American Society of Gene + Cell Therapy

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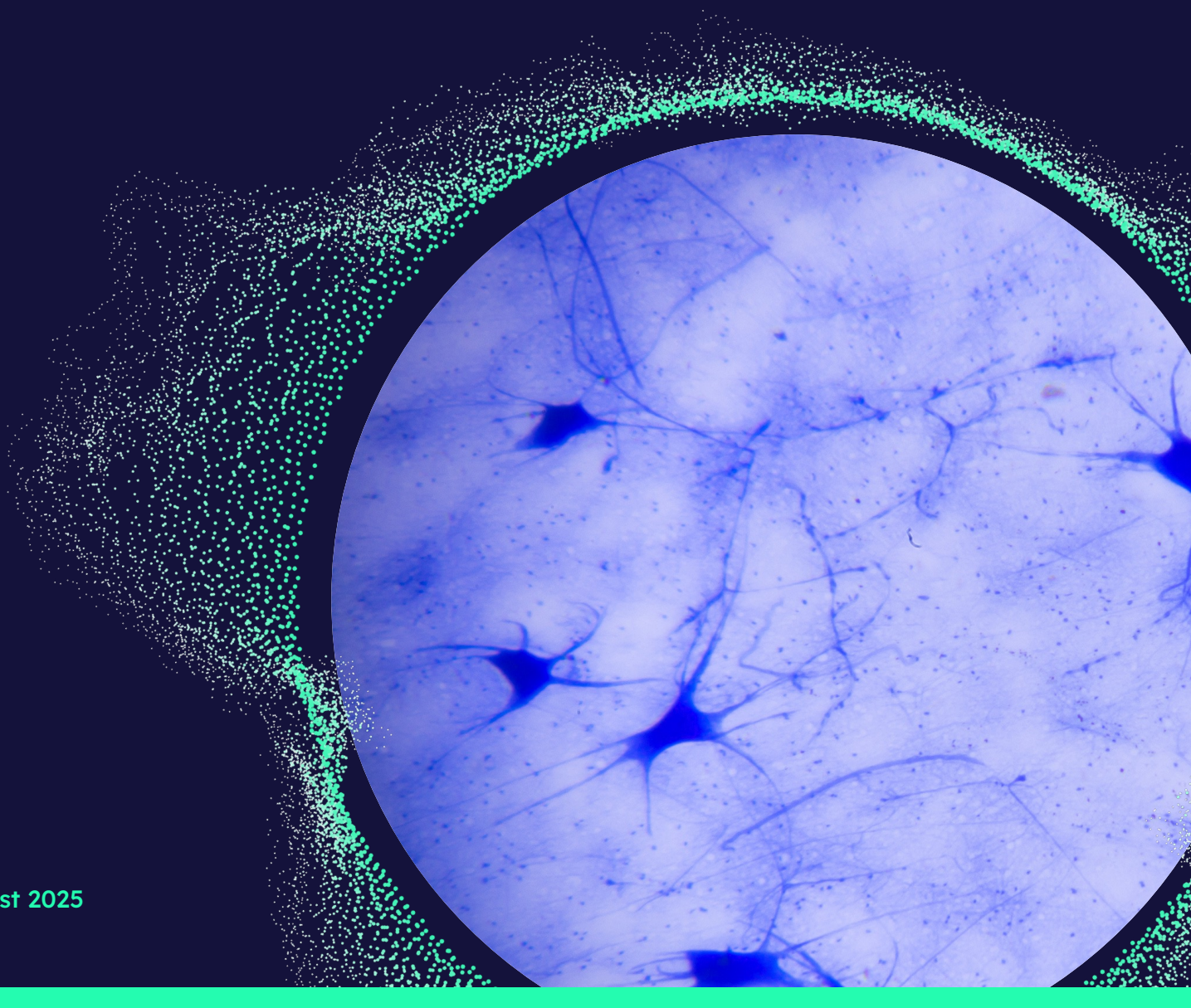
Gene, Cell, & RNA Therapy Landscape Report

Q2 2025 Quarterly Data Report



August 2025

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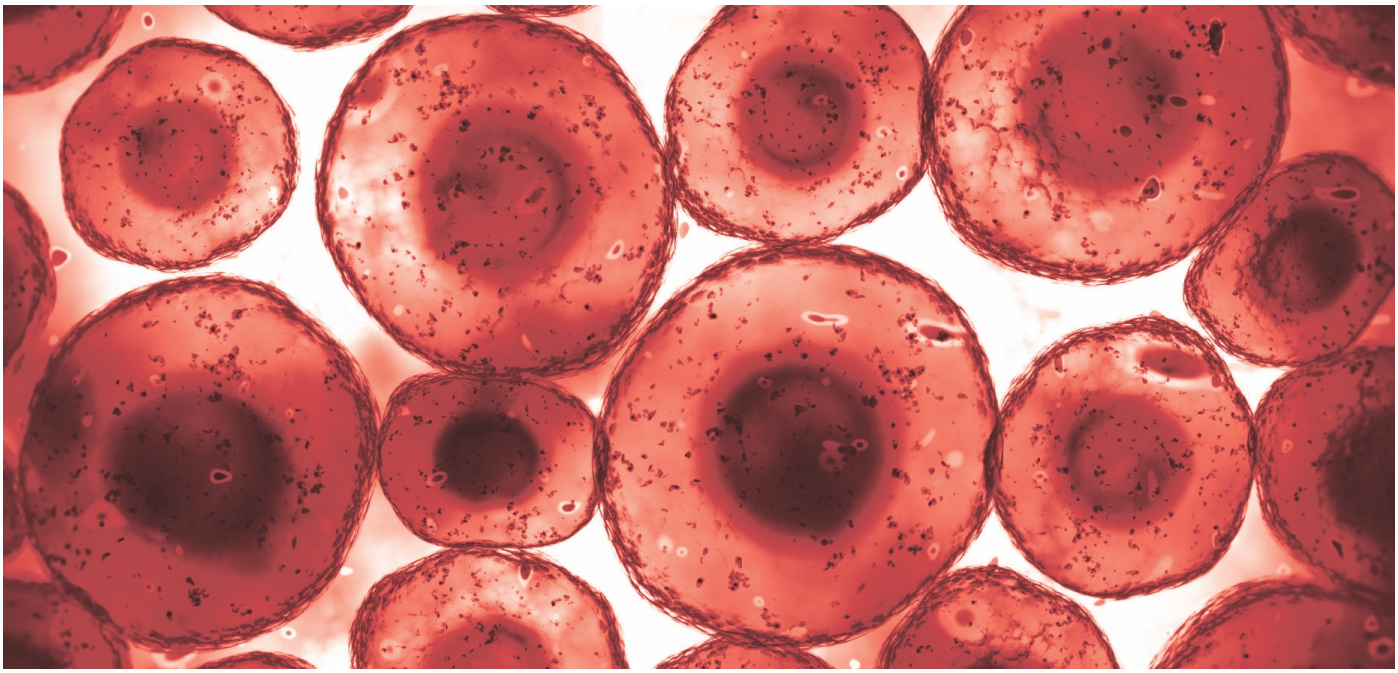
Introduction

Welcome to 2025's second quarterly report from ASGCT, Citeline, and Evaluate! This past quarter, three new therapeutics were approved, and the number of gene therapy programs continues to climb. In the US, FDA approved the cell-based gene therapy Zevaskyn for recessive dystrophic epidermolysis bullosa (RDEB) as well as the lower-dose mRNA vaccine mNexspike for COVID-19. In China, the country's first gene therapy for hemophilia B was approved.

Gene therapy programs at each stage from preclinical through Phase III have continued to increase in Q2, with oncology and rare diseases remaining the top areas of development. Eight of the top 10 rare diseases in the overall pipeline are oncological, continuing a trend from the past three years. Of the 80 gene therapy trials initiated in Q2, 64% are for oncology indications, the highest proportion of the past year. In the cell therapy pipeline, oncology and rare diseases also remain the top areas of non-genetically modified cell therapy development. Of the 33 cell therapy trials initiated in Q2, 76% were for non-oncology indications. In the RNA therapy pipeline, 38 trials were initiated in Q2, up from 35 last quarter, of which 74% were for non-oncology indications.

Acquisitions of CGT assets continue to increase quarter by quarter, with a 33% jump in volume, including four deals worth more than a billion dollars each. There were three start-up financings, which raised $197 million in Q2, continuing the decline of the past three quarters in both volume and value.

Thank you,
David Barrett, JD
CEO, ASGCT



Key takeaways from Q2 2025

Three new approvals across the gene, cell, and RNA landscape in Q2 2025

- The US FDA approved Abeona's cell-based gene therapy, Zevaskyn, for recessive dystrophic epidermolysis bullosa (RDEB), as well as Moderna's next-generation, lower-dose mRNA vaccine, mNexspike, for COVID-19
- In China, Belief BioMed achieved NMPA approval for BBM-H901 — China's first approved hemophilia B gene therapy

Over the past quarter, more RNA therapies that are being pursued in oncology indications are reaching the clinic

- 679 asset-indications are at the clinical trial stage compared to 645 in Q1 2025 — a 5.3% rise quarter on quarter and a two-percent-point increase in relation to respective preclinical asset-indications
- 26% of RNA therapy trials initiated in Q2 2025 were targeting oncology indications, as opposed to 17% in Q1 2025, marking the highest proportion of RNA oncology trials initiated in a quarter, for over two years

Overall dealmaking was flat, and start-up financing continued downward trend

- Advanced molecular therapy companies signed 91 deals in Q2 2025, nearly equivalent to last quarter's volume
- Acquisition activity picked up, with 12 such deals in Q2 compared with nine in the previous quarter, highlighted by three billion-dollar takeovers by big pharma
- Financing activity overall was down, especially among start-ups which saw a total $197 million raised from only three transactions

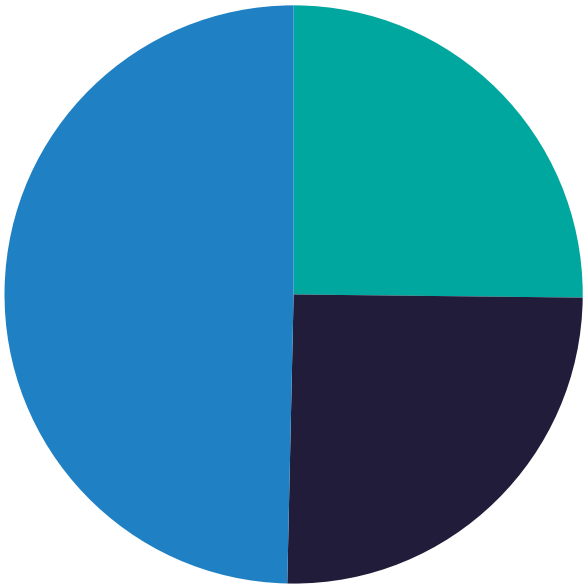
Key highlights in Q2 2025

APPROVED GENE, CELL, AND RNA THERAPIES

Globally, for clinical use:

- **36 gene therapies have been approved** (including genetically modified cell therapies)
  - Belief BioMed achieved NMPA approval in China for its hemophilia B gene therapy, BBM-H901; the FDA approved Abeona's cell-based gene therapy, Zevaskyn, for recessive dystrophic epidermolysis bullosa (RDEB).
- **36 RNA therapies have been approved**
  - Moderna's next-generation, lower-dose mRNA vaccine, mNexspike, was approved in the US for COVID-19.
- **71 non-genetically modified cell therapies have been approved**

Source: Pharmaprojects | Citeline, July 2025



APPROVED GENE THERAPIES AS OF Q2 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	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

