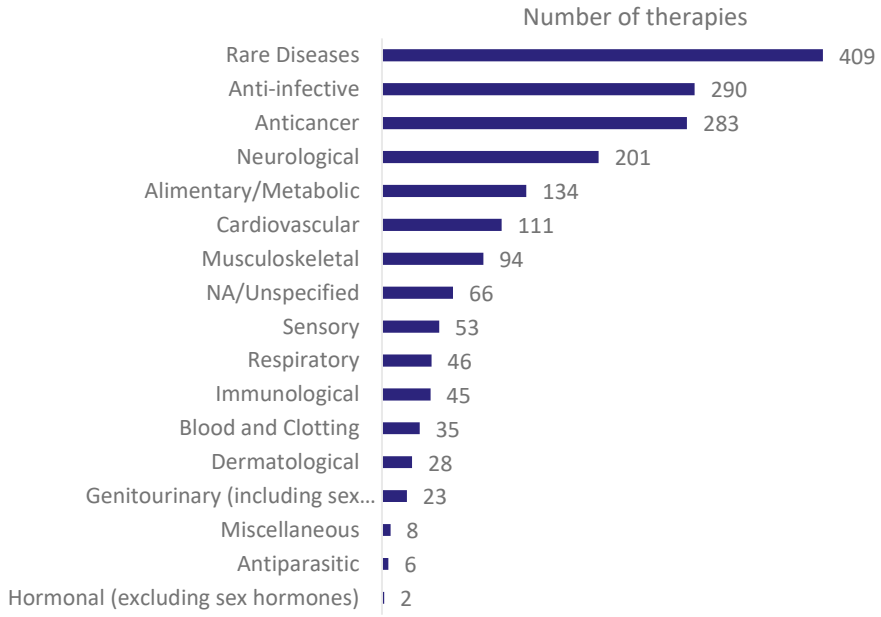


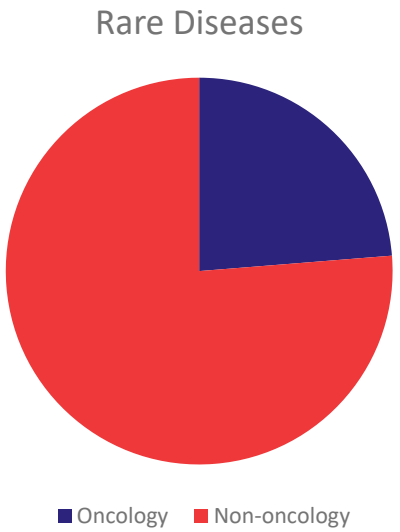
Source: Pharmaprojects | Citeline, April 2025

RNA therapies: most commonly targeted therapeutic areas

Of the 1,298 RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Rare diseases remained the top targeted therapeutic area by RNA therapies, while anti-infective indications remain as the second most commonly targeted
- Non-oncology indications continued to be the most targeted rare diseases by RNA therapies, representing a majority of 77%





