Gene, Cell, & RNA Therapy Landscape Report

Q3 2024 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.

Citeline, a Norstella company, powers a full suite of complementary business intelligence offerings to meet the evolving needs of life science professionals to accelerate the connection of treatments to patients and patients to treatments. These patient-focused solutions and services deliver and analyze data used to drive clinical, commercial, and regulatory-related decisions and create real-world opportunities for growth.

Our global teams of analysts, journalists, and consultants keep their fingers on the pulse of the pharmaceutical, biomedical, and medtech industries, covering it all with expert insights: key diseases, clinical trials, drug R&D and approvals, market forecasts, and more. For more information on one of the world's most trusted life science partners, visit Citeline.

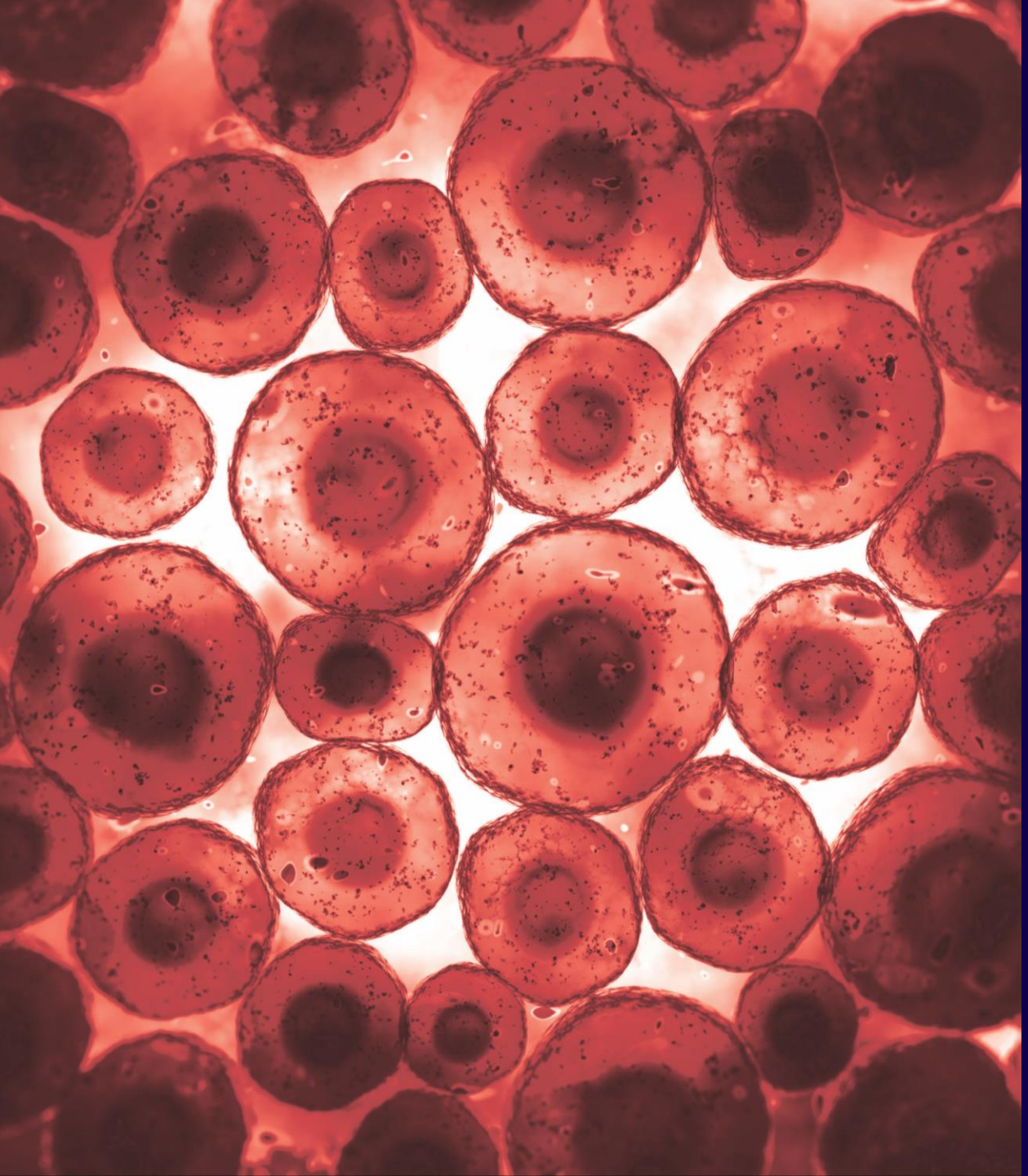

Table of contents

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Introduction

Q3 2024 marks a potential pivot in the evolution of cell, gene, and RNA therapies. While we saw only one new approval (Tecelra for synovial sarcoma), there are dynamic shifts deeper in the pipeline. The field is steadily diversifying; 51% of newly initiated gene therapy trials are now for non-oncology indications, up from just 39% year-over-year. The maturation of clinical research in these therapeutic areas evidences a progression of the field, which we expect to accelerate.

Dealmaking held steady at 101 transactions, but we're reporting a substantial rebound in start-up financing. The tripling of seed and Series A rounds to $484 million signals a resurgence of confidence in next-generation CGT technologies. Coupled with strategic CDMO acquisitions, we're watching this field mature in real-time, and it's primed for breakthrough innovations.

At ASGCT, we're not just observers but catalysts in this transformation. The Society and its members are actively working to expand the application of these therapies, foster cross-disciplinary collaboration, and shape policies that will accelerate development. The future of medicine is here, and we're proud to be at the forefront, driving meaningful connections and progress that will redefine patient care across a spectrum of diseases.

Thank you,
David Barrett, JD
CEO, ASGCT

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Key takeaways from Q3 2024

One new approval across the cell, gene, and RNA landscape in Q3 2024

- Adaptimmune's Tecelra (afamitresgene autoleucel) was approved by the US FDA for the treatment of synovial sarcoma. Tecelra is a melanoma-associated antigen A4 (MAGE-A4)-directed genetically modified autologous T-cell immunotherapy

Initiation of gene therapy clinical trials shows continued shift towards non-oncology research

- Across the past year there has been a consistent trend of an increasing proportion of initiated trials each quarter that target non-oncology indications, rising from 39% of trials started in Q4 2023 to 51% of trials started in the past quarter
- When looking at pipeline drug development, however, the focus on oncology development remains clear, with 52% of all pipeline gene therapies targeting at least one oncology indication, and with 53% of rare disease pipeline gene therapy development focused on oncology indications

Despite overall dealmaking volume remaining flat, start-up financing rebounded

- Advanced molecular therapy companies signed a total of 101 acquisition, alliance, and financing transactions in Q3 2024, essentially flat from the 100 deals in the previous quarter
- Q3 saw four acquisitions, which was half the volume in Q2, with notable activity in the CDMO space including Agilent's $925m purchase of Biovectra
- The volume of seed and Series A financing tripled to 19 transactions in Q3 with an aggregate $484.0m, nearly a 2x increase over the $266.3m from Q2

Key highlights in Q3 2024

Q3 2024

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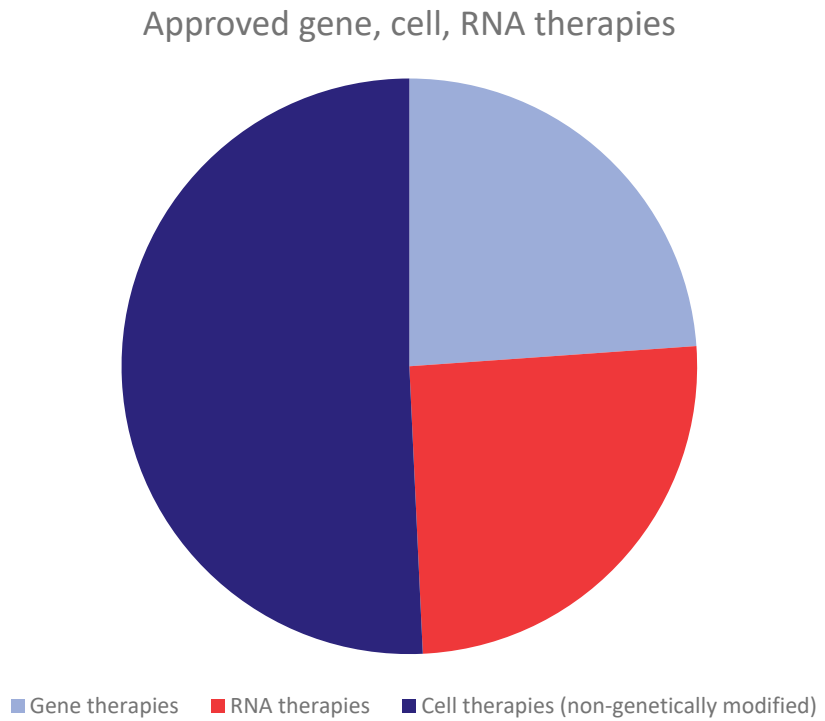
Approved gene, cell, and RNA therapies

Globally, for clinical use:

- 32 gene therapies have been approved (including genetically modified cell therapies)
  - Tecelra, an autologous T-cell immunotherapy, was approved by the US FDA for synovial sarcoma
- 34 RNA therapies have been approved
- 68 non-genetically modified cell therapies have been approved



Source: Pharmaprojects | Citeline, October 2024

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Approved gene therapies as of Q3 2024 (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, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Approved gene therapies as of Q3 2024 (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, Australia, Canada, China	Legend Biotech
Upstaza	eladocagene exuparvovec	2022	Aromatic L-amino acid decarboxylase (AADC) deficiency	EU, UK	PTC Therapeutics
Roctavian	valoctocogene roxaparvovec	2022	Hemophilia A	EU, US	BioMarin
Hemgenix	etranacogene dezaparvovec	2022	Hemophilia B	US, EU, UK, Canada, Switzerland	uniQure
Adstiladrin	nadofaragene firadenovec	2022	Bladder cancer	US	Merck & Co.
Elevidys	delandistrogene moxeparvovec	2023	Duchenne muscular dystrophy	US	Sarepta Therapeutics
Vyjuvek	beremagene geperpavec	2023	Dystrophic epidermolysis bullosa	US	Krystal Biotech
Fucaso	equecabtagene autoleucel	2023	Multiple myeloma	China	Nanjing IASO Biotechnology

Source: Pharmaprojects | Citeline, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Approved gene therapies as of Q3 2024 (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	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
Beqvez	fidanacogene elaparvovec	2024	Hemophilia B	Canada, US, EU	Pfizer
Tecelra	afamitresgene autoleucel	2024	Synovial sarcoma	US	Adaptimmune

Source: Pharmaprojects | Citeline, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Approved RNA therapies as of Q3 2024 (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, Canada, US, 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, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Approved RNA therapies as of Q3 2024 (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
Nulibry	fosdenopterin	2021	Molybdenum cofactor deficiency	US, EU, UK, Israel	Orphatec
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
Sinocelltech COVID-19 vaccine	COVID-19 alpha/beta/delta/Omicron variants S-trimer quadrivalent recombinant protein vaccine	2023	Infection, coronavirus, novel coronavirus prophylaxis	China, UAE, US	Sinocelltech