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
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
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, 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

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
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
Vyjuvek	beremagene geperpavec	2023	Dystrophic epidermolysis bullosa	US, EU	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	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, UK	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

Source: Pharmaprojects | Citeline, July 2025

APPROVED RNA THERAPIES AS OF Q2 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

Product name	Generic name	Year first approved	Disease(s)	Locations approved*	Originator company
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
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

Product name	Generic name	Year first approved	Disease(s)	Locations approved*	Originator company
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
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
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

Product name	Generic name	Year first approved	Disease(s)	Locations approved*	Originator company
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	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

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

Source: Pharmaprojects | Citeline, July 2025

KEY HIGHLIGHTS IN Q2 2025 (NOTEWORTHY EVENTS THAT HAPPENED IN Q2 2025)

Drug	Event Type	Indication	Molecule	Event Date
BIIB080	Regulatory - Fast Track Status	Alzheimer's Disease (AD)	Antisense	04/02/2025
RVB-003	Regulatory - Orphan Drug Designation (U.S.)	Congenital Ichthyosis	Cellular	04/03/2025
MNV-201	Regulatory - Rare Pediatric Disease (RPD) Designation	Mitochondrial Respiratory-Chain Diseases	Cellular	04/03/2025
ALLO-329	Regulatory - Fast Track Status	Systemic Lupus Erythematosus (SLE)	Cellular	04/07/2025
ALLO-329	Regulatory - Fast Track Status	Systemic Sclerosis	Cellular	04/07/2025
CBP-4888	Regulatory - Orphan Drug Designation (Europe)	Eclampsia/Pre-Eclampsia	siRNA/ RNAi	04/08/2025
ARCT-2304	Regulatory - Fast Track Status	Pandemic Influenza Vaccines	Other Nucleic Acid	04/10/2025
BBM-H901	Regulatory - Approval (China)	Hemophilia B	Viral Gene Therapy	04/10/2025
FT819	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Systemic Lupus Erythematosus (SLE)	Cellular	04/14/2025
LYL314	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Non-Hodgkin's Lymphoma (NHL)	Cellular	04/15/2025
ATSN-201	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	X-Linked Retinoschisis	Viral Gene Therapy	04/15/2025
PBGENE-HBV	Regulatory - Fast Track Status	Hepatitis B (HBV) Treatment (Antiviral)	Cellular	04/15/2025
AMT-130	Regulatory - Breakthrough Therapy Designation (U.S.)	Huntington's Disease	Viral Gene Therapy	04/17/2025
BCB-276	Regulatory - Breakthrough Therapy Designation (U.S.)	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	Cellular	04/22/2025
Vyjuvek	Regulatory - Approval (Europe)	Epidermolysis Bullosa	Viral Gene Therapy	04/23/2025
DYNE-251	Regulatory - Orphan Drug Designation (Europe)	Duchenne Muscular Dystrophy (DMD)	Antisense	04/24/2025
MVdeltaC	Regulatory - Orphan Drug Designation (U.S.)	Mesothelioma	Viral Gene Therapy	04/25/2025
PS-002	Regulatory - Orphan Drug Designation (Europe)	Immunoglobulin A (IgA) Nephropathy (Berger's Disease)	Viral Gene Therapy	04/28/2025
Zevaskyn	Regulatory - Approval (U.S.)	Epidermolysis Bullosa	Cellular	04/28/2025
IGNK001	Regulatory - FDA Response	Acute Myelogenous Leukemia (AML)	Cellular	04/30/2025
ION-581	Regulatory - Orphan Drug Designation (Europe)	Angelman Syndrome	Antisense	04/30/2025

Drug	Event Type	Indication	Molecule	Event Date
4D-150	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Diabetic Macular Edema (Ophthalmology)	Viral Gene Therapy	05/01/2025
AAV-GAD	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Parkinson's Disease (PD)	Viral Gene Therapy	05/09/2025
BEAM-302	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Alpha-1 Antitrypsin Deficiency (A1AD or AATD)	Viral Gene Therapy	05/12/2025
RGX-121	Regulatory - Priority Review	Mucopolysaccharidosis II (MPS II; Hunter Syndrome)	Viral Gene Therapy	05/13/2025
Elevidys	Regulatory - Approval (Japan)	Duchenne Muscular Dystrophy (DMD)	Other Nucleic Acid	05/13/2025
GIVI-MPC	Regulatory - Orphan Drug Designation (Europe)	Becker Muscular Dystrophy (BMD)	Cellular	05/14/2025
CRD-002	Regulatory - Orphan Drug Designation (U.S.)	Spinocerebellar Ataxia	Antisense	05/14/2025
BCB-276	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	Cellular	05/15/2025
mRNA-1083	Regulatory - NDA/BLA Withdrawal	COVID-19 Prevention	Other Nucleic Acid	05/21/2025
Aucatzyl	Regulatory - CHMP (European Panel) Results (Positive)	Acute Lymphoblastic Leukemia (ALL)	Cellular	05/22/2025
OCU410ST	Regulatory - Rare Pediatric Disease (RPD) Designation	Stargardt Disease (Ophthalmology)	Viral Gene Therapy	05/27/2025
C-CAR168	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Systemic Lupus Erythematosus (SLE)	Cellular	05/27/2025
CAN-2409	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Prostate Cancer	Other Nucleic Acid	05/28/2025
BEAM-302	Regulatory - Orphan Drug Designation (U.S.)	Alpha-1 Antitrypsin Deficiency (A1AD or AATD)	Viral Gene Therapy	05/29/2025
mNexspike	Regulatory - Approval (U.S.)	COVID-19 Prevention	mRNA (messenger RNA)	05/30/2025
AMX0114	Regulatory - Fast Track Status	Amyotrophic Lateral Sclerosis (ALS)	Antisense	06/03/2025
CHM 2101	Regulatory - Fast Track Status	Neuroendocrine Tumors (NET)	Cellular	06/04/2025
Deramiocel	Regulatory - Orphan Drug Designation (U.S.)	Becker Muscular Dystrophy (BMD)	Cellular	06/17/2025

Drug	Event Type	Indication	Molecule	Event Date
SENTI-202	Regulatory - Orphan Drug Designation (U.S.)	Acute Myelogenous Leukemia (AML)	Cellular	06/18/2025
UM171	Regulatory - CHMP (European Panel) Results (Positive)	Bone Marrow and Stem Cell Transplant - Graft vs. Host Disease (GVHD) Prophylaxis	Cellular	06/19/2025
OST-HER2	Regulatory - FDA Response	Osteosarcoma	Cellular	06/24/2025
SNUG01	Regulatory - Orphan Drug Designation (U.S.)	Amyotrophic Lateral Sclerosis (ALS)	Viral Gene Therapy	06/24/2025
PBGENE-DMD	Regulatory - Rare Pediatric Disease (RPD) Designation	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	06/25/2025
EG-70	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Bladder Cancer	Cellular	06/25/2025
GTX-102	Regulatory - Breakthrough Therapy Designation (U.S.)	Angelman Syndrome	Antisense	06/27/2025
VGN-R09b	Regulatory - Fast Track Status	Parkinson's Disease (PD)	Viral Gene Therapy	06/30/2025

Source: Biomedtracker | Evaluate, July 2025

Pipeline overview

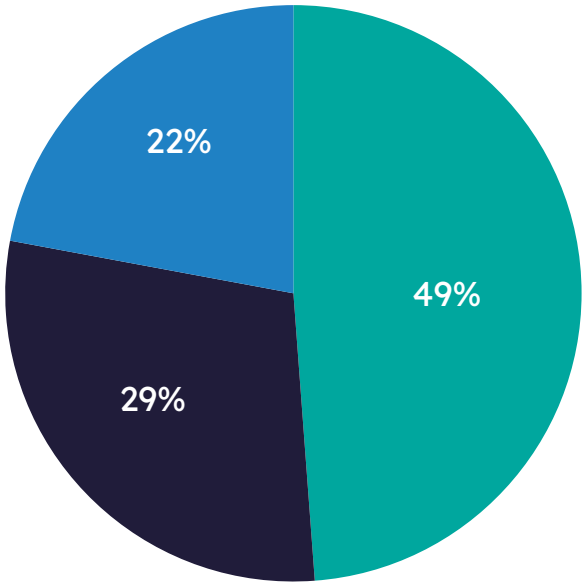
PIPELINE OF GENE, CELL, AND RNA THERAPIES

4,469 therapies are in development, ranging from preclinical through pre-registration

- 2,210 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
- 962 non-genetically modified cell therapies are in development, accounting for 22% of gene, cell, and RNA therapies

Source: Pharmaprojects | Citeline, July 2025

Pipeline therapies by category



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 pipeline development
- The number of gene therapies currently in pre-registration remains the same as Q1 2025, though the assets have changed slightly, including the addition of Orchard Therapeutics and Fondazione Telethon's OTL-103 now filed in both the US and EU, and Belief BioMed recently attaining approval for BBM-H901
- Therapies currently in pre-registration:

In the US

- RP-L201 (Rocket Pharmaceuticals)
- RGX-121 (Regenxbio)
- SEL-212 (3SBio)
- UX111 (Ultragenyx)
- vusolimogene oderparepvec (Replimune)
- PRGN-2012 (Precigen)
- OTL-103 (Fondazione Telethon/ Orchard Therapeutics)

In the EU

- RP-L102 (Rocket Pharmaceuticals)
- 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

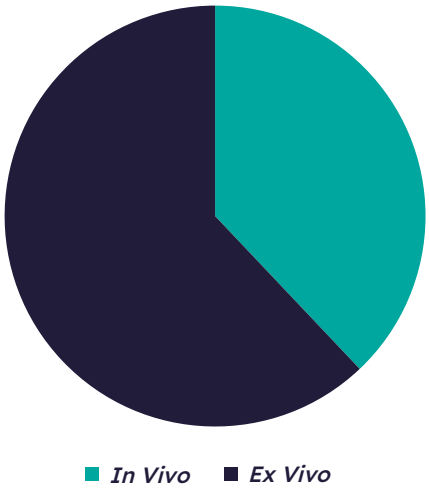
- Anbal-cel (Curocell)

Global Status	Q2 2024	Q3 2024	Q4 2024	Q1 2025	Q2 2025
Preclinical	1,436	1,393	1,424	1,432	1,461
Phase I	314	318	341	350	361
Phase II	279	289	306	319	330
Phase III	34	35	35	41	45
Pre-registration	5	6	11	13	13
Total	**2,068**	**2,041**	**2,117**	**2,155**	**2,210**

Source: Pharmaprojects | Citeline, July 2025

GENETIC MODIFICATION: *IN VIVO* VS. *EX VIVO*

- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q2 2025, *in vivo* delivery techniques were used in 38% of gene therapies