Source: Pharmaprojects | Citeline, April 2025

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

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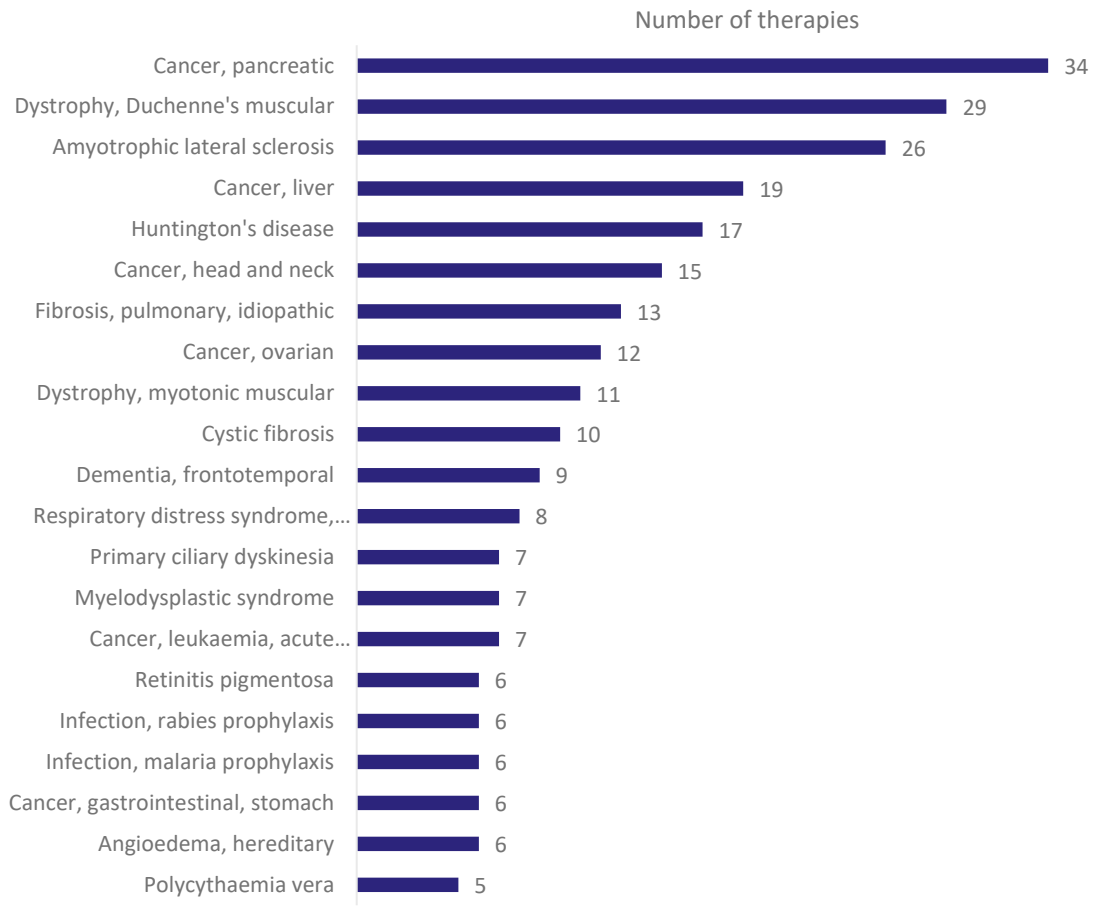
RNA therapies: most common rare diseases targeted

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, Duchenne muscular dystrophy, amyotrophic lateral sclerosis, and Huntington’s disease were the most targeted indications