Source: Pharmaprojects | Citeline, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Approved RNA therapies as of Q3 2024 (3/3)

Product name	Generic name	Year first approved	Disease(s)	Locations approved*	Originator company
Izervay	avacincaptad pegol sodium	2023	Wet age-related macular degeneration	US	Archemix
Qalsody	tofersen	2023	Amyotrophic lateral sclerosis	US, EU	Ionis Pharmaceuticals
ARCT-154	COVID-19 mRNA vaccine, Arcturus	2023	Infection, coronavirus, novel coronavirus prophylaxis	Japan	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	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	Geron
mRESVIA	respiratory syncytial virus vaccine, Moderna Therapeutics	2024	Respiratory syncytial virus prophylaxis	US, EU	Moderna Therapeutics

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

Source: Pharmaprojects | Citeline, October 2024

Text highlighted in yellow represents new approvals during Q3 2024

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Key highlights in Q3 2024

Noteworthy events that happened in Q3 2024

Drug	Event Type	Indication	Molecule	Event Date
Lomecel-B	Regenerative Medicine Advanced Therapy (RMAT) Designation	Alzheimer's Disease (AD)	Cellular	10 July 2024
INO-3107	Innovative Licensing and Access Pathway (ILAP) (U.K.)	Head and Neck Cancer	Other Nucleic Acid	11 July 2024
Lomecel-B	Fast Track Status	Alzheimer's Disease (AD)	Cellular	17 July 2024
Ryoncil	NDA/BLA Accepted	Graft vs. Host Disease (GVHD) - Treatment	Cellular	23 July 2024
UCART22	Orphan Drug Designation (US)	Acute Lymphoblastic Leukemia (ALL)	Cellular	23 July 2024
Beqvez	Conditional Marketing Authorisation (Europe)	Hemophilia B	Viral Gene Therapy	24 July 2024
Elevidys	European Filing Accepted	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	25 July 2024
UCART22	Rare Pediatric Disease (RPD) Designation	Acute Lymphoblastic Leukemia (ALL)	Cellular	25 July 2024
AKUUGO	Approval (Japan)	Traumatic Brain Injury (TBI)	Cellular	31 July 2024
Obe-cel	Filing for Approval (UK)	Acute Lymphoblastic Leukemia (ALL)	Other Nucleic Acid	31 July 2024
Tecelra	Approval (US)	Synovial Sarcoma	Cellular	01 August 2024
Ixo-vec	Regenerative Medicine Advanced Therapy (RMAT) Designation	Wet Age-Related Macular Degeneration (Wet AMD) (Ophthalmology)	Viral Gene Therapy	01 August 2024
DB-OTO	Regenerative Medicine Advanced Therapy (RMAT) Designation	Otoferlin Gene-Mediated Hearing Loss	Viral Gene Therapy	01 August 2024
NGN-401	Regenerative Medicine Advanced Therapy (RMAT) Designation	Rett Syndrome	Viral Gene Therapy	07 August 2024
SRP-9003	Fast Track Status	Limb-Girdle Muscular Dystrophy (LGMD)	Viral Gene Therapy	07 August 2024
Elevidys	Priority Review (Japan)	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	14 August 2024
ATSN-201	Rare Pediatric Disease (RPD) Designation	X-Linked Retinoschisis	Viral Gene Therapy	14 August 2024
ATSN-201	Orphan Drug Designation (US)	X-Linked Retinoschisis	Viral Gene Therapy	21 August 2024
Carvykti	Approval (China)	Multiple Myeloma (MM)	Other Nucleic Acid	27 August 2024
Bepirovirsen	Sakigake Designation (Japan)	Hepatitis B (HBV) Treatment (Antiviral)	Antisense	28 August 2024
CB-012	Fast Track Status	Acute Myelogenous Leukemia (AML)	Cellular	03 September 2024
Plozasiran	Breakthrough Therapy Designation (US)	Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	siRNA/RNAi	10 September 2024
P-BCMA-ALLO1	Regenerative Medicine Advanced Therapy (RMAT) Designation	Multiple Myeloma (MM)	Cellular	16 September 2024
Collategene	Breakthrough Therapy Designation (US)	Peripheral Arterial Disease (PAD)	Non-Viral Gene Therapy	18 September 2024
AMT-191	Orphan Drug Designation (US)	Fabry's Disease	Viral Gene Therapy	19 September 2024
EXG34217	Rare Pediatric Disease (RPD) Designation	Aplastic Anemia	Non-Viral Gene Therapy	25 September 2024

Source: Biomedtracker | Citeline, October 2024

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Pipeline overview

Q3 2024

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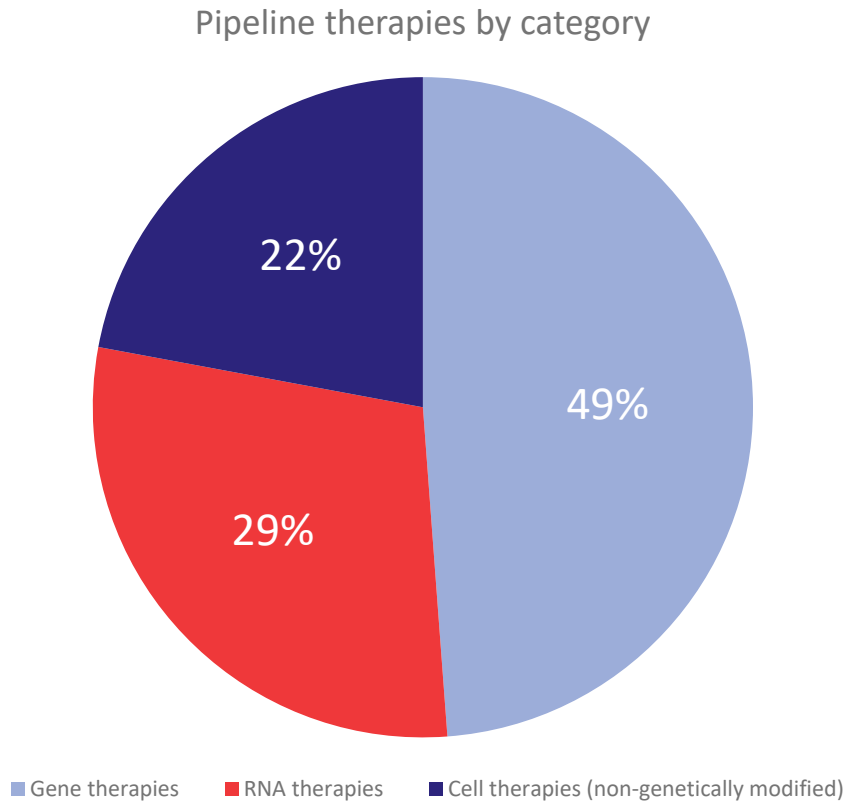
Pipeline of gene, cell, and RNA therapies

4,099 therapies are in development, ranging from preclinical through pre-registration

- 2,042 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
- 923 non-genetically modified cell therapies are in development, accounting for 22% of gene, cell, and RNA therapies



Source: Pharmaprojects | Citeline, October 2024

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Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Q3 2024

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Gene therapy pipeline: quarterly comparison

- The only pipeline stage to see a decline in gene therapy numbers was preclinical development, with a decrease of 3% since Q2 2024
- Three new filings were seen in Q3 2024, all in China
- Abeona's EB-101 received a complete response letter from the US FDA that outlined CMC-related issues. Resubmission is expected in H2 2024
- Therapies currently in pre-registration:
  - In the US
    - RP-L201 (Rocket Pharmaceuticals)
  - In the US and EU
    - obe-cel (Autolus Therapeutics)
  - In the EU
    - RP-L102 (Rocket Pharmaceuticals)
  - In China
    - BBM-H901 (Belief BioMed)
    - donaperminogene seltoplasmid (Helixmith)
    - pulkilumab (pCAR-19B) cells (Chongqing Precision Biotech)

Global Status	Q3 2023	Q4 2023	Q1 2024	Q2 2024	Q3 2024
Preclinical	1,522	1,528	1,471	1,436	1,393
Phase I	256	270	301	314	318
Phase II	267	274	282	279	289
Phase III	30	33	35	34	35
Pre-registration	7	6	4	5	6
Total	**2,082**	**2,111**	**2,093**	**2,068**	**2,041**

Sources: Pharmaprojects | Citeline, October 2024; Company presentation, Abeona Therapeutics, July 2024, slide 21, link

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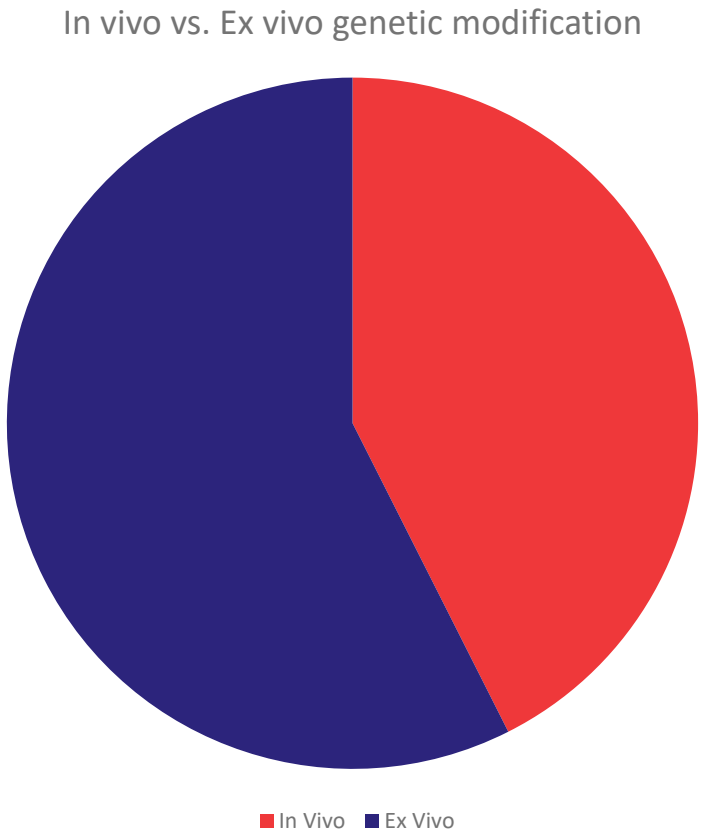
Genetic modification: *In vivo* vs. *Ex vivo*

- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q3 2024, *in vivo* delivery techniques were used in 43% of gene therapies