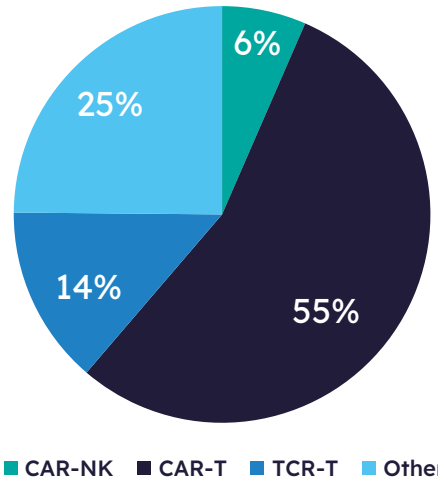


Source: Cell and Gene Therapy dashboard | Citeline, July 2025

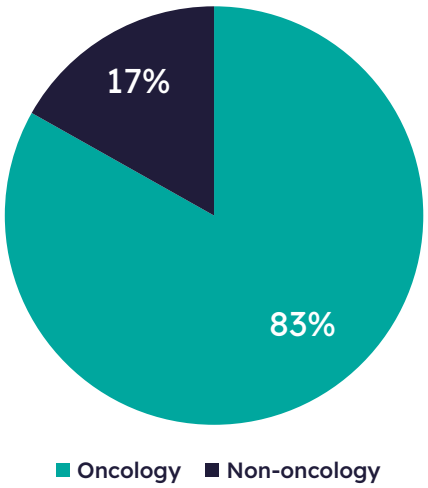
GENE THERAPY BREAKDOWN: CAR-TS CONTINUE TO DOMINATE THE PIPELINE

- CAR-T cell therapies remained the most common technology used in the pipeline of genetically modified cell therapies (preclinical through to pre-registration), representing 55%, followed by the "other" category at 25%, which includes a list of less commonly used technologies such as TCR-NK, CAR-M, and TAC-T
- 83% 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

Genetically modified cell therapy breakdown



CAR-T breakdown

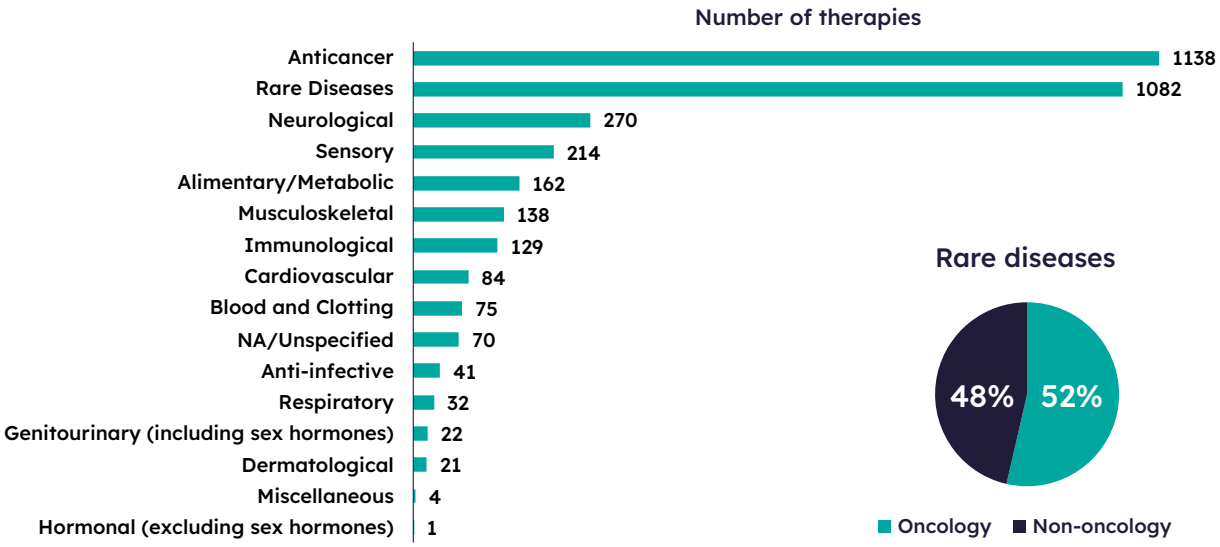


Source: Cell and Gene Therapy dashboard | Citeline, July 2025

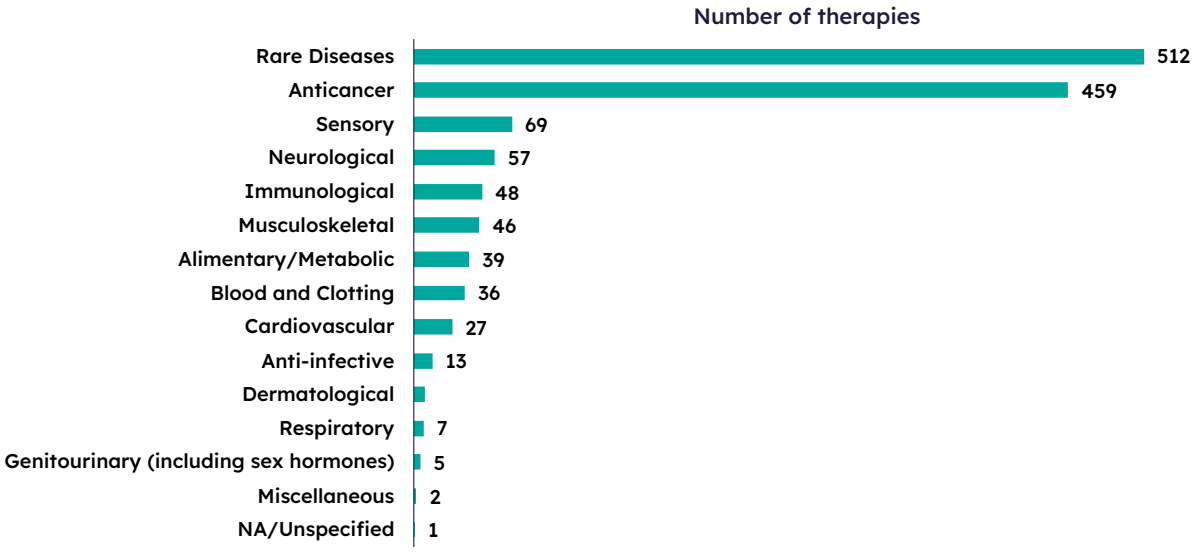
GENE THERAPY PIPELINE: MOST COMMONLY TARGETED THERAPEUTIC AREAS

- 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, the same proportion as the previous quarter

Number of therapies from preclinical through pre-registration



Therapies in the clinic (excludes preclinical development)

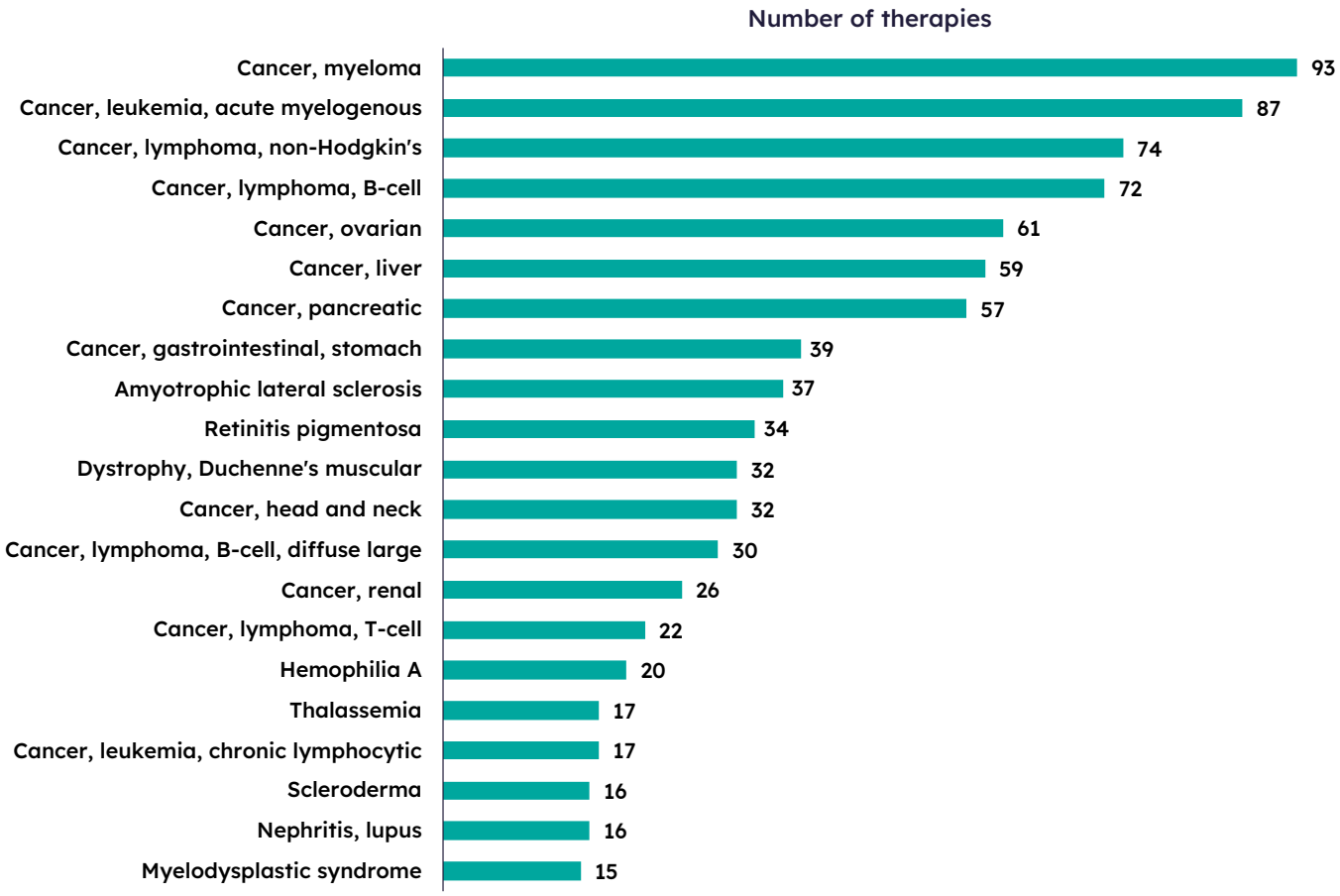


Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

GENE THERAPY PIPELINE: MOST COMMON RARE DISEASES TARGETED

- For the 1,464 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
- Ovarian cancer marks a new addition to the top five rare diseases for which gene therapies are being developed:
  - Myeloma
  - Acute myelogenous leukemia
  - Non-Hodgkin's lymphoma
  - B-cell lymphoma
  - Ovarian cancer