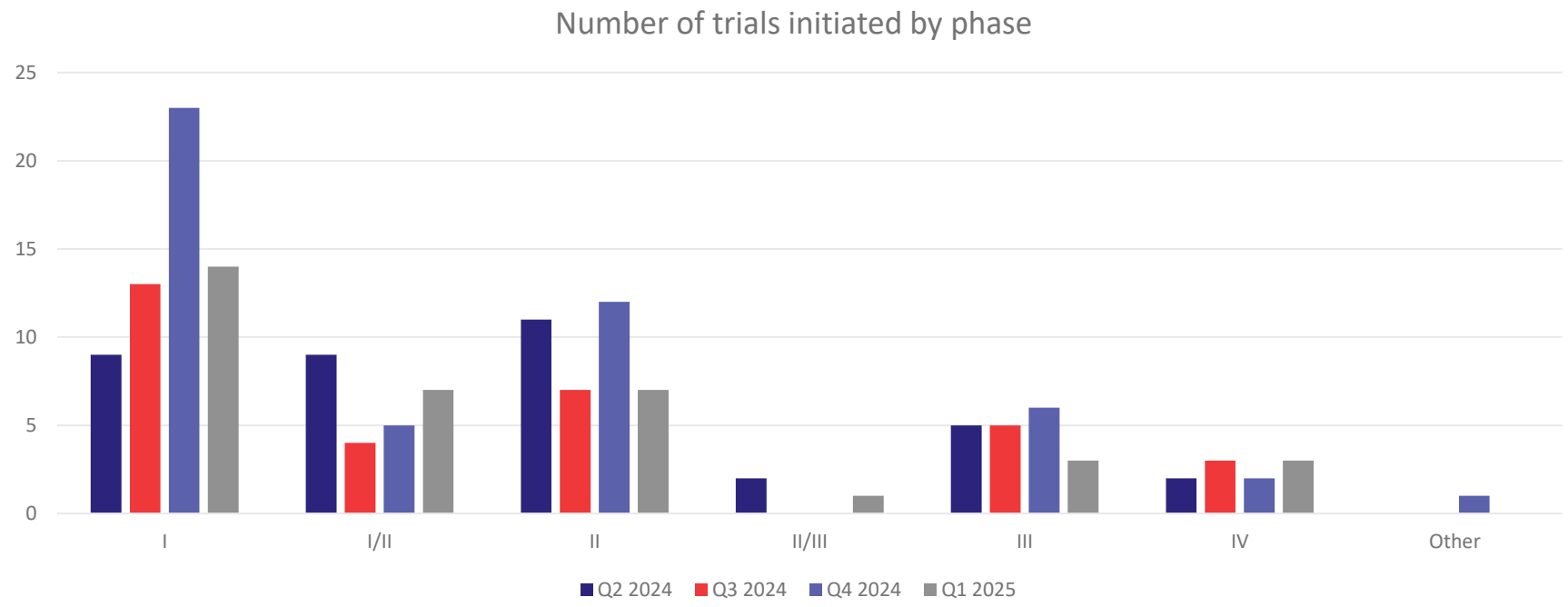


Source: Pharmaprojects | Citeline, April 2025

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

RNA therapy pipeline: clinical trial activity

- 35 RNA trials were initiated in Q1 2025, compared to 49 in Q4 2024, 83% of which were for non-oncology indications



Source: Trialtrove | Citeline, April 2025

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

Q1 2025

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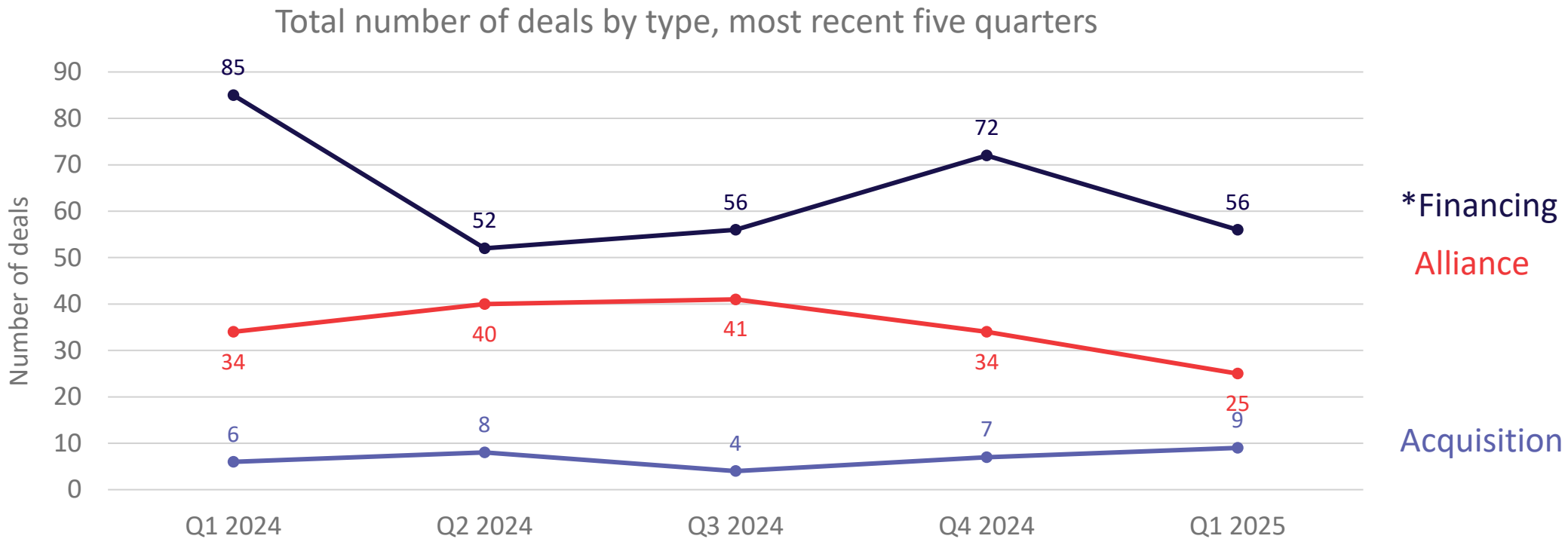
Alliance, acquisition, and financing in gene, cell, and RNA therapy