Source: Cell and Gene Therapy dashboard | Citeline, October 2024

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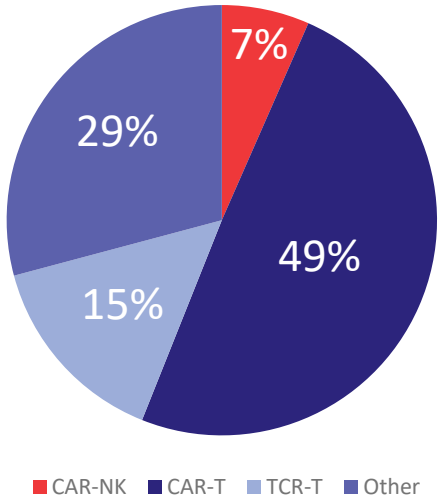
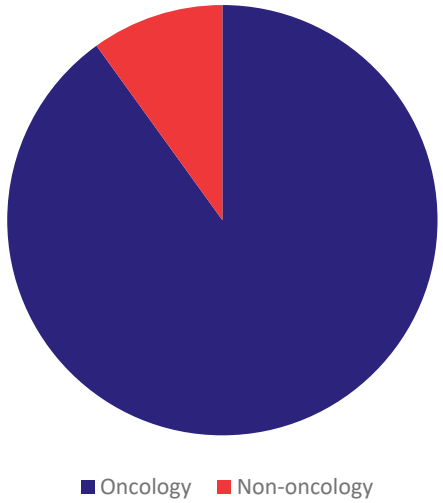
Gene therapy breakdown: CAR-Ts continue to dominate the pipeline

- CAR-T cell therapies remained the most common technology used in the pipeline of genetically modified cell therapies (preclinical through to pre-registration), representing 49%, 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
- 96% of CAR-T cell therapies were in development for cancer indications. The remaining non-oncology indications included scleroderma, HIV/AIDS, and autoimmune disease (unspecified)

Genetically modified cell therapy breakdown



CAR-T breakdown



Source: Cell and Gene Therapy dashboard | Citeline, October 2024

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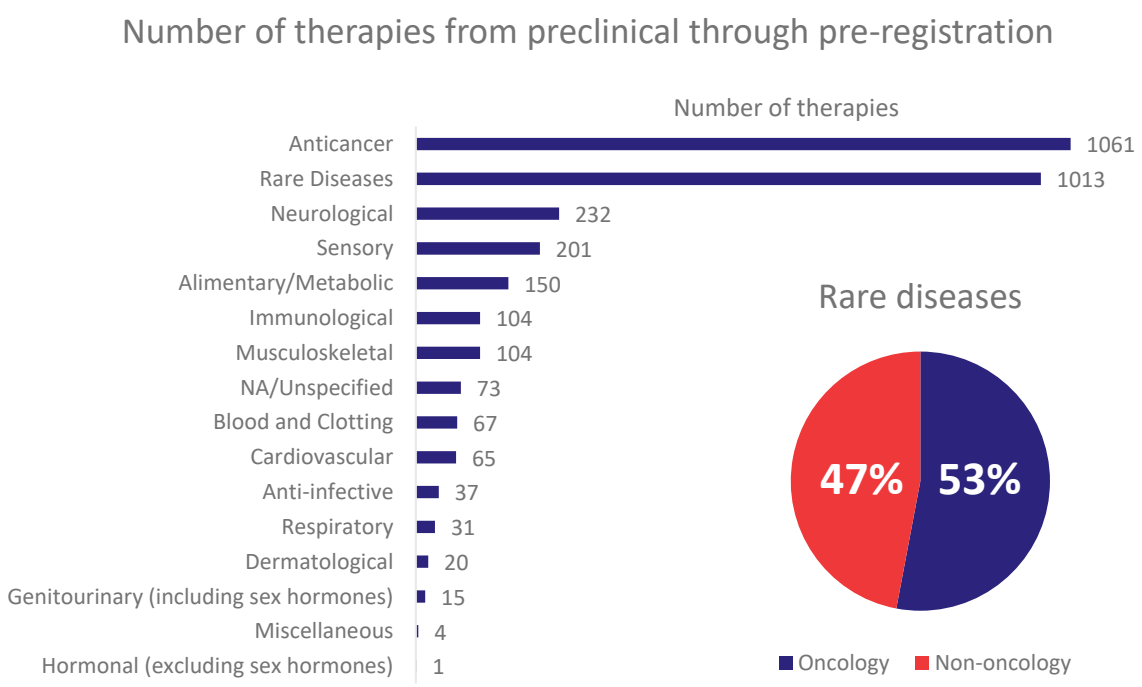
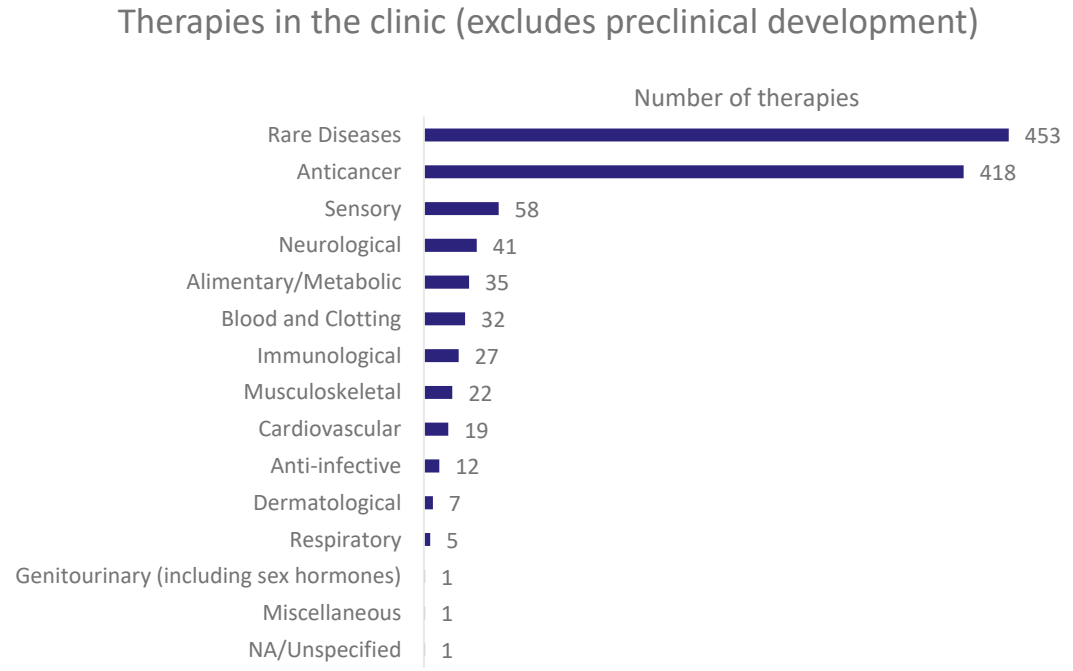
Gene therapy pipeline: most commonly targeted therapeutic areas

- Oncology and rare diseases remained the top areas of gene therapy development in both the overall pipeline (preclinical to pre-registration) and in the clinic (Phase I to pre-registration)
- Development for rare diseases most commonly occurred in oncology, representing a majority of 53% compared to non-oncology rare disease gene therapy pipeline development, one percentage point lower than the previous quarter





Source: Pharmaprojects | Citeline, October 2024

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

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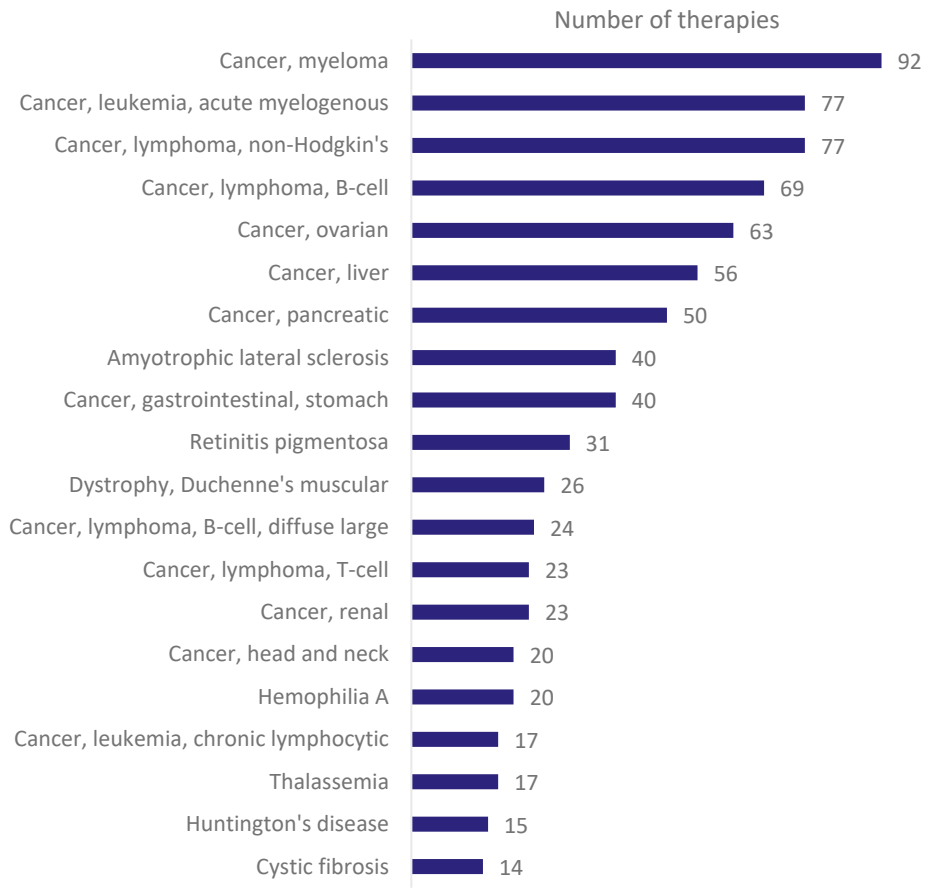
Gene therapy pipeline: most common rare diseases targeted

- For the 1,013 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 2022, 2023, and H1 2024
- In the same order as the previous two quarters, the top five rare diseases for which gene therapies are being developed are:

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



Source: Pharmaprojects | Citeline, October 2024

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

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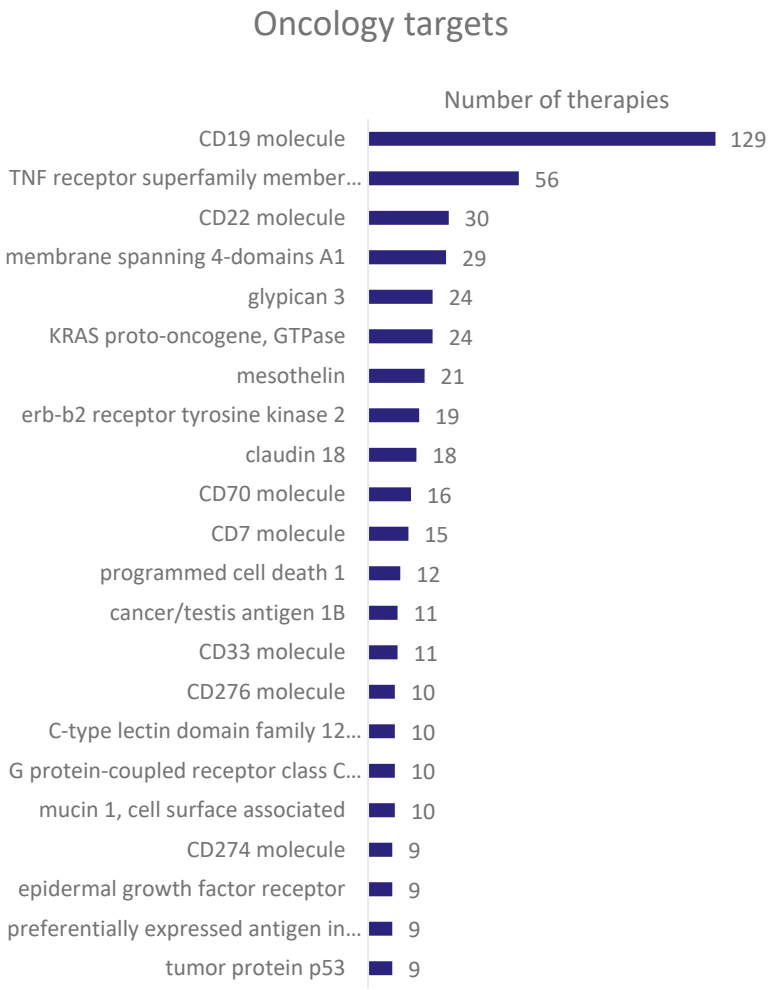
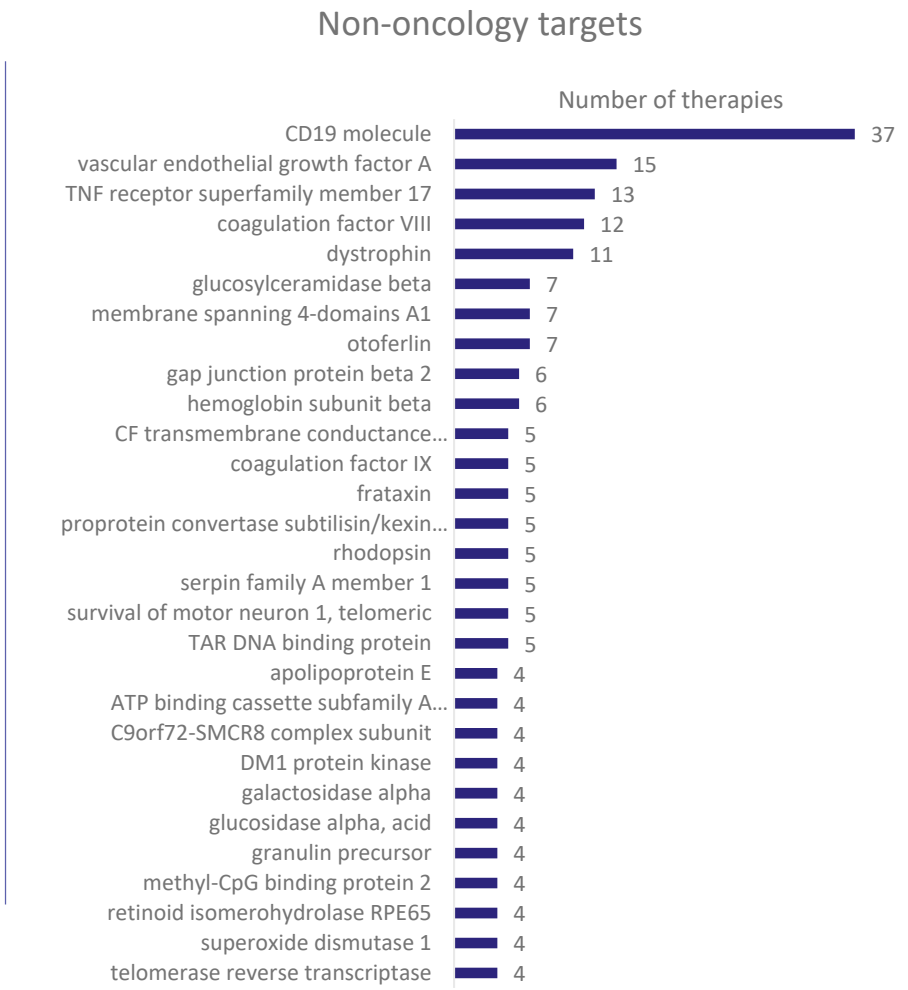
Gene therapy pipeline: most common targets

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

- CD19 molecule; B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17; and CD22 molecule continued to be the top three most common targets for oncology indications
- CD19 molecule was the most common target for non-oncology indications, while vascular endothelial growth factor A and TNF receptor superfamily member 17 jumped to being the second and third most common





Source: Pharmaprojects | Citeline, October 2024

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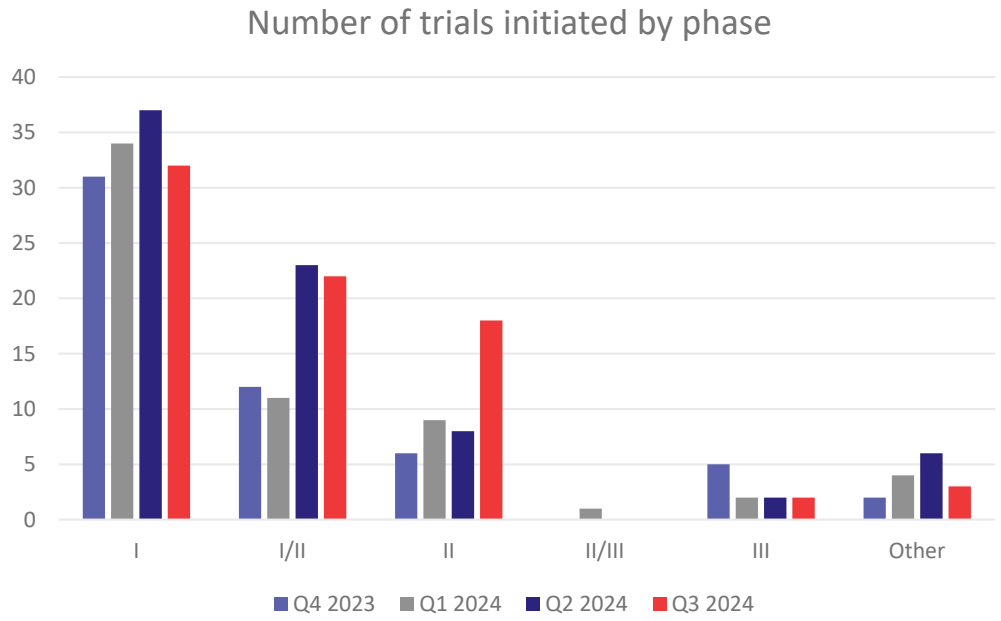
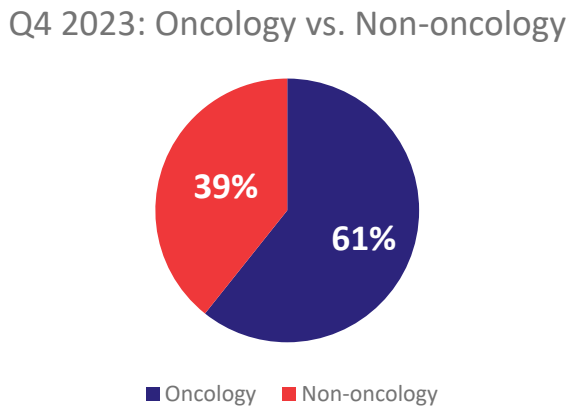
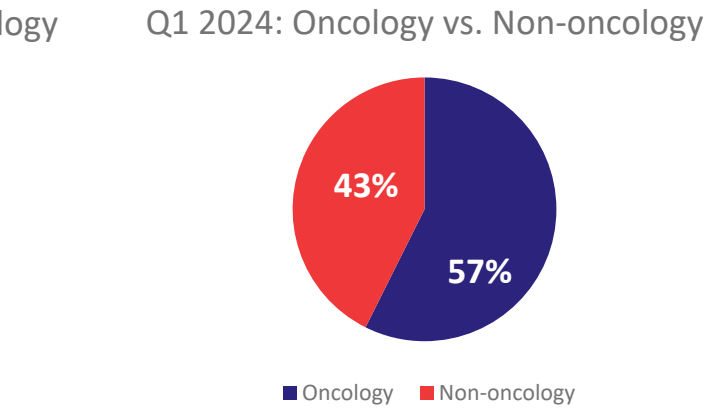
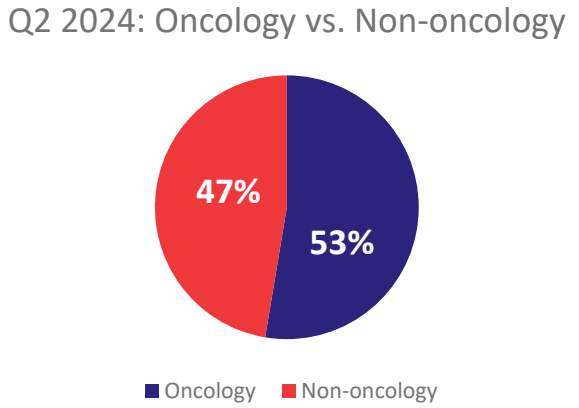
Gene therapy clinical trial activity

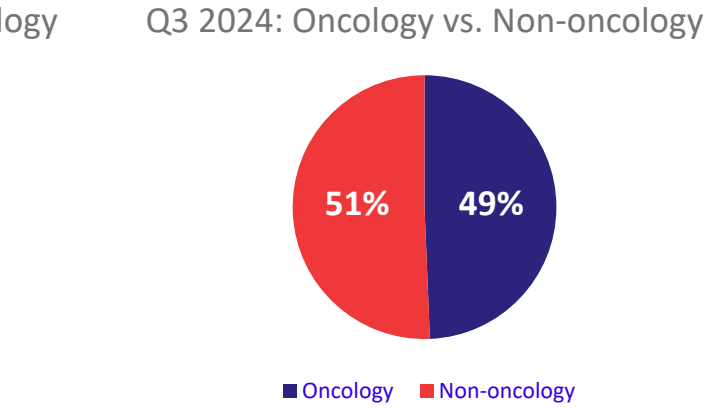
- The proportion of gene therapy trials for non-oncology indications has increased by another four percentage points since the previous quarter, to 51%, continuing the trend of growth in the proportion of non-oncology gene therapy trials
- 77 gene therapy trials were initiated in Q3 2024, one more than the previous quarter











Source: Trialtrove | Citeline, October 2024

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Non-genetically modified cell therapy pipeline