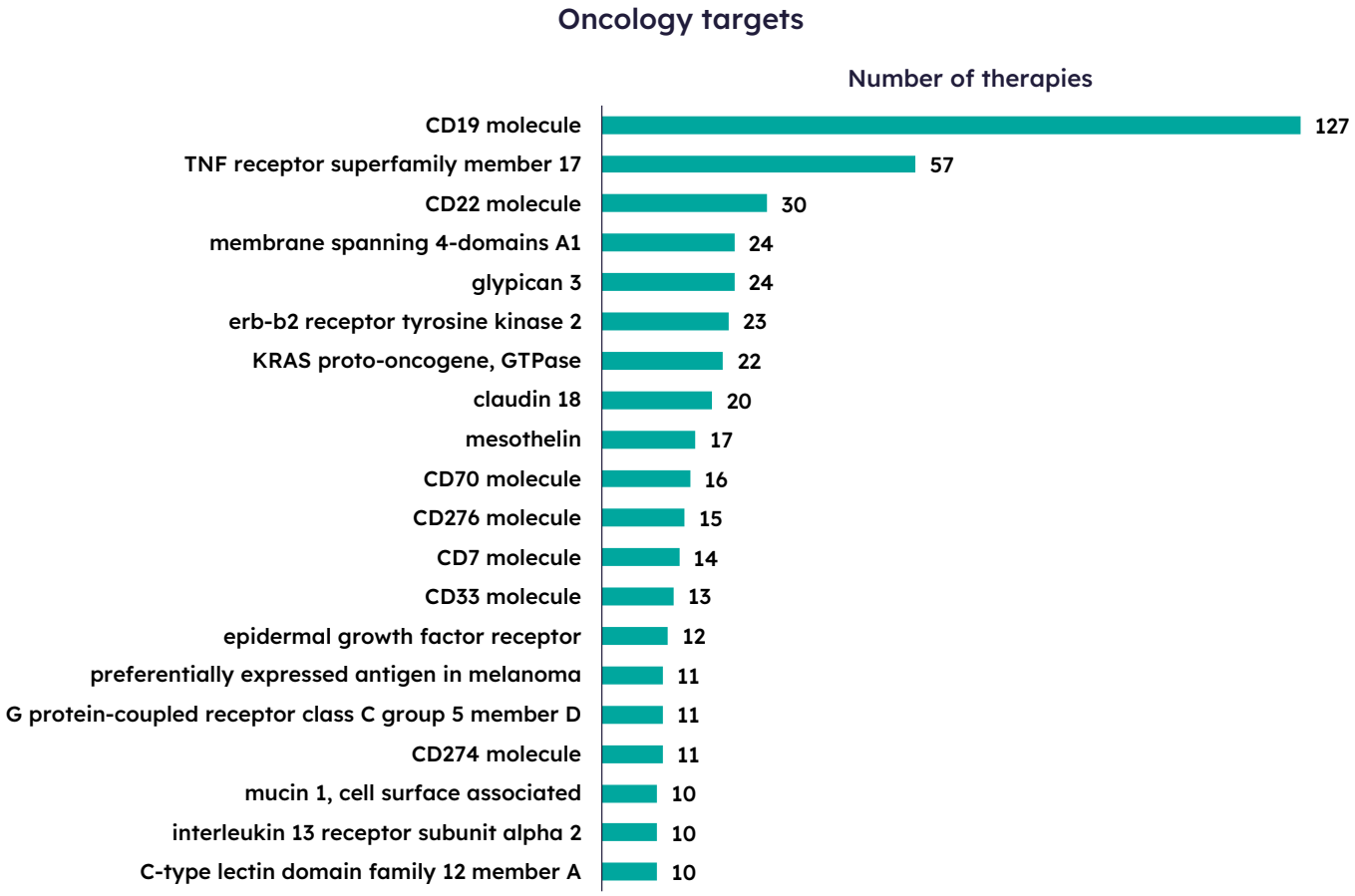
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

GENE THERAPY PIPELINE: MOST COMMON TARGETS

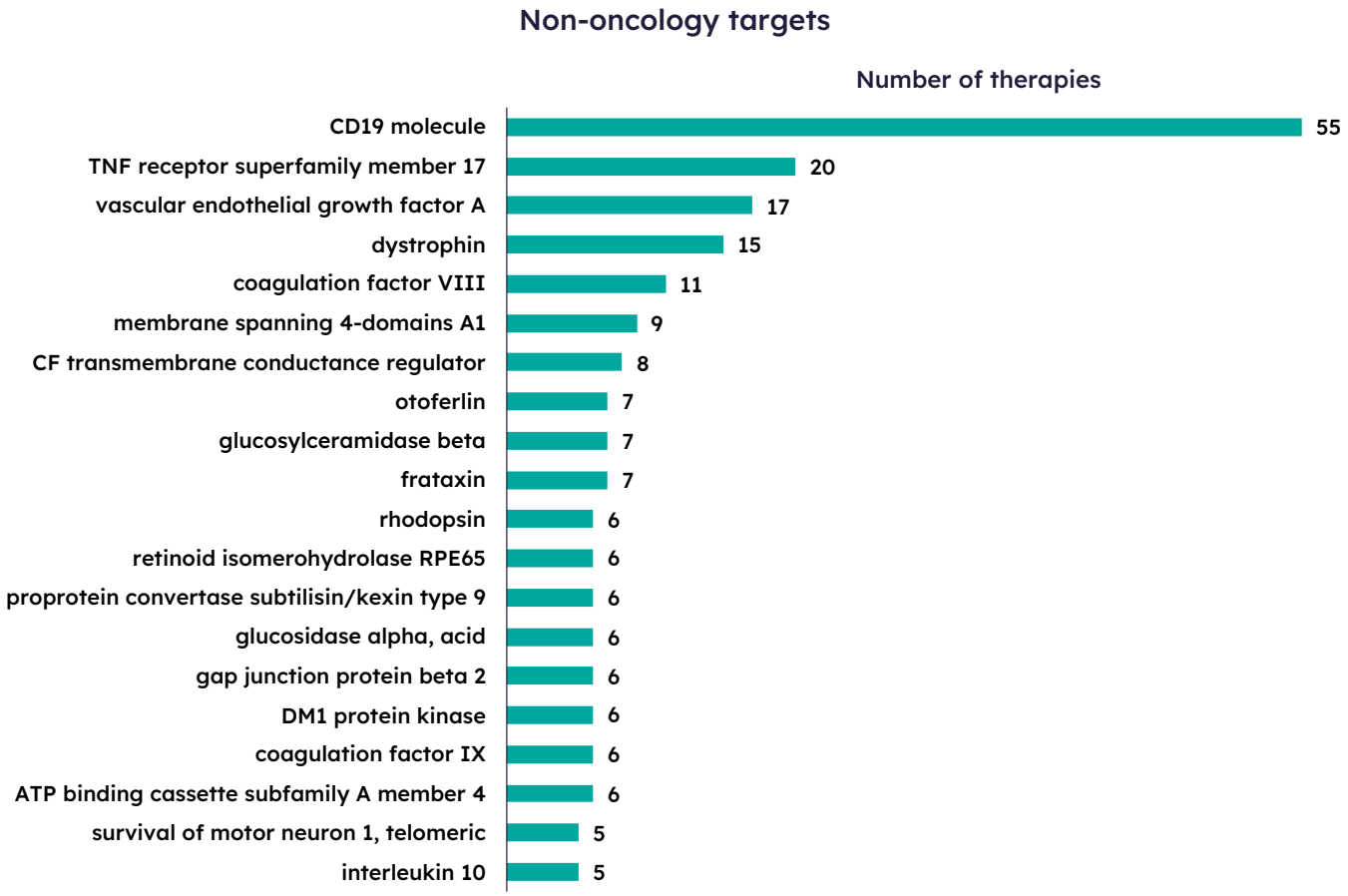
Of the gene therapies at preclinical through pre-registration for which targets were disclosed:

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



Source: Pharmaprojects | Citeline, July 2025

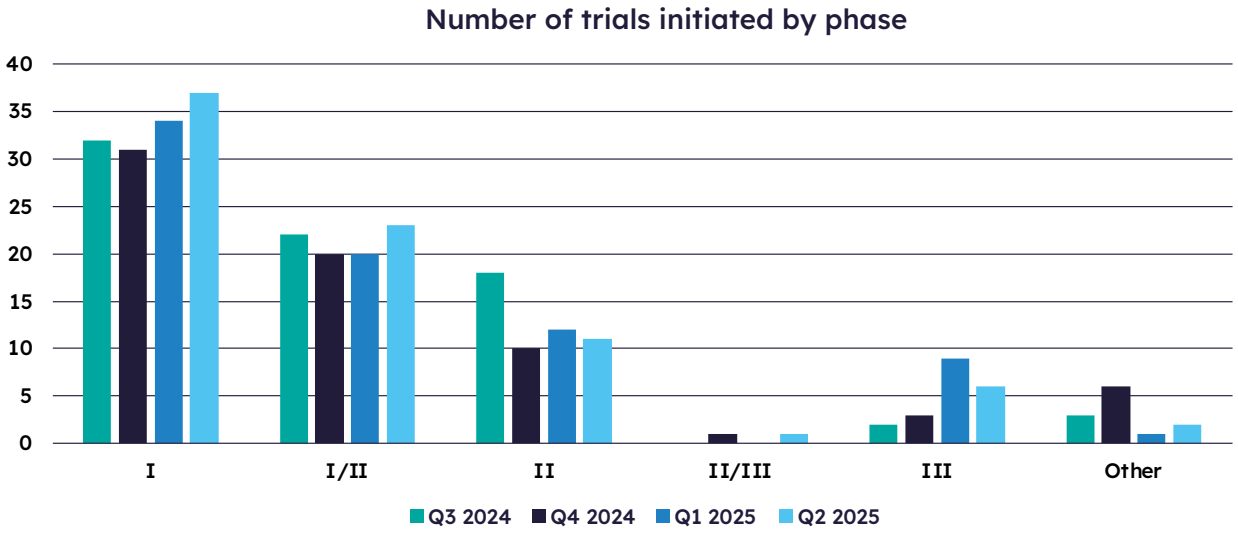
GENE THERAPY PIPELINE: MOST COMMON TARGETS Continued

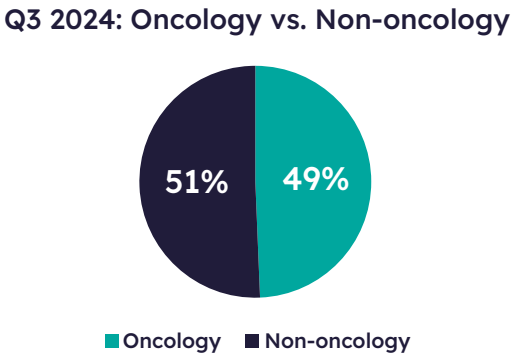


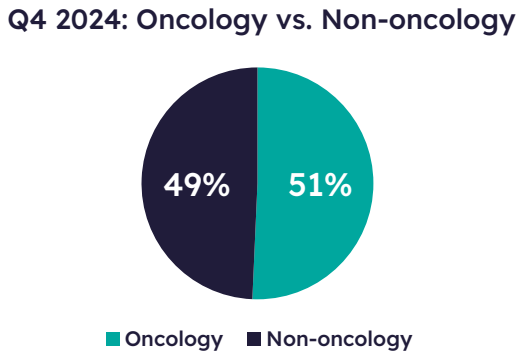
Source: Pharmaprojects | Citeline, July 2025

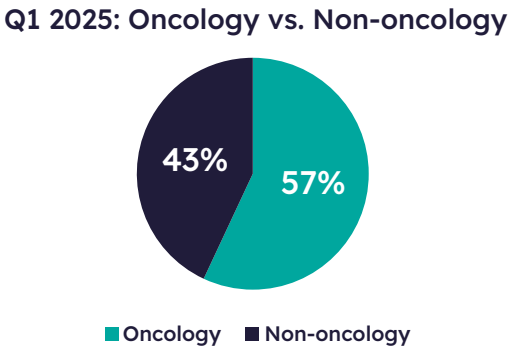
GENE THERAPY CLINICAL TRIAL ACTIVITY

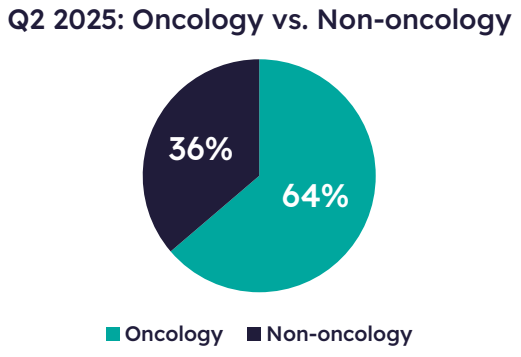
- The proportion of gene therapy trials for non-oncology indications decreased for the third quarter in a row to 36%, while the proportion of gene therapy trials for oncology indications is the highest for the past year
- 80 gene therapy trials were initiated in Q2 2025, one more than the previous quarter