- Advanced molecular therapy companies announced 90 total deals during 2025's opening quarter
- Q1 2025's total represented a 20% decrease from the previous quarter's 113, and was 28% lower than the opening quarter of 2024 when 125 deals were signed
- Alliances and financings were down in Q1 2025 compared with the last quarter of 2024, but acquisitions saw a slight uptick



Sources: Biomedtracker | Citeline, BioSciDB | Evaluate, April 2025

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

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Q1 2025 acquisitions in gene, cell, and RNA therapy

- Nine acquisitions of advanced molecular therapy companies occurred in Q1 2025, the highest quarterly total within the last year
- The largest deal saw AstraZeneca paying up to $1 billion — including $425 million up front plus up to $575 million in earn-outs — for in vivo cell therapy company EsoBiotec
- Bluebird bio agreed to be acquired by funds managed by Carlyle and SK Capital, and later in the quarter, received an unsolicited bid from Ayrmid; 2seventy bio, a bluebird bio spin-off, will be acquired by Bristol Myers Squibb for $286 million

Deal date	Deal title	Potential deal value (USD $)
30 January 2025	MaxCyte Acquires SeQure Dx	7,000,000
21 February 2025	bluebird bio Announces Definitive Agreement to be Acquired by Carlyle and SK Capital	Undisclosed
21 February 2025	Skyline Therapeutics Divests US Division to Tebao Biotechnology	58,000,000
27 February 2025	Pacira BioSciences Acquires Remainder of GQ Bio Therapeutics	32,000,000
10 March 2025	Bristol Myers Squibb to Acquire 2seventy bio for $286M	286,000,000
14 March 2025	iOncologi to Acquire TargImmune Therapeutics to Expand Immunotherapy Pipeline Against Solid Tumors	Undisclosed
17 March 2025	AstraZeneca to Acquire EsoBiotec for $1B	1,000,000,000
26 March 2025	Alcon Acquires Majority Interest in Aurion Biotech	Undisclosed
28 March 2025	bluebird bio Gets Unsolicited Bid to be Acquired by Ayrmid	n/a – unsolicited bid

Sources: Biomedtracker | Citeline, BioSciDB | Evaluate, April 2025

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

Q1 2025

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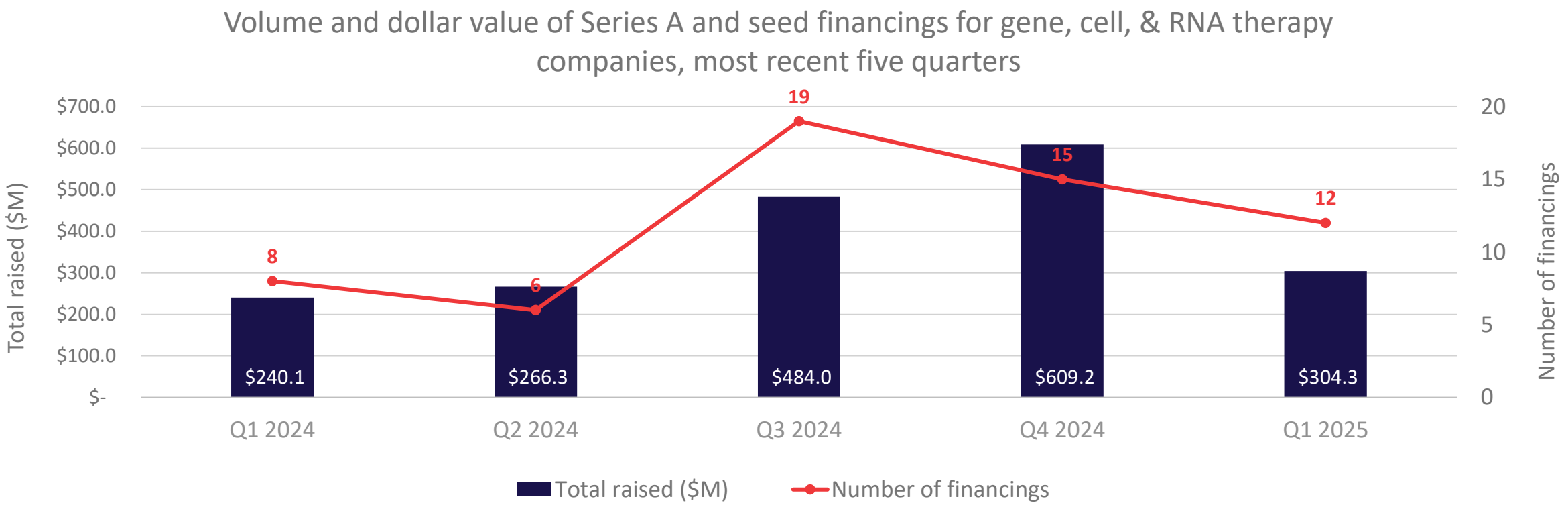
Start-up financing for gene, cell, and RNA therapy companies