Q3 2024

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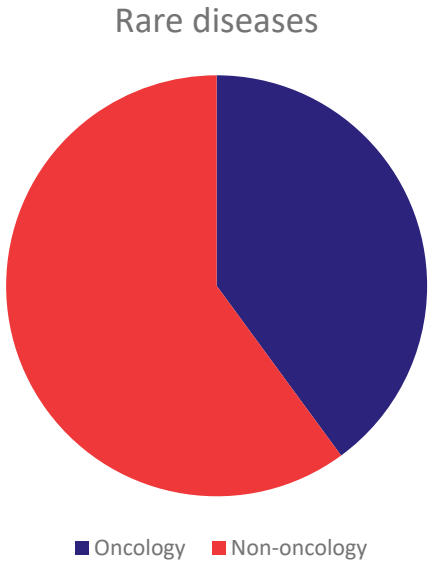
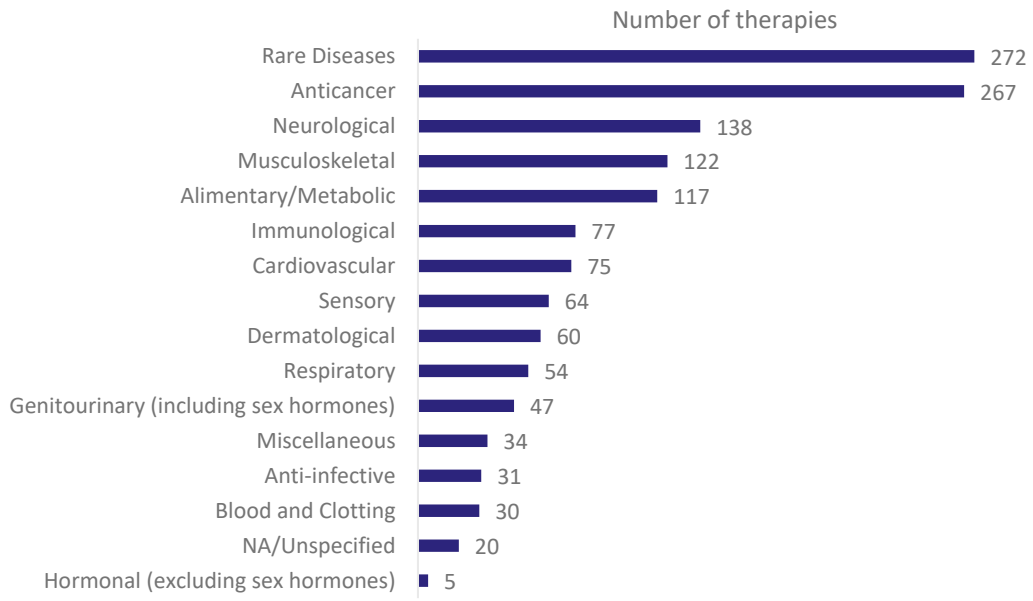
Non-genetically modified cell therapy pipeline: most commonly targeted therapeutic areas

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, 61% were in development for non-oncology rare diseases, one percentage point lower than in the previous quarter





Source: Pharmaprojects | Citeline, October 2024

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

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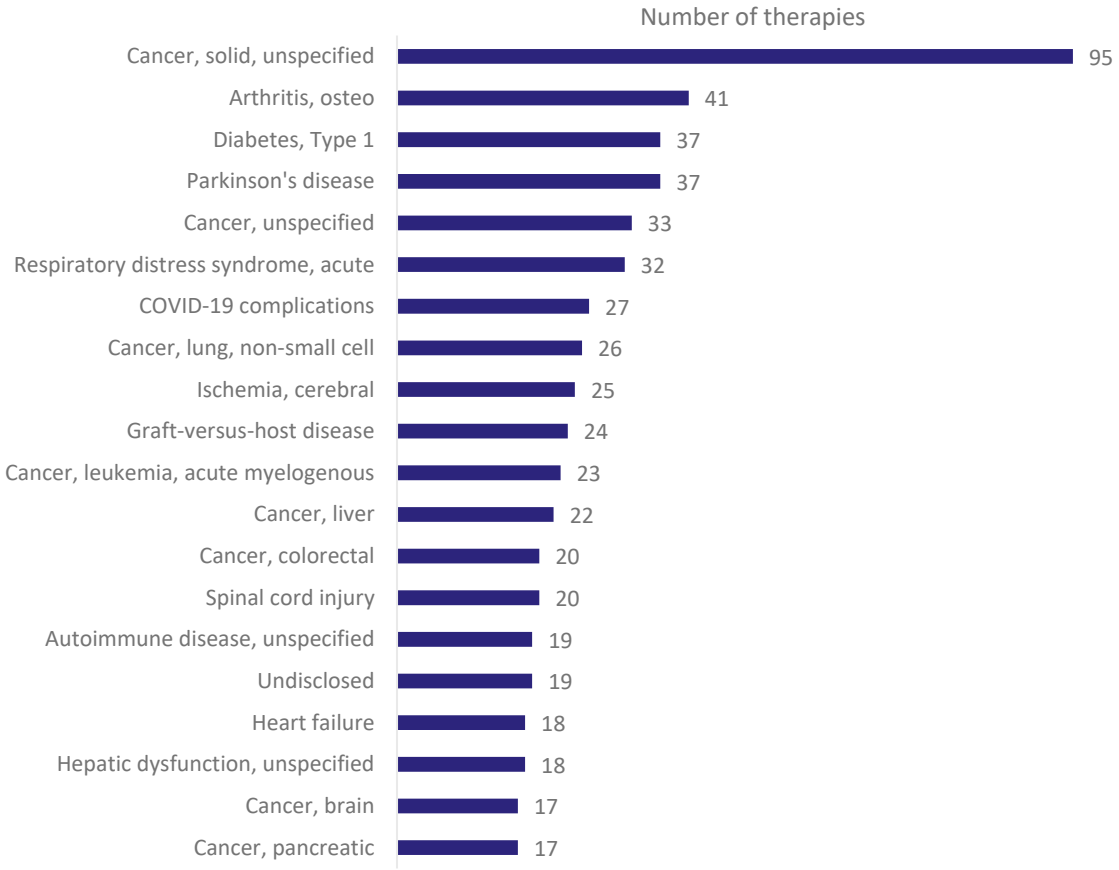
Non-genetically modified cell therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, Parkinson's disease has dropped to third most targeted indication, after being the most targeted for H1 2024:

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



Source: Pharmaprojects | Citeline, October 2024

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

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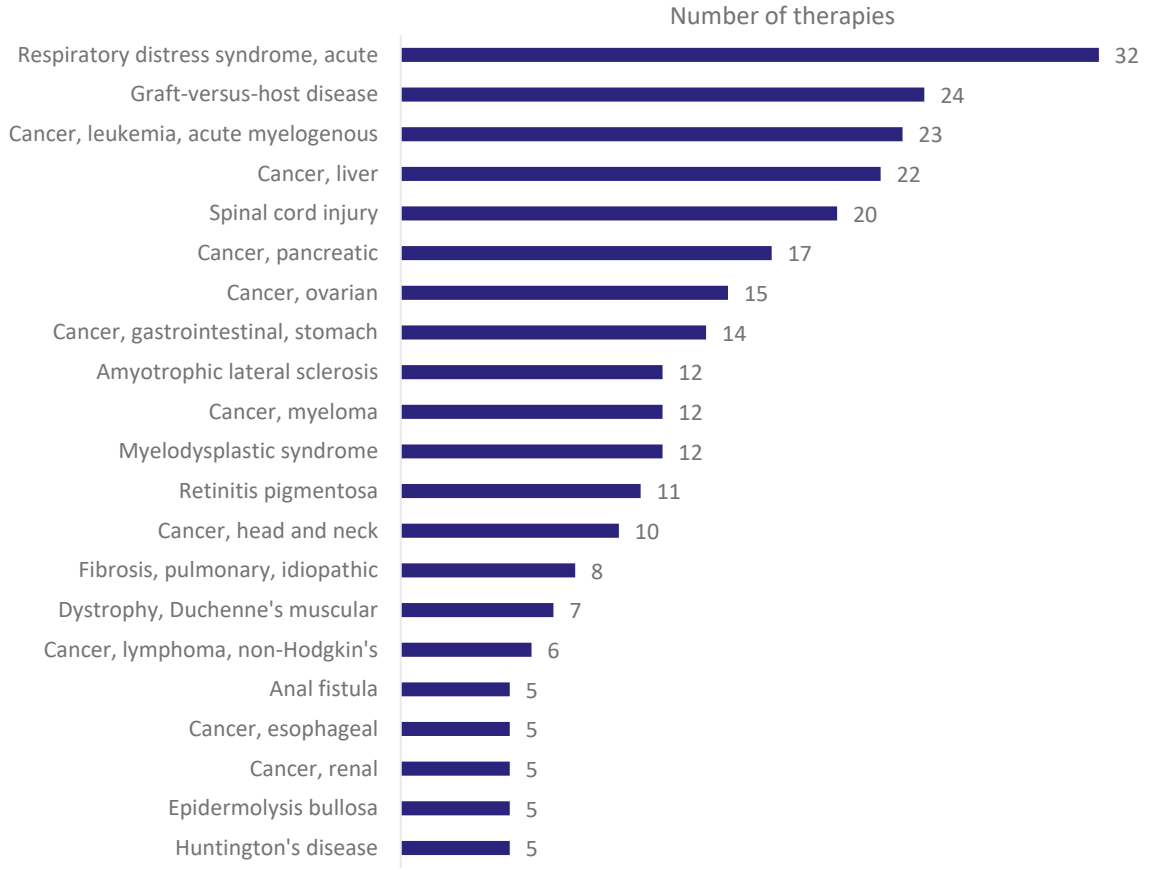
Non-genetically modified cell therapy pipeline: most common rare diseases targeted