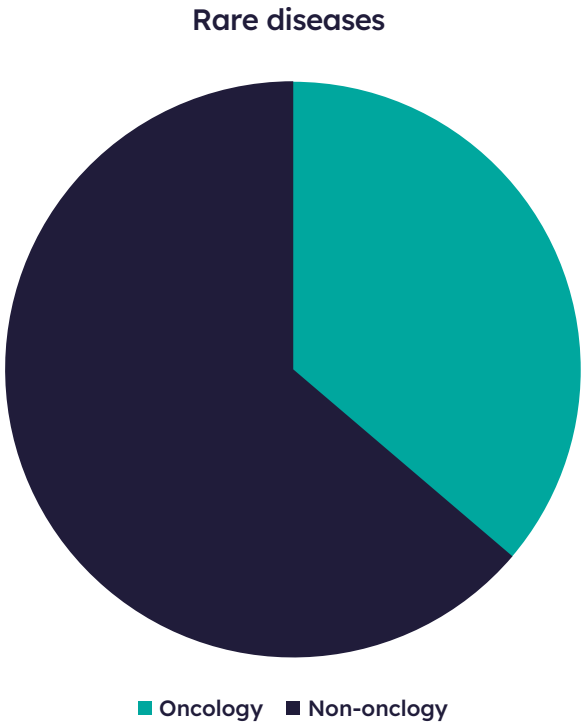
Source: Trialtrove | Citeline, July 2025

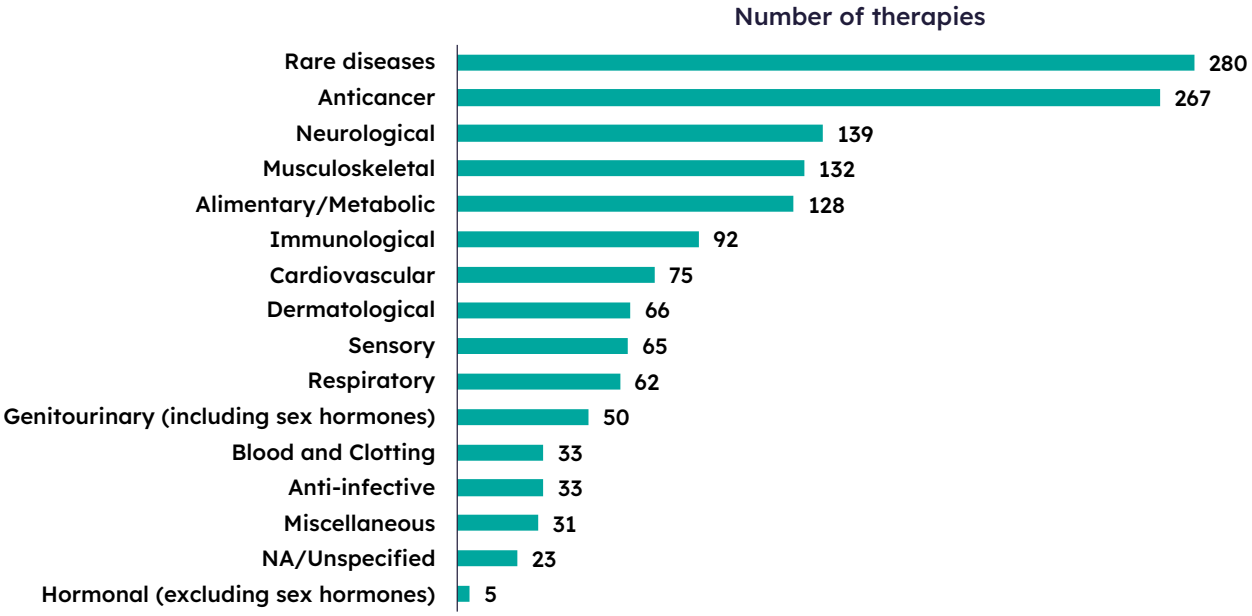
Non-genetically modified cell therapy pipeline

NON-GENETICALLY MODIFIED CELL THERAPY PIPELINE: MOST COMMONLY TARGETED THERAPEUTIC AREAS

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

- Oncology and rare diseases remained the top areas of non-genetically modified cell therapy development
- Of the non-genetically modified cell therapies in preclinical to pre-registration stages for rare diseases, 64% were in development for non-oncology rare diseases, a two-percentage-point increase from the previous quarter





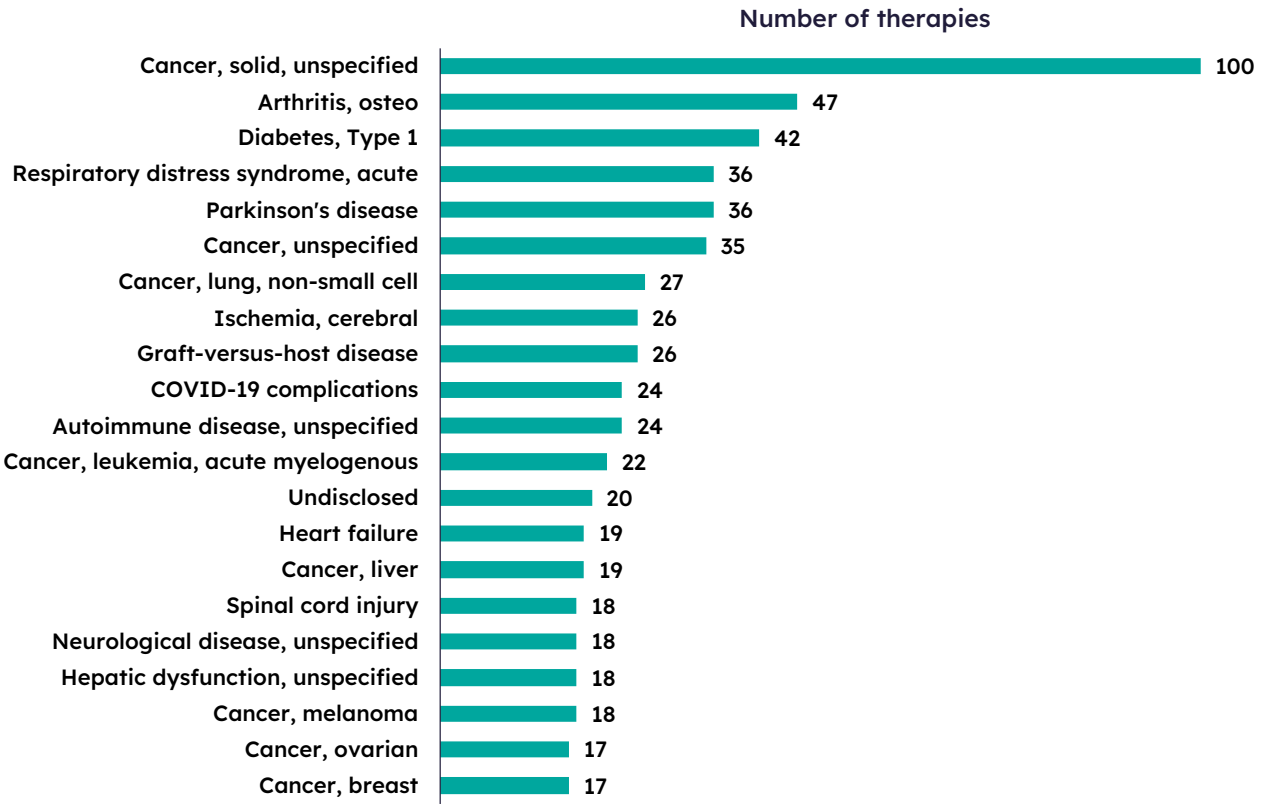
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

NON-GENETICALLY MODIFIED CELL THERAPY PIPELINE: MOST COMMON DISEASES TARGETED

Of the therapies for which indications are specified, the most targeted indications in Q2 2025 were:

- Osteoarthritis
- Type 1 diabetes
- Acute respiratory distress syndrome (with a count of pipeline assets equal to Parkinson's disease)



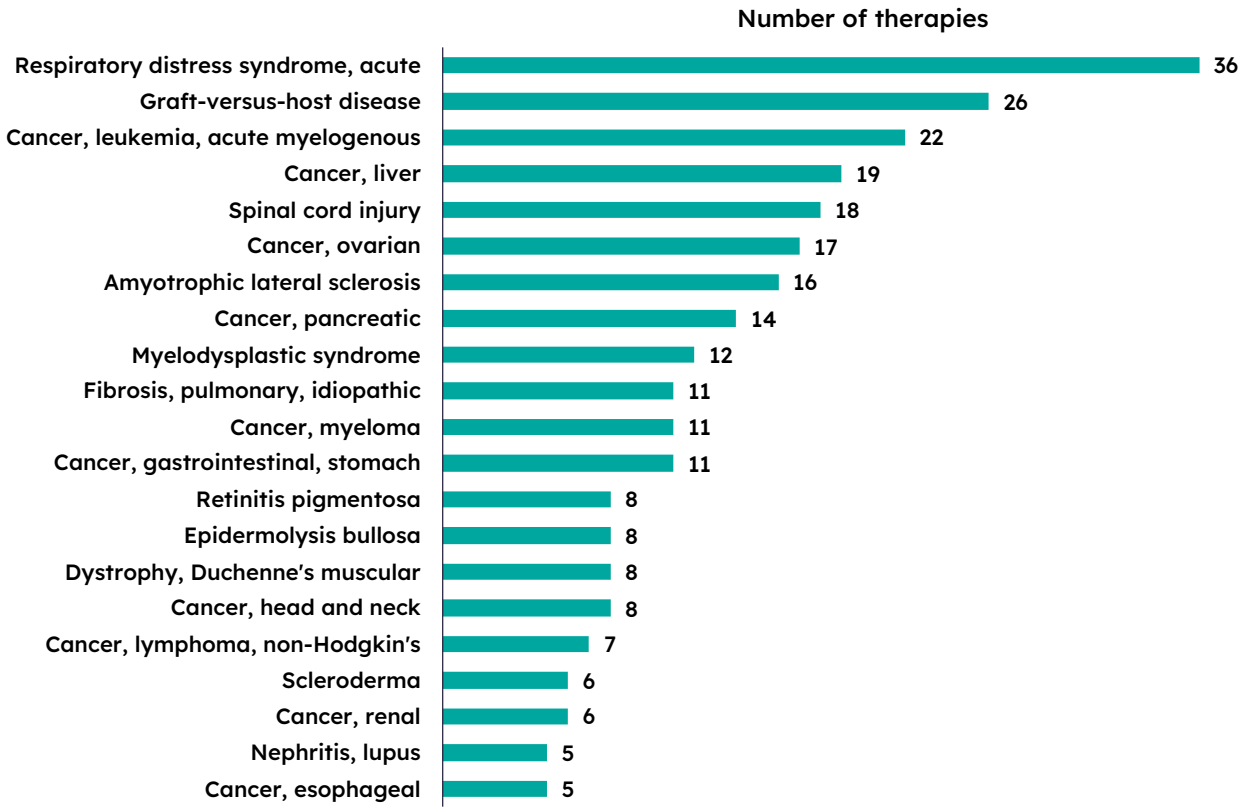
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

NON-GENETICALLY MODIFIED CELL THERAPY PIPELINE: MOST COMMON RARE DISEASES TARGETED

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

- The top three oncology indications were acute myelogenous leukemia, liver cancer, and ovarian cancer
- The top three non-oncology indications were acute respiratory distress syndrome, graft-versus-host disease, and spinal cord injury

