American Society of Gene + Cell Therapy

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Gene, Cell, & RNA Therapy Landscape Report

Q2 2026 Quarterly Data Report

July 2026

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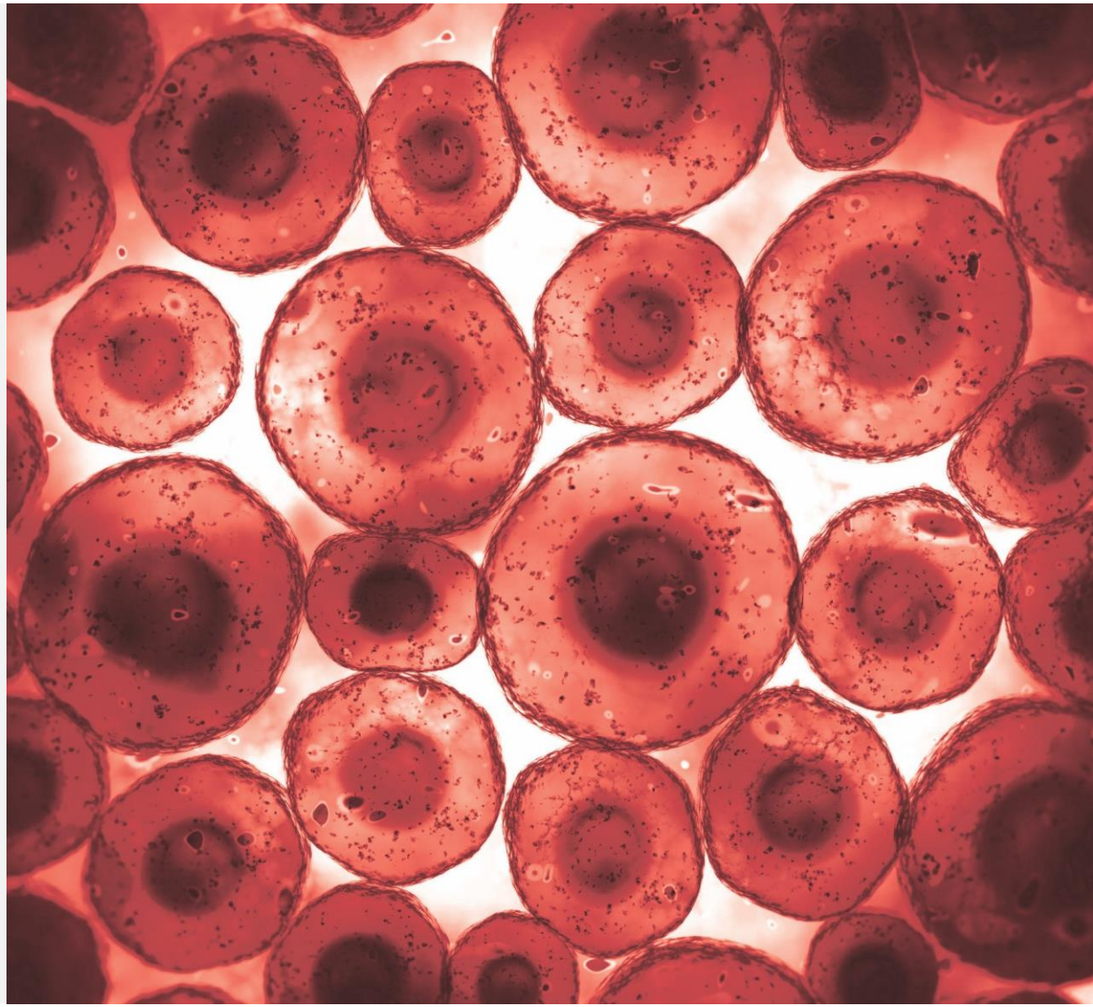
Introduction

The second quarter of 2026 marked a renewed acceleration in regulatory activity across the gene, cell, and RNA therapy landscape, with eight new approvals globally following a comparatively subdued first quarter. Five gene therapies received marketing authorization, including treatments for hearing loss, cancer, and limb ischemia. Moderna's mCombriax became the latest approved RNA therapy in the European Union for seasonal influenza and COVID-19, while Japan authorized two non-genetically modified cell therapies for epidermolysis bullosa and meniscal injuries. Together, these approvals reflect continued diversification across therapeutic modalities and indications, bringing global totals to 46 approved gene therapies (including genetically modified cell therapies), 37 RNA therapies, and 76 non-genetically modified cell therapies.

Innovation also accelerated during the quarter, with 11 new mRNA-encoded CAR-T cell therapies entering development, matching the total reported across all of 2025. This rapid expansion signals growing confidence in next-generation engineering approaches designed to improve the scalability and accessibility of cell therapies. Cartesian Therapeutics' Phase III candidate, Descartes-08, remains the most advanced program in this emerging modality.

Industry activity strengthened alongside scientific progress. Total dealmaking increased 15% quarter over quarter to 118 transactions—the highest quarterly total in the past year—while early-stage financing rebounded sharply, with 20 companies raising a combined $500.4 million in seed or Series A funding. Following the disciplined portfolio optimization observed in late 2025 and the steady regulatory progress seen in Q1, Q2 suggests the field has regained momentum across regulatory, scientific, and investment fronts, reinforcing continued confidence in the long-term growth of advanced molecular therapies.