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

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



Source: Pharmaprojects | Citeline, October 2024

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

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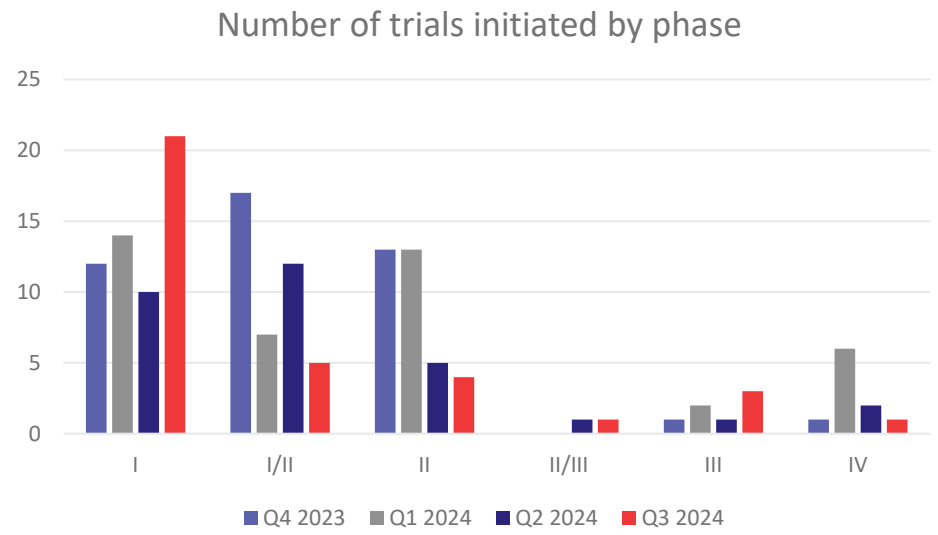
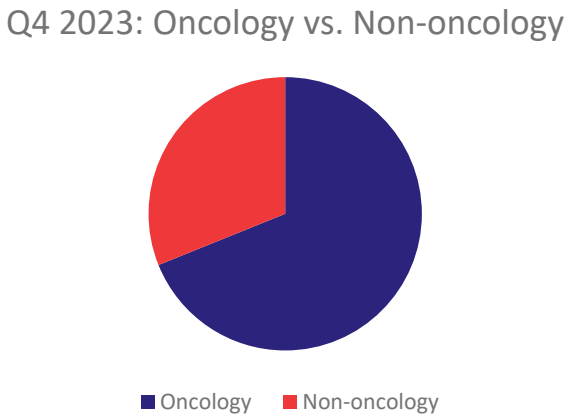
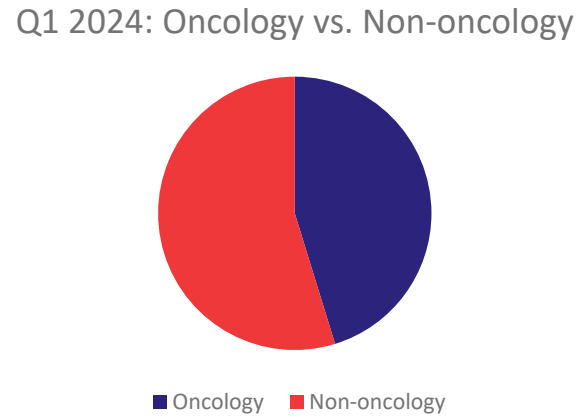
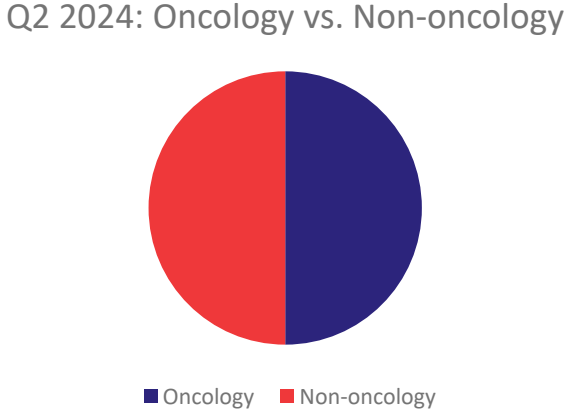
Non-genetically modified cell therapy trial activity

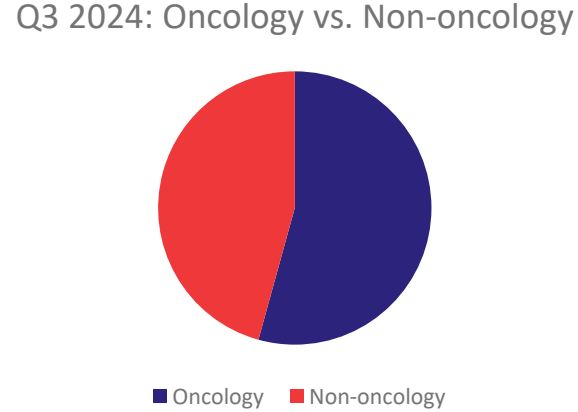
- 35 trials were initiated for non-genetically modified cell therapies in Q3 2024, four more than in the previous quarter
- Of these 35, 46% were for non-oncology indications











Source: Trialtrove | Citeline, October 2024

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RNA therapy pipeline

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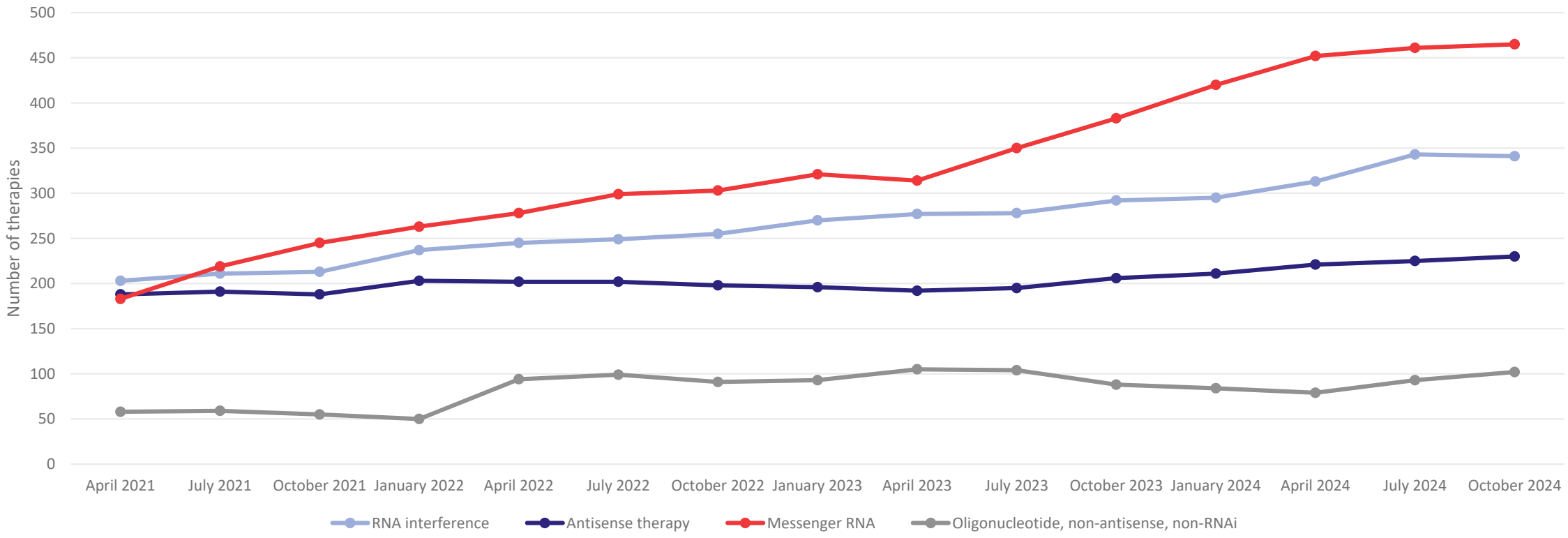
RNA therapy pipeline: most common modalities

- 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, October 2024

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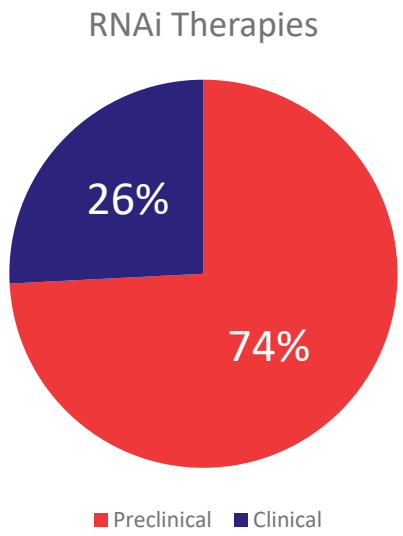
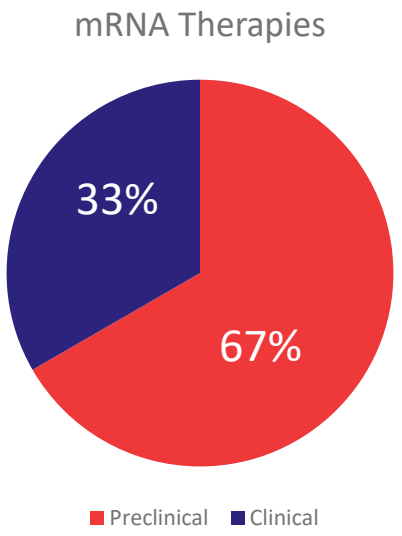
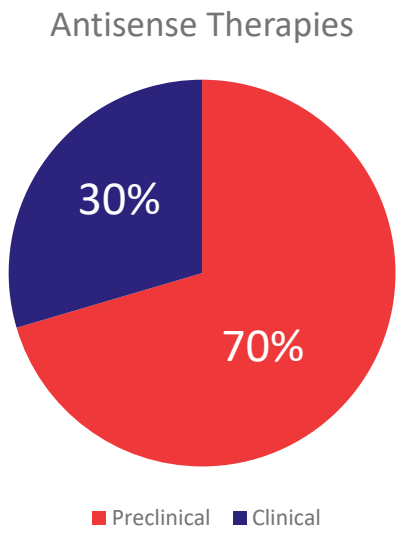
RNAi, mRNA, and antisense oligonucleotides: preclinical vs. clinical

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 74%, 67%, and 70% of their respective pipelines







Source: Pharmaprojects | Citeline, October 2024

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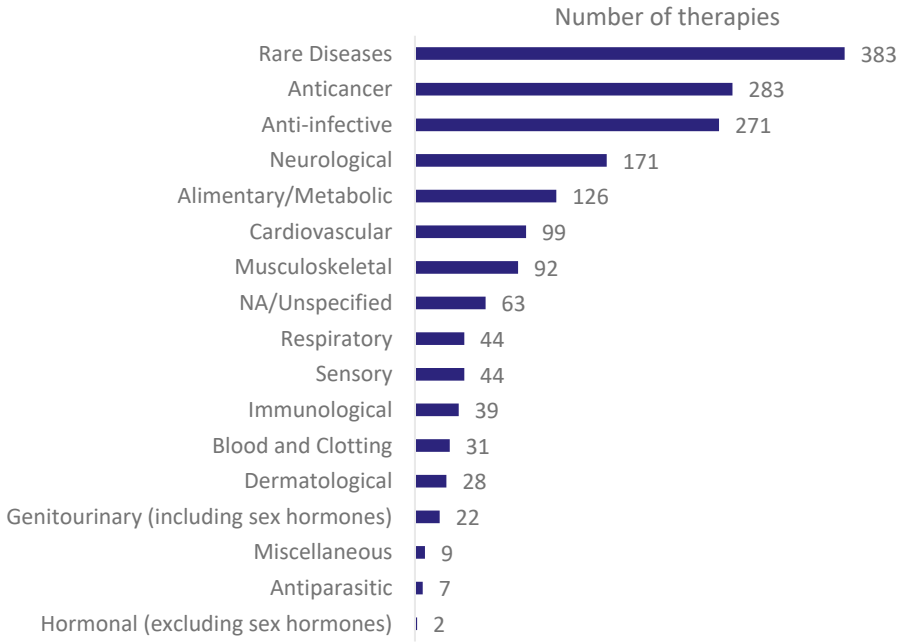
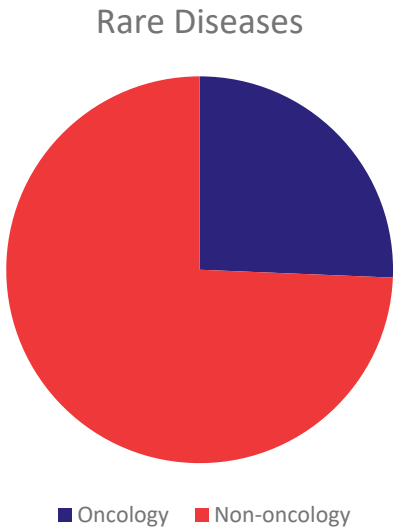
RNA therapies: most commonly targeted therapeutic areas