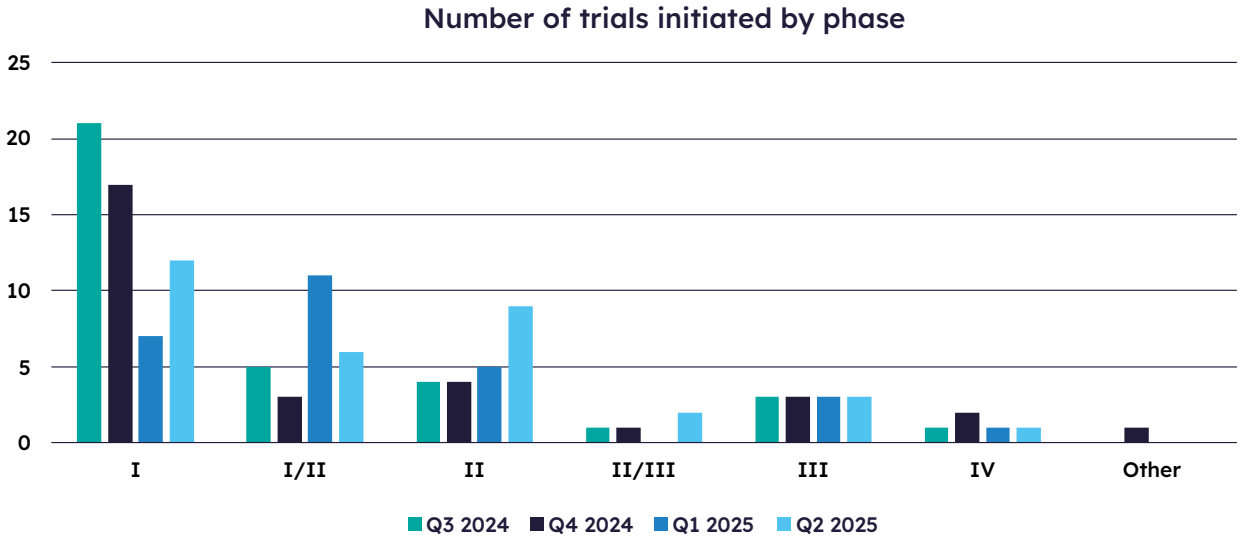


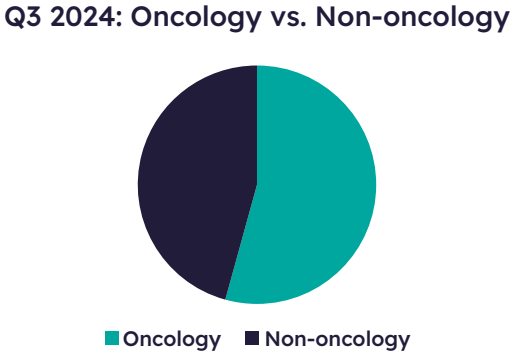
Note: Figures based on indications in pipeline development only for each therapy

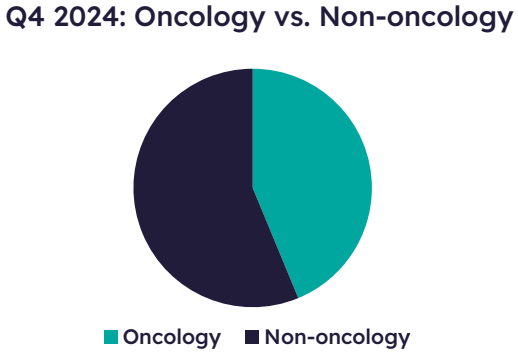
Source: Pharmaprojects | Citeline, July 2025

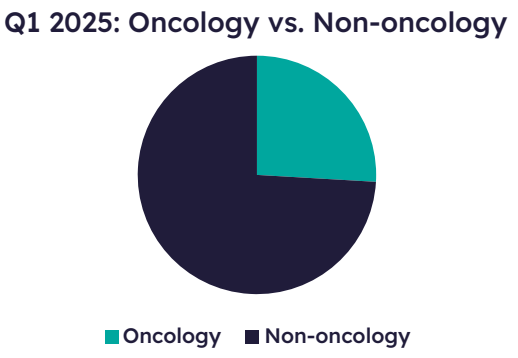
NON-GENETICALLY MODIFIED CELL THERAPY TRIAL ACTIVITY

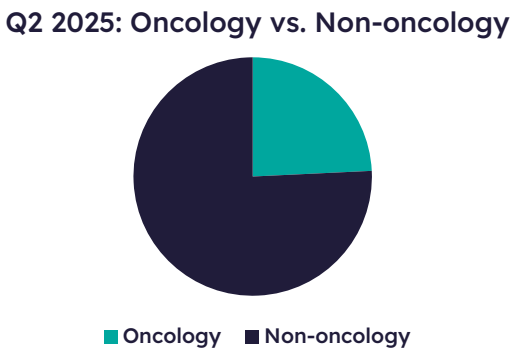
- 33 trials were initiated for non-genetically modified cell therapies in Q2 2025, six more than in Q1 2025
- Of these 33, 76% were for non-oncology indications, two percentage points higher than the previous quarter









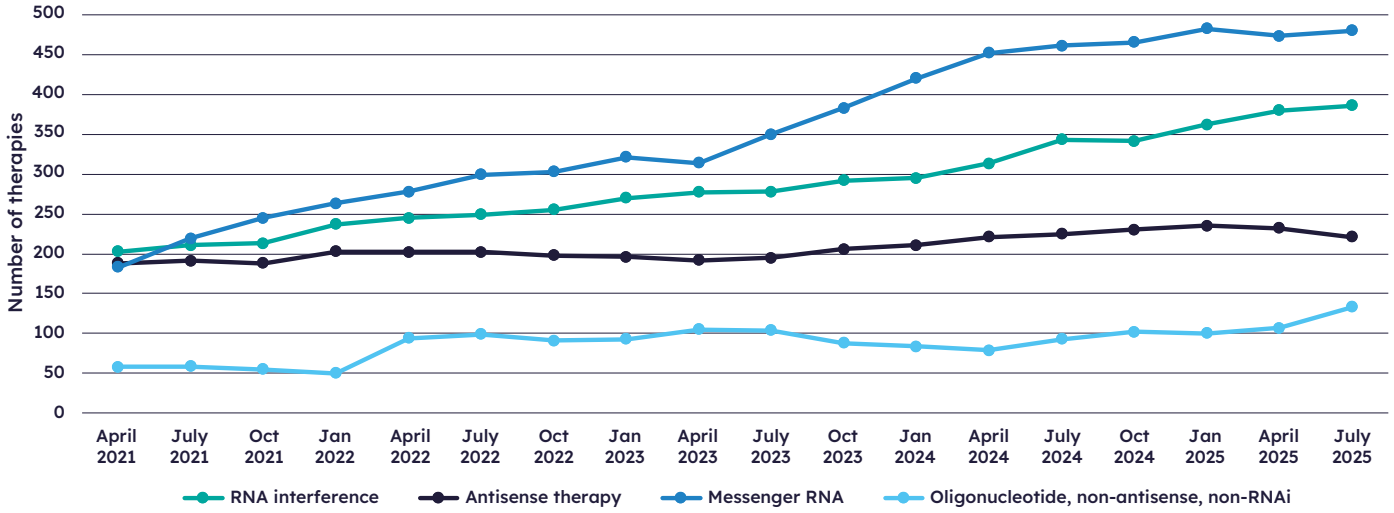


Source: Trialtrove | Citeline, July 2025

RNA therapy pipeline

RNA THERAPY PIPELINE: MOST COMMON MODALITIES

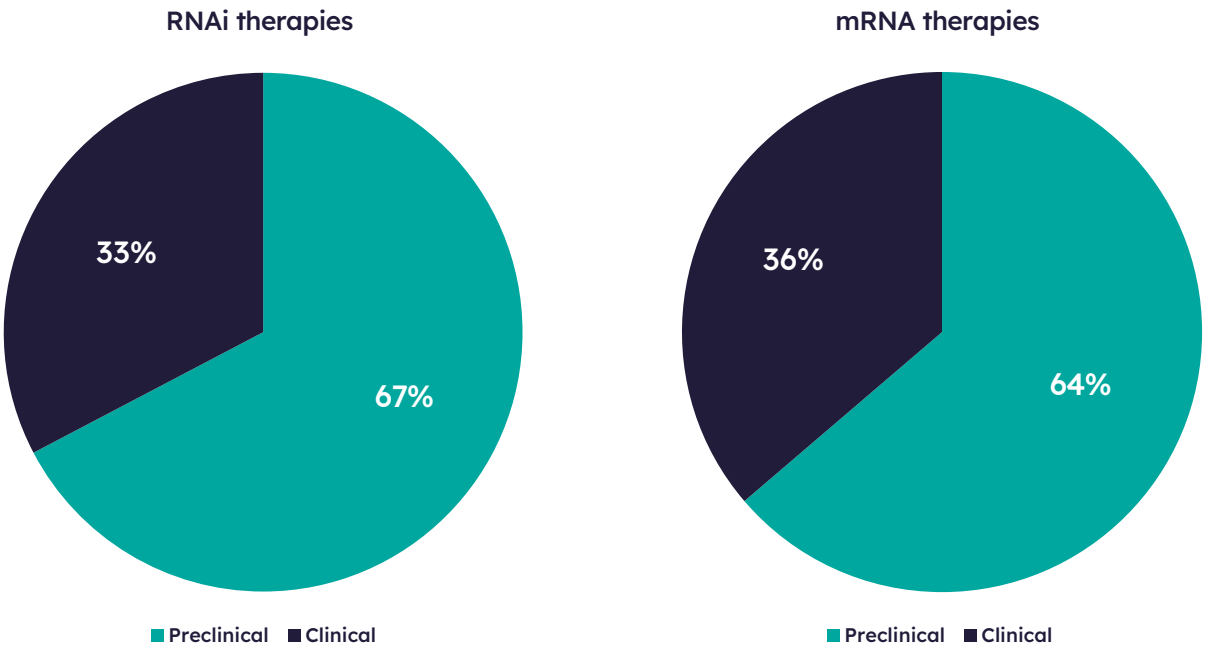
- Of RNA therapies in the pipeline, messenger RNA (mRNA) and RNA interference (RNAi) continued to be the preferred RNA modalities for research

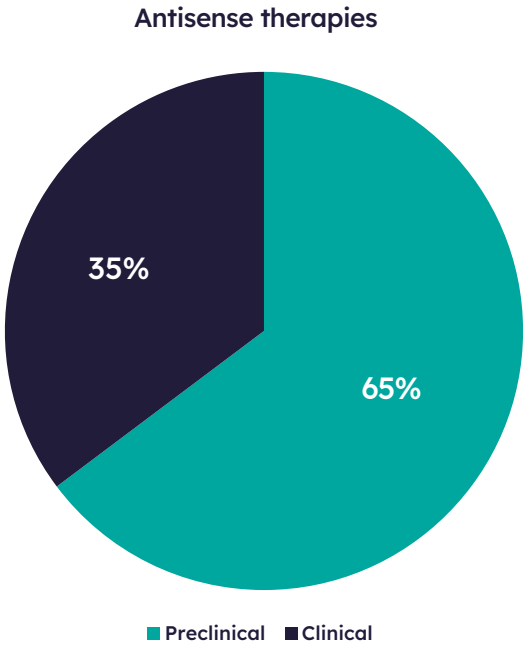


Source: Pharmaprojects | Citeline, July 2025

RNAI, MRNA, AND ANTISENSE OLIGONUCLEOTIDES: PRECLINICAL VS. CLINICAL

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 67%, 64%, and 65% of their respective pipelines