- In Q1 2025, 12 start-ups together raised $304.3 million in seed and Series A financing
- The totals from Q1 represent a 20% decrease in volume and 50% decrease in value compared with the previous quarter, but are still ahead of the 8 start-up financings that brought in $240.1 million in the same quarter one year ago



Sources: Biomedtracker | Citeline, Evaluate, April 2025

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Q1 2025 start-up financing for gene, cell, and RNA therapy companies (1/2)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
08 January 2025	Neumirna Therapeutics Raises €20M in Series A Financing	miRNA	Denmark / Copenhagen	University of Copenhagen; Aalborg University	21
09 January 2025	Coave Therapeutics Secures €32M Series A Financing	Gene therapy	France / Paris	n/a, formerly known as Horama, whose scientific founders are from INSERM and Hospital Center of Nantes	33
09 January 2025	RheumaGen Raises $15M in Series A Financing	Gene editing	United States / Colorado / Aurora	University of Colorado	15
10 January 2025	RhyGaze Raises $86M Series A Financing	Gene therapy	Switzerland / Basel	Institute of Molecular and Clinical Ophthalmology Basel	86
11 January 2025	Immunis Raises $25M in A-1 Financing	Cell therapy	United States / California / Irvine	Undisclosed	25
22 January 2025	Arctic Therapeutics Raises €26.5M in Oversubscribed Series A Financing	siRNA	Iceland / Reykjavik	Center for Applied Genomics	28
13 February 2025	PulseSight Therapeutics Closes Series A Financing to Fund Clinical Development of PST-611 in dry AMD	Non viral gene therapy	France / Paris	Cochin Hospital; INSERM; University of Paris Cité	Undisclosed
19 February 2025	EnPlusOne Biosciences Raises $10M in Early Financing	RNA therapy	United States / Massachusetts / Watertown	Wyss Institute at Harvard University	10

Sources: Biomedtracker | Citeline, Evaluate, April 2025

Q1 2025 start-up financing for gene, cell, and RNA therapy companies (2/2)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
25 February 2025	Inceptor Bio Raised $21M Series A2 Financing	CAR-T	United States / North Carolina / Morrisville	Undisclosed founder - acquired team and manufacturing site from Arranta Bio	21
04 March 2025	Garuda Therapeutics Closes $50M Series A-1 Financing	Cell therapy	United States / Massachusetts / Natick	Harvard Stem Cell Institute; Broad Institute of MIT and Harvard; Brigham and Women's Hospital	50
18 March 2025	RegCell Secures $8.5M Seed Financing	Epigenetic T-cell reprogramming	Japan / Suita	Osaka University; Kyoto University	8.5
25 March 2025	Hubble Therapeutics Raises $7.3M in Series A Financing	Gene therapy	United States / New Hampshire / Hanover	University of Wisconsin-Madison School of Medicine and Public Health; Wisconsin Alumni Research Foundation	7.3