Of the 1,217 RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Rare diseases remained the top targeted therapeutic area by RNA therapies, while oncology indications have taken over anti-infective indications as the second most commonly targeted
- Non-oncology indications continued to be the most targeted rare diseases by RNA therapies, representing a majority of 75%





Source: Pharmaprojects | Citeline, October 2024

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

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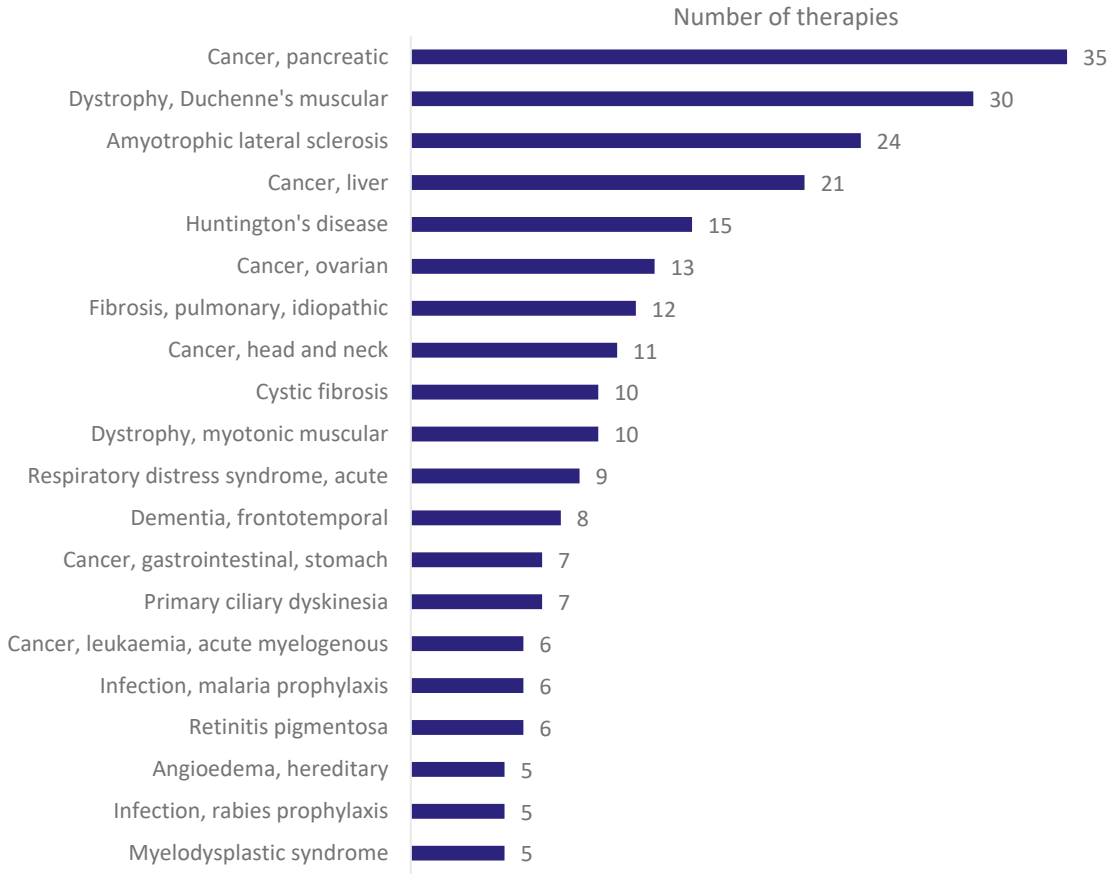
RNA therapies: most common rare diseases targeted

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

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



Source: Pharmaprojects | Citeline, October 2024

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

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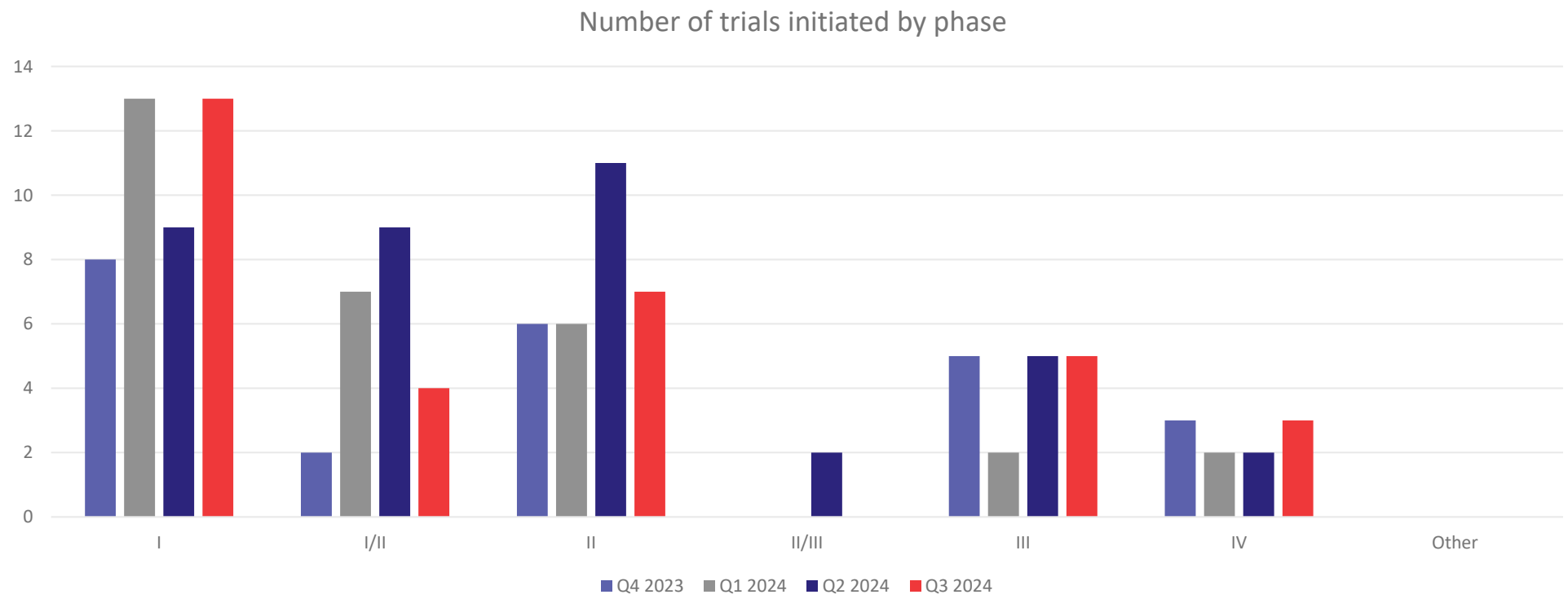
RNA therapy pipeline: clinical trial activity

- 32 RNA trials were initiated in Q3 2024, compared to 38 in Q2 2024, 81% of which were for non-oncology indications



Source: Trialtrove | Citeline, October 2024

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

Q3 2024

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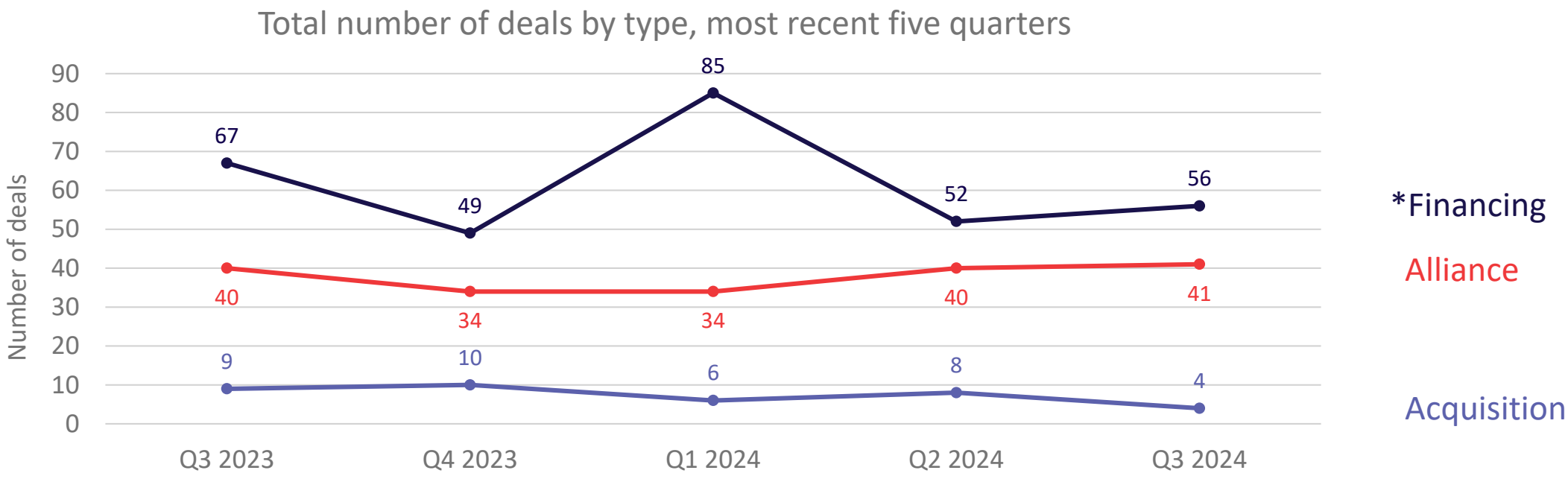
Alliance, acquisition, and financing in gene, cell, and RNA therapy

- Deal volume in the advanced molecular therapies market totaled 101 transactions in Q3 2024, about flat compared with 100 from the previous quarter, and down 13% compared with 116 from the same quarter in 2023
- Financings saw the biggest jump in Q3 versus Q2, increasing by four transactions, with the trend indicating that Q1 2024 may have been an outlier
- Alliances remained on par with the previous quarter's activity, while acquisitions were down



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

*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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Q3 2024 acquisitions in gene, cell, and RNA therapy

- There were four advanced molecular therapy company acquisitions done in Q3 2024, half of the volume seen in the previous quarter
- Q3 2024 acquisition volume also represented a quarterly low within the last year
- CMDOs were an active space – Agilent acquired Biovectra for $925m, and Genezen paid $25m for a viral vector manufacturing facility from uniQure

Deal date	Deal title	Potential deal value (USD $)
05 September 2024	Medera to be Listed on NASDAQ Through Reverse Merger with SPAC Keen Vision Acquisition	Undisclosed
05 August 2024	Mallinckrodt to Sell Therakos Business to CVC Capital for $925M	925,000,000
22 July 2024	Agilent to Acquire CDMO Biovectra	925,000,000
01 July 2024	CDMO Genezen Pays $25M for uniQure's Commercial Viral Vector Manufacturing Facility	25,000,000