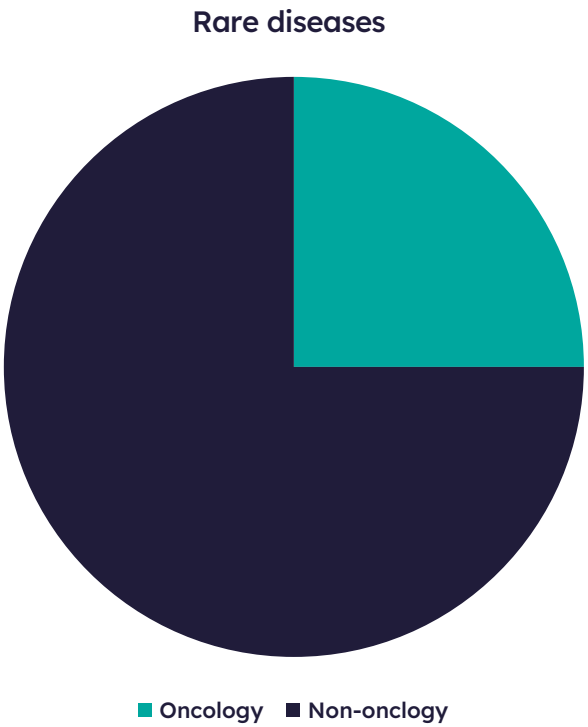


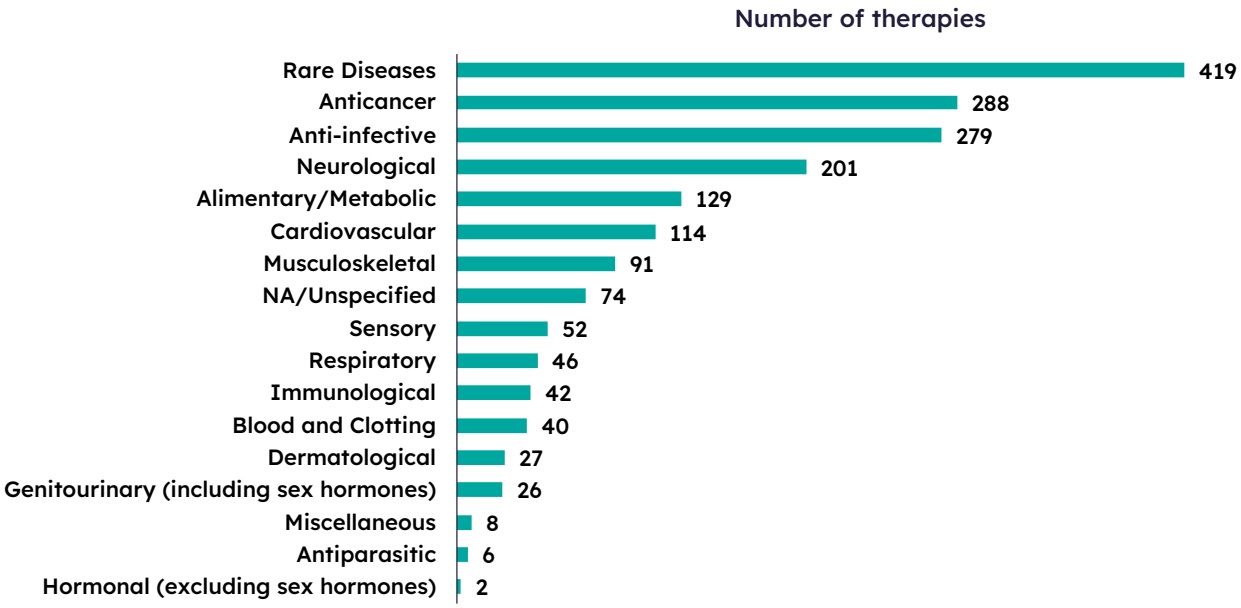
Source: Pharmaprojects | Citeline, July 2025

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%





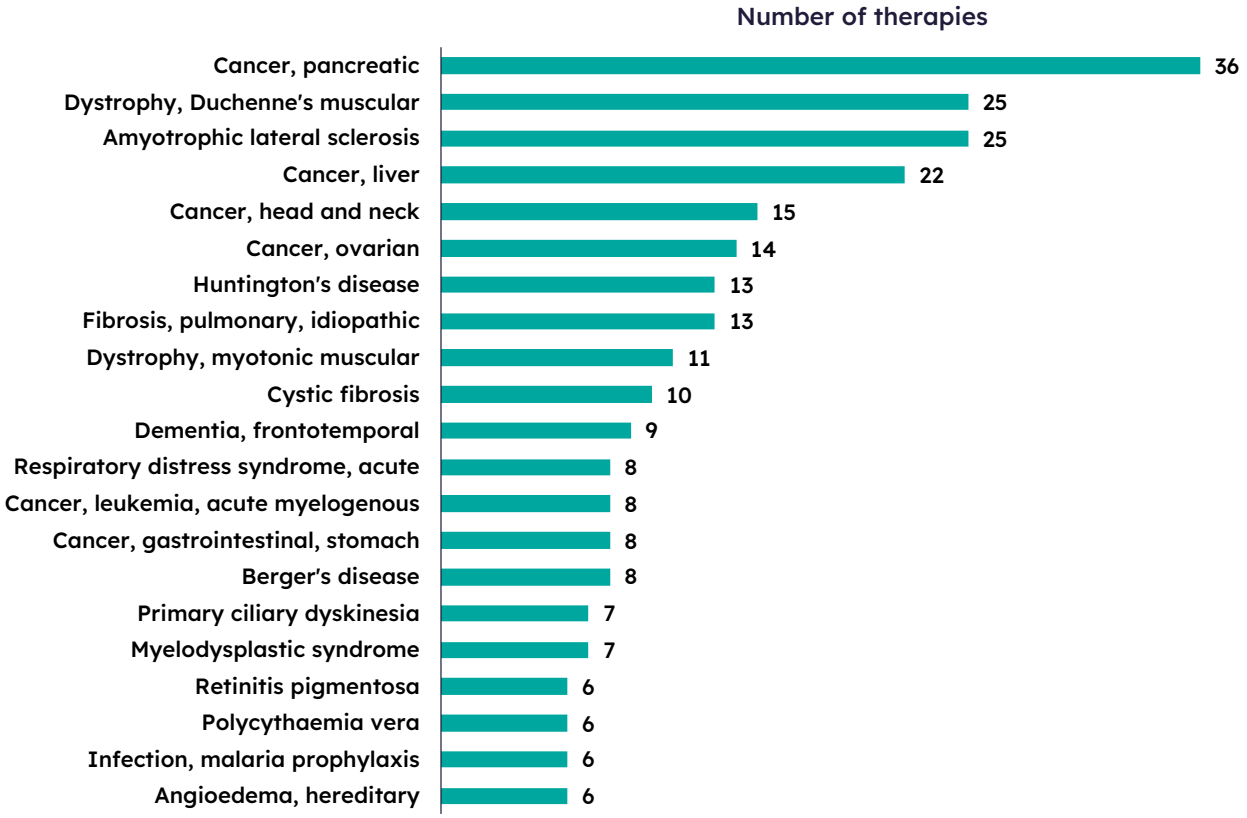
Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

RNA THERAPIES: MOST COMMON RARE DISEASES TARGETED

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

- Top specified rare oncology indications were pancreatic, liver, and head and neck cancer
- For non-oncology rare diseases, Duchenne muscular dystrophy, amyotrophic lateral sclerosis, and Huntington's disease were the most targeted indications

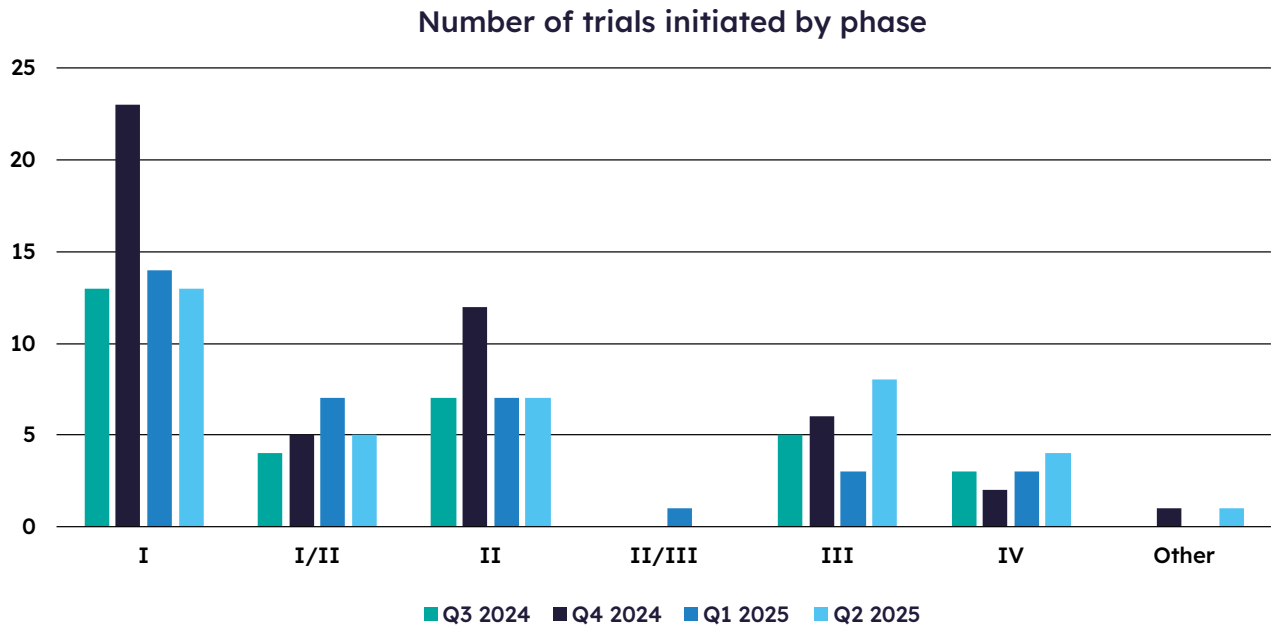


Note: Figures based on indications in pipeline development only for each therapy

Source: Pharmaprojects | Citeline, July 2025

RNA THERAPY PIPELINE: CLINICAL TRIAL ACTIVITY

- 38 RNA trials were initiated in Q2 2025, compared to 35 in Q1 2025, 74% of which were for non-oncology indications



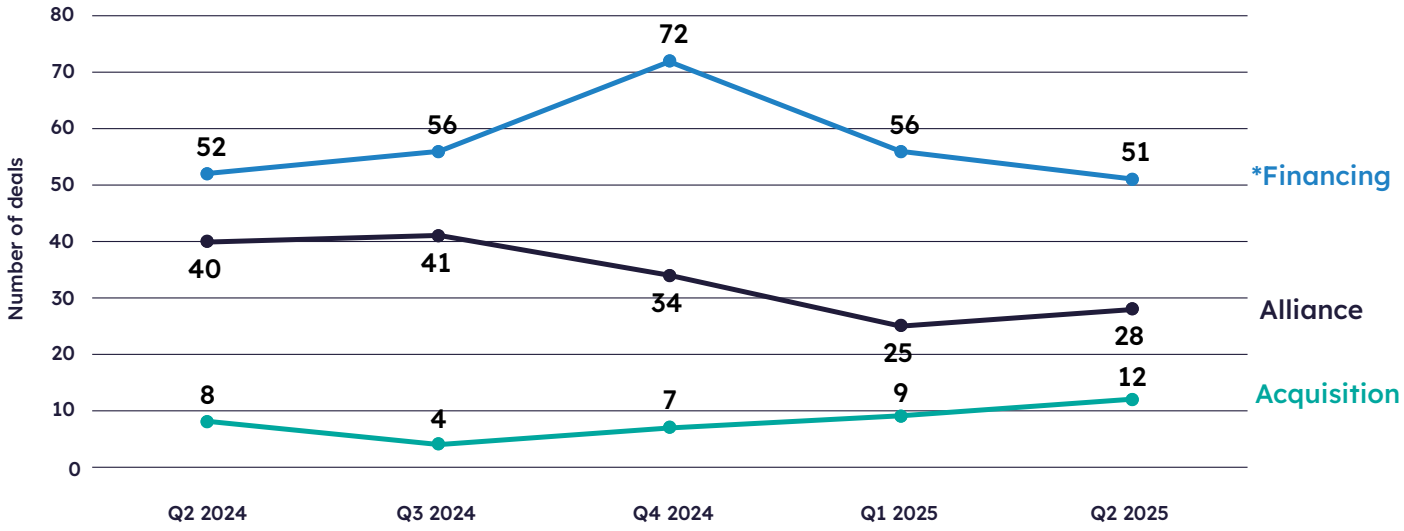
Source: Trialtrove | Citeline, October 2024