Sources: Biomedtracker | Citeline, Evaluate, April 2025

Notable Q1 2025 start-up gene, cell, and RNA therapy companies

	Company details	Academic source	Financing type/ amount raised	Lead investor(s)	Therapy areas of interest
RhyGaze	Cone optogenetics: Delivery of light sensor gene to cone cells to restore ability to detect light	Institute of Molecular and Clinical Ophthalmology Basel	Series A/$86M	GV (Google Ventures)	Ophthalmic (blindness)
GARUDA THERAPEUTICS	Off the shelf hematopoietic stem cell therapies that are HLA compatible and transgene free	Harvard Stem Cell Institute; Broad Institute of MIT and Harvard; Brigham and Women's Hospital	Series A-1/$50M	Lead unspecified; investors included OrbiMed; Northpond Ventures; Cormorant Asset Management; Kyowa Kirin	Hematology (blood disorders)
coave THERAPEUTICS	ALIGATER (Advanced Vectors-Ligand Conjugates) platform: Conjugating chemical ligands to specific amino acids of AAV capsid	n/a, formerly known as Horama, whose scientific founders are from INSERM and Hospital Center of Nantes	Series A/$33M	Novo Holdings; Bpifrance	Neurology (ALS) and ophthalmic (inherited retinal dystrophies)

Sources: Biomedtracker | Citeline, Evaluate, April 2025

Upcoming catalysts

Q1 2025

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Upcoming Catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q2 2025

Therapy	Generic name	Disease	Catalyst	Catalyst date
UM171	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	25 February 2025 - 25 April 2025
Oxlumo	lumasiran	Hyperoxaluria	Supplemental Approval Europe (PH1)	31 January 2025 - 30 April 2025
UM171	dorocubicel	Myelodysplastic Syndrome (MDS)	Accelerated CHMP Result	27 February 2025 - 31 May 2025
DCVax-L		Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	Approval Decision (U.K.)	31 March 2025 - 30 June 2025
RP-L102		Fanconi Anemia	CHMP Opinion	01 December 2024 - 30 June 2025
Aucatzyl	obecabtagene autoleucel	Acute Lymphoblastic Leukemia (ALL)	CHMP Opinion	01 January 2025 - 31 July 2025
Elevidys	delandistrogene moxeparvovec	Duchenne Muscular Dystrophy (DMD)	CHMP European Panel Results	01 February 2025 - 31 August 2025
Elevidys	delandistrogene moxeparvovec	Duchenne Muscular Dystrophy (DMD)	Approval Decision (Japan)	01 February 2025 - 31 August 2025
Amvuttra	vutrisiran	Transthyretin Amyloid Cardiomyopathy (ATTR-CM, Wild Type Or Hereditary)	CHMP Supplemental Opinion	01 March 2025 - 31 August 2025
RP-L102		Fanconi Anemia	Approval Decision (Europe)	02 March 2025 - 02 September 2025
Aucatzyl	obecabtagene autoleucel	Acute Lymphoblastic Leukemia (ALL)	Approval (Europe)	01 March 2025 - 30 September 2025
Amtagvi	lifileucel	Melanoma	CHMP Opinion	01 March 2025 - 30 September 2025
Beqvez	fidanacogene elaparvovec	Hemophilia B	Approval Decision (Japan)	01 March 2025 - 30 September 2025
Tryngolza		Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	Approval Decision (Europe)	19 February 2025 - 31 December 2025
BT524	fibrinogen	Hemostasis	PDUFA for BLA - 1st Review	09 January 2025 - 31 December 2025
Kresladi	marnetegragene autotemcel	Autoimmune Disorders	PDUFA Decision	01 January 2025 - 31 December 2025
OST-HER2		Osteosarcoma	PDUFA Approval	04 February 2025 - 30 September 2026

Source: Biomedtracker | Citeline, April 2025

Appendix

Methodology, sources, and glossary of key terms

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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

Glossary of Key Terms

Therapy type definitions

Gene therapy is the use of genetic material to treat or prevent disease.
For the purpose of this report, the following terms shall mean the following:

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

Glossary of Key Terms

Therapy type definitions, cont.

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

Glossary of Key Terms

Therapy type definitions, cont.

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

Glossary of Key Terms

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

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	Buyout, divestiture, spin-out, full acquisition, partial acquisition, reverse acquisition

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