David Barrett, JD
CEO, ASGCT



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Key takeaways from Q2 2026

Eight new approvals across the gene, cell, and RNA categories took place in Q2 2026

- 5 gene therapies were approved:
  - Regeneron's Otarmeni (lunsotogene parvec) in the US for hearing loss
  - Curocell's Rimqarto (anbalcabtagene autoleucel) in South Korea for diffuse large B-cell lymphoma and primary mediastinal large B-cell lymphoma
  - Helixmith's Huasuoling (donaperminogene seltoplasmid) in China for limb ischemia
  - Oncolys BioPharma's Telomelysin (suratadenoturev) in Japan for esophageal cancer
  - CARsgen Therapeutics' Satri-cel (satricabtagene autoleucel) in China for gastric/gastroesophageal junction adenocarcinoma
- 1 RNA therapy was approved: Moderna's mCombriax in the EU for seasonal influenza (flu) and COVID-19
- 2 non-genetically modified cell therapies were approved:
  - Ishin Pharma's Allostem (nivadostrocel) in Japan for epidermolysis bullosa
  - Fujifilm's Savyscus (synovial mesenchymal stem cell therapy) in Japan for meniscal injuries

Accelerating growth of non-genetically modified, mRNA-engineered CAR-T cells

- Q2 2026 saw 11 new non-genetically modified CAR-T cell therapies engineered using mRNA-encoded chimeric antigen receptors in T cells - matching the total number reported across the entirety of 2025
- These 11 new therapies are being developed across 12 companies, with only ParcelBio associated with more than one of these new assets
- The most advanced mRNA-encoded CAR-T therapy to date is Cartesian Therapeutics' Descartes-08, which is in Phase III trials for the treatment of myasthenia gravis

Deal activity for advanced molecular therapy companies rebounded in Q2 2026

- All deal types increased over the previous quarter to reach 118 transactions in Q2 2026, a 15% jump, and represented a quarterly high within the last year
- Start-up financing also had a strong showing with 20 companies raising an aggregate $500.4 million in seed or Series A funding, a 54% and 29% increase in volume and value, respectively, over Q1 2026
- Serapha Bio, one of several companies to announce a reverse merger in Q2, completed the largest Series A round at $138 million, which will be used to develop its *in vivo* base editing therapy SERP-01 (licensed from YolTech) for alpha-1 antitrypsin deficiency

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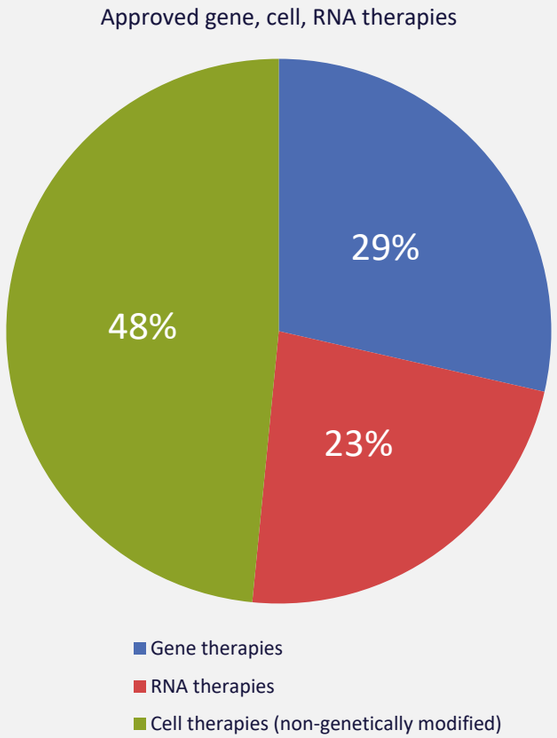
Key highlights in Q2 2026

Approved gene, cell, and RNA therapies

Globally, for clinical use:

- 46 gene therapies have been approved (including genetically modified cell therapies)
  - Regeneron's Otarmeni (lunsotogene parvec) has been approved in the US for hearing loss
  - Curocell's Rimqarto (anbalcabtagene autoleucel) has been approved in South Korea for diffuse large B-cell lymphoma (DLBCL) and primary mediastinal large B-cell lymphoma (PMBCL)
  - Helixmith's Huasuoling (donaperminogene seltoplasmid) has been approved in China for limb ischemia
  - CARsgen Therapeutics' Satri-cel (satricabtagene autoleucel) has been approved in China for gastric/gastroesophageal junction adenocarcinoma
  - Oncolys BioPharma's Telomelysin (suratadenoturev) has been approved in Japan for esophageal cancer
- 37 RNA therapies have been approved
  - Moderna's mCombriax has been approved in the EU for seasonal influenza (flu) and COVID-19
- 76 non-genetically modified cell therapies have been approved
  - Ishin Pharma's Allostem (nivadostrocel) has been approved in Japan for epidermolysis bullosa
  - Fujifilm's Savyscus (synovial mesenchymal stem cell therapy) has been approved in Japan for meniscal injuries



Source: Pharmaprojects | Citeline, July 2026

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Approved gene therapies as of Q2 2026

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Gendicine	recombinant p53 gene	2004	Head and neck cancer	China	Shenzhen SiBiono GeneTech
Oncorine	E1B/E3 deficient adenovirus	2005	Head and neck cancer; nasopharyngeal cancer	China	Shanghai Sunway Biotech
Rexin-G	mutant cyclin-G1 gene	2006	Solid tumors	Philippines	Epeius Biotechnologies
Neovasculgen	vascular endothelial growth factor gene	2011	Peripheral vascular disease; limb ischemia	Russian Federation, Ukraine	Human Stem Cells Institute
Imlygic	talimogene laherparepvec	2015	Melanoma	US, EU, UK, Australia, China	Amgen
Strimvelis	autologous CD34+ enriched cells	2016	Adenosine