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

Total number of deals by type, most recent five quarters



*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, July 2025

Q3 2024 ACQUISITIONS IN GENE, CELL, AND RNA THERAPY

- Acquisitions of advanced molecular therapy players continue to increase quarter by quarter, with a 33% jump in volume in Q2 2025 from nine to 12 transactions
- Big pharma was involved in three billion-dollar takeovers: AbbVie paid $2.1 billion for Capstan (in vivo CAR-T); Eli Lilly bought Verve (gene editing) for $1.3 billion; and Novartis spent $1.7 billion on Regulus (microRNA therapy)
- In another billion-dollar deal, BioNTech acquired CureVac (mRNA cancer immunotherapy) for $1.25 billion

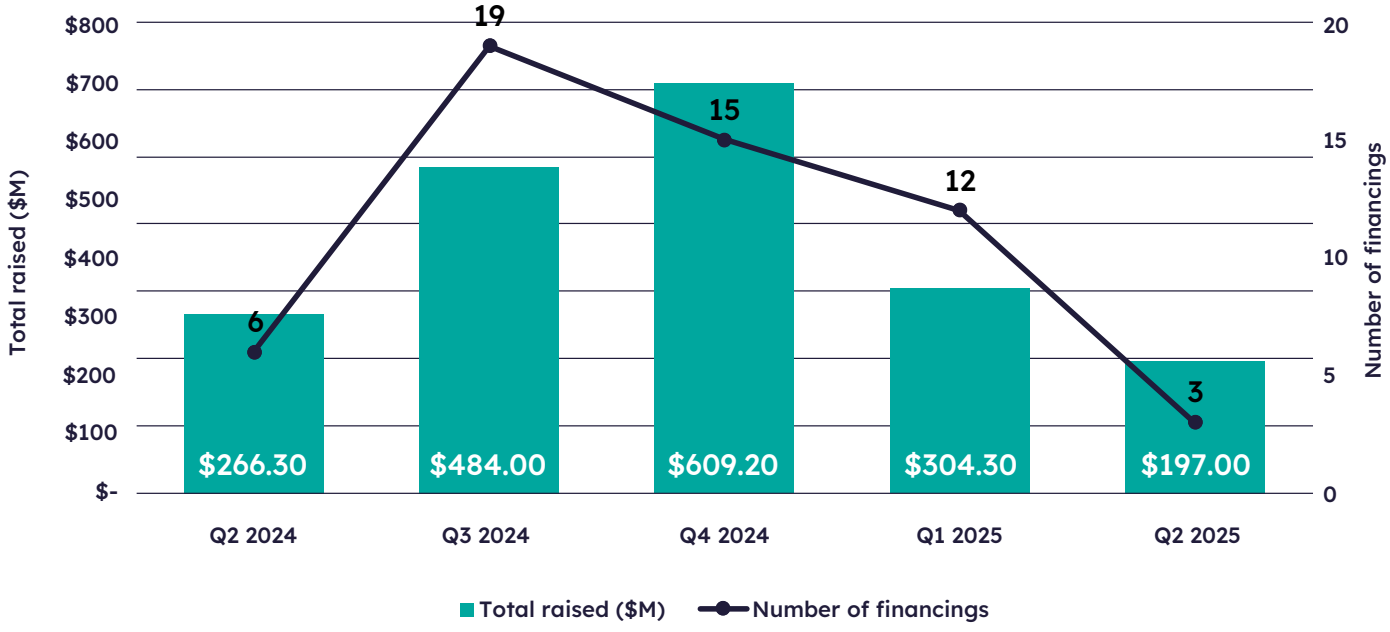
Deal date	Deal title	Potential deal value (USD $)
April 2, 2025	Artis BioSolutions Emerges from Stealth, Announces Acquisition of Landmark Bio	Undisclosed
April 14, 2025	NAYA Biosciences to Separate Fertility and Oncology Businesses into Distinct Operations; Separation Complete	Undisclosed
April 25, 2025	Coeptis Therapeutics to Spin Out Biopharma Operations into NewCo Concurrent with Z Squared Merger	Undisclosed
April 29, 2025	AlphaRose Therapeutics Acquires Alpha Anomeric SA	Undisclosed
April 30, 2025	Novartis to acquire Regulus Therapeutics	1,700,000,000
May 28, 2025	TQ Therapeutics Acquires German Subsidiary from BMS's Juno	Undisclosed
June 2, 2025	STEMCELL Technologies Acquires Cellular Highways	Undisclosed
June 12, 2025	BioNTech Enters Agreement to Acquire CureVac in Public Exchange Offer	1,250,000,000
June 17, 2025	Lilly to Acquire Verve Therapeutics for up to $1.3B Including CVRs	1,300,000,000
June 23, 2025	Carisma Therapeutics to Merge with Ocugen's OrthoCellix Division in All-Stock Deal	Undisclosed
June 27, 2025	Turnstone Biologics to be Acquired by XOMA	Undisclosed
June 30, 2025	AbbVie to Acquire Capstan Therapeutics	2,100,000,000

Sources: Biomedtracker, BioSciDB, July 2025

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

- Series A and seed volume and value continue their quarter-by-quarter decline, with a total of $197 million raised in Q2 2025 from three start-ups
- Q2's figures represent 75% and 35% decreases in volume and value, respectively, vs. the previous quarter's 12 financings reaching $304.3 million
- Q2 was also slightly behind the six start-up financings announced in the same quarter last year that together were worth $266.3 million

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



Source: Biomedtracker | Citeline, BioSciDB | Evaluate, October 2024

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)
08 May 2025	HAYA Therapeutics Gets $65M in Series A Round	Precision RNA guided therapies	Switzerland / Vaud	Lausanne University Hospital	65
13 May 2025	Somite AI Raises Over $47M in Series A Round	Cell therapy	United States / Massachusetts / Boston	MIT; Brigham and Women's Hospital; Harvard Medical School; University of Washington	47
13 May 2025	Stylus Medicine Launches With $45M Series A Extension Round, Bringing Total Round to $85M	CAR-T therapy	United States / Massachusetts / Cambridge	UC Berkeley; Stanford University	85

Source: Biomedtracker | Evaluate, July 2025

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
STYLUS	Uses engineered recombinases with affinity for human genes, combined with cell-targeted lipid nanoparticles that deliver in vivo CAR-T therapies	UC Berkeley; Stanford University	$85M/Series A	Lead undisclosed; investors included RA Capital Management, Khosla Ventures, Chugai Venture Fund, Eli Lilly, and Johnson & Johnson Innovation – JJDC	Oncology, autoimmune, and genetic diseases
HAYA Therapeutics	Disease-modifying therapeutics targeting long noncoding RNA	Lausanne University Hospital	$65M/Series A	Sofinnova Partners and Earlybird Venture Capital	Oncology, cardiology, respiratory, hepatic, and renal
SOMITE	DeltaStem AI foundation model platform to optimize cell therapy in human diseases	MIT; Brigham and Women's Hospital; Harvard Medical School; University of Washington	$47M/Series A	Khosla Ventures	Metabolic, orthopedic, musculoskeletal, and hematological diseases

Source: Biomedtracker | Evaluate, July 2025

Upcoming catalysts

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

Therapy	Generic name	Disease	Catalyst	Catalyst date
RP-1	vusolimogene oderparepvec	Melanoma	PDUFA for NDA - First Review	July 22, 2025
Aucatzyl	obecabtagene autoleucel	Acute Lymphoblastic Leukemia (ALL)	Approval Decision (Europe)	May 22, 2025 – July 28, 2025
UX111	rebisufligene etisparvovec	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A Syndrome)	PDUFA for BLA - First Review	Aug. 18, 2025
Donidalorsen	donidalorsen	Hereditary Angioedema (HAE)	Approval Decision (U.S.)	Aug. 21, 2025
PRGN-2012	zopapogene imadenovec	Respiratory Papillomatosis (RP)	PDUFA for BLA - First Review	Aug. 27, 2025
Deramiocel		Duchenne Muscular Dystrophy (DMD)	PDUFA for BLA - First Review	Aug. 31, 2025
UM171	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	June 19, 2025 – Aug. 31, 2025
Elevidys	delandistrogene moxeparvovec	Duchenne Muscular Dystrophy (DMD)	CHMP European Panel Results	Feb. 1, 2025 – Aug. 31, 2025
RP-L102		Fanconi Anemia	Approval Decision (Europe)	March 2, 2025 – Sept. 2, 2025
RP-L102		Fanconi Anemia	CHMP Opinion	June 30, 2025 – Sept. 30, 2025
Amtagvi	lifileucel	Melanoma	CHMP Opinion	March 1, 2025 – Sept. 30, 2025
Beqvez	fidanacogene elaparvovec	Hemophilia B	Approval Decision (Japan)	March 1, 2025 – Sept. 30, 2025
Elevidys	delandistrogene moxeparvovec	Duchenne Muscular Dystrophy (DMD)	Approval Decision (Europe)	April 1, 2025 – Oct. 31, 2025
Amtagvi	lifileucel	Melanoma	Approval Decision (Europe)	May 1, 2025 – Nov. 30, 2025
Tryngolza		Familial Chylomicronemia Syndrome (FCS)/ Lipoprotein Lipase Deficiency (LPLD)	Approval Decision (Europe)	July 1, 2025 – Dec. 31, 2025
BT524	fibrinogen	Hemostasis	PDUFA for BLA - 1st Review	Jan. 9, 2025 – Dec. 31, 2025
Kresladi	marnetegragene autotemcel	Autoimmune Disorders	PDUFA Decision	Jan. 1, 2025 – Dec. 31, 2025
OST-HER2		Osteosarcoma	Product Approval (US)	April 22, 2025 – Dec. 31, 2025
Tryngolza		Familial Chylomicronemia Syndrome (FCS)/ Lipoprotein Lipase Deficiency (LPLD)	CHMP Opinion	Aug. 1, 2025 – Feb. 28, 2026
Izervay	avacincaptad pegol	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Conditional Approval Decision (Japan)	Aug. 1, 2025 – Feb. 28, 2026
MT1621	doxribtimine + doxecitine	Metabolic - General	CHMP Opinion	Sept. 1, 2025 - March 31, 2026
Nex-z	ziclumeran	Hereditary Transthyretin (hATTR) Amyloidosis with Polyneuropathy (Familial Amyloid Polyneuropathy)	BLA Filing	April 3, 2025 – Dec. 31, 2028

Source: Biomedtracker | Evaluate, July 2025

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

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

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

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

Devin Rose
Communications Manager
American Society of Gene + Cell Therapy

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American Society of Gene + Cell Therapy

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.

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

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Contact: clientservices@citeline.com

Contact: info@evaluate.com