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

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

Q3 2024

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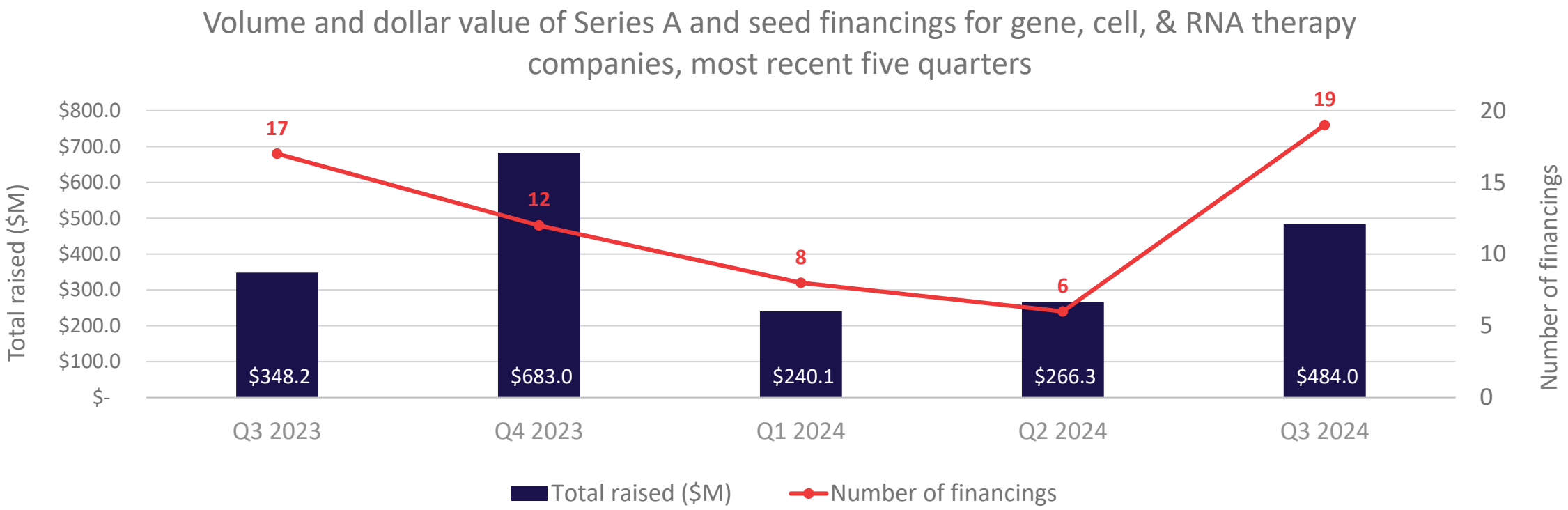
Start-up financing for gene, cell, and RNA therapy companies

- Seed and Series A financings rebounded in Q3 2024, with advanced molecular therapy companies completing 19 rounds
- Start-up financing volume was more than triple the amount done in Q2, and a slight increase over the same quarter a year ago
- Dollars raised were also up in Q3 2024, with companies bringing in $484.0m, representing 1.8x the $266.3m total from Q2



Source: Biomedtracker | Citeline, Evaluate, October 2024

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Q3 2024 start-up financing for gene, cell, and RNA therapy companies (1/3)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
10 July 2024	Granza Bio Gets $7.14M in Seed Round	Precision shell technology to deliver multiple cargoes, including RNA and genetic modifiers	United Kingdom / London	University of Oxford	7.1
14 July 2024	AusperBio Secures $37M in Series A Financing	Antisense oligonucleotides	China / Hangzhou	Undisclosed	37.0
19 July 2024	Rona Therapeutics Gets $35M in Series A+ Round	siRNA therapy	China / Shanghai	Undisclosed	35.0
20 July 2024	Cell BioEngines Secures $2M in Seed Financing	Allogeneic cell therapy	United States / Massachusetts / Boston	Icahn School of Medicine at Mount Sinai	2.0
24 July 2024	Oisín Biotechnologies Closes First Round of $15M Series A Financing Round	Proteo-lipid vehicle for DNA delivery	United States / Washington / Seattle	n/a - technology developed by Entos Pharmaceuticals	15.0
02 August 2024	Amlogenyx Gets $14M in Seed Round	Gene therapy	United States / Massachusetts / Somerville	St. Jude Children's Research Hospital	14.0
06 August 2024	Release Therapeutics Secures CHF3.3M in Seed Funding	Devices containing genetically modified human myoblast cell line; for delivery of therapeutic proteins	Switzerland / Genève	Swiss Federal Institute of Technology Lausanne	3.8

Source: Biomedtracker | Citeline, Evaluate, October 2024

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Q3 2024 start-up financing for gene, cell, and RNA therapy companies (2/3)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
08 August 2024	CorrectSequence Therapeutics Completes CNY100M ($14M) Series A+ Financing	Base editing	China / Shanghai	Chinese Ministry of Science and Technology	14.0
22 August 2024	Borealis Biosciences Spins off from Novartis and Launches with $150M Series A Financing	RNA therapy	Canada / British Columbia / Vancouver	n/a - jointly launched by Versant and Novartis	150.0
27 August 2024	Tern Therapeutics Launches with $15M Series A Round	Gene therapy	United States / Washington	n/a - founded by former Regenxbio executives	15.0
12 September 2024	Vironexis Biotherapeutics Raises $26M in Seed Round*	AAV-delivered T-cell immunotherapy	United States / Texas / Austin	Nationwide Children's Hospital	26.0
11 September 2024	Safi Biotherapeutics Secures $5M in Seed Funding	Cell therapy	United States / Massachusetts	Founded by former DARPA, Vertex, Massachusetts Institute of Technology, and Massachusetts General Hospital executives	5.0
12 September 2024	Base Therapeutics Raises Series A2 Financing	Gene editing	China / Shanghai	Undisclosed	Undisclosed
16 September 2024	Kano Therapeutics Raises $5M Seed Round	Non-viral gene therapy	United States / Massachusetts / Cambridge	MIT's Laboratory for Nucleic Acid Nanotechnology - Bathe BioNanoLab	5.0
18 September 2024	Brenus Pharma Raises $25M in Series A Financing	Cell therapy	France / Issoire	ERC Belgium	25.0
19 September 2024	GC Therapeutics Launches with $65M Series A Financing	Cell therapy	United States / Massachusetts / Cambridge	Harvard Medical School; Wyss Institute at Harvard University	65.0

Source: Biomedtracker | Citeline, Evaluate, October 2024

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Q3 2024 start-up financing for gene, cell, and RNA therapy companies (3/3)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
24 September 2024	LIfT BioSciences Raises £10M in Series A Financing	Cell therapy	United Kingdom / London	Founded around neutrophil infusion research by Zheng Cui and Lloyd J. Old	13.4
24 September 2024	AptaDir Therapeutics Launches with $1.6M in Pre-Seed Funding	RNA therapy	Italy / Milan	Beth Israel Deaconess Medical Center of the Harvard Medical School; Italian Research National Council; Cancer Science Institute of Singapore	1.6
26 September 2024	Mirai Bio Launches with $50M from Flagship Pioneering	Platform for delivery, design, and manufacturing of gene therapy	United States / Massachusetts / Cambridge	Undisclosed	50.0

Source: Biomedtracker | Citeline, Evaluate, October 2024

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Notable Q3 2024 start-up gene, cell, and RNA therapy companies

Company details	Academic source	Financing type/ amount raised	Lead investor(s)	Therapy areas of interest
xRNA technology (platform from Novartis) to target natural mRNA to modulate production of disease-causing proteins	n/a - jointly launched by Versant and Novartis	Series A/$150M	Versant Ventures, Novartis	Kidney
TFome iPSC programming platform	Harvard Medical School, Wyss Institute at Harvard University	Series A/$65M	Cormorant Asset Management	Gastrointestinal, neurology, immunology
End-to-end development capabilities (delivery and design, testing, manufacturing) for genetic medicines	Undisclosed	Initial commitment/$50M	Flagship Pioneering	Undisclosed

Source: Biomedtracker | Citeline, Evaluate, October 2024

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Upcoming catalysts

Q3 2024

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Upcoming Catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q4 2024

Therapy	Generic name	Disease	Catalyst	Catalyst date
Wainua	eplontersen	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	CHMP (European Panel) Results	07 May 2024 - 31 October 2024
Upstaza	eladocagene exuparvovec	Neurology - Other	PDUFA/Approval Decision (U.S.)	13 November 2024 - 13 November 2024
Izervay	avacincaptad pegol	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	CHMP (European Panel) Results	01 May 2024 - 30 November 2024
Olezarsen	olezarsen	Familial Chylomicronemia Syndrome (FCS)/Lipoprotein Lipase Deficiency (LPLD)	PDUFA/Approval Decision (U.S.)	19 December 2024 - 19 December 2024
Rytelo	imetelstat	Myelodysplastic Syndrome (MDS)	CHMP (European Panel) Results	01 June 2024 - 31 December 2024
Vyjuvek	beremagene geperpavec	Epidermolysis Bullosa	CHMP (European Panel) Results	01 July 2024 - 31 December 2024
Wainua	eplontersen	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	Approval Decision (Europe)	08 January 2024 - 31 December 2024
DCVax-L	n/a	Brain Cancer (Malignant Glioma; AA and Glioblastoma (GBM))	Approval Decision (U.K.)	27 September 2024 - 31 December 2024
HPC-Cord Blood Therapy	n/a	Ischemic Stroke	PDUFA/Approval Decision (U.S.)	26 August 2024 - 31 December 2024
Vyjuvek	beremagene geperpavec	Epidermolysis Bullosa	Approval Decision (Europe)	01 July 2024 - 31 December 2024
Oxlumo	lumasiran	Hyperoxaluria	Supplemental Approval (Europe)	29 January 2024 - 31 January 2025
UM171	dorocubicel	Myelodysplastic Syndrome (MDS)	CHMP (European Panel) Results	25 December 2024 - 25 February 2025
Rytelo	imetelstat	Myelodysplastic Syndrome (MDS)	Approval Decision (Europe)	01 August 2024 - 28 February 2025
Izervay	avacincaptad pegol	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	Approval Decision (Europe)	01 October 2024 - 31 March 2025
RP-L102	n/a	Fanconi Anemia	CHMP (European Panel) Results	01 December 2024 - 30 June 2025

Source: Biomedtracker | Citeline, October 2024

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Appendix

Methodology, sources, and glossary of key terms

Q3 2024

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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

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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:

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
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)
Lytic virus *(falls under gene therapy in this report)*	Therapies that have a replication-competent virus, that lyse pathogenic cells directly. These are normally genetically modified to render them harmless to normal tissues. Examples include oncolytic viruses that specifically attack cancer cells

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Glossary of Key Terms

Therapy type definitions, cont.

Cell therapy includes the following therapeutic classes:

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
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

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Therapy type definitions, cont.

RNA therapy includes the following therapeutic classes:

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
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

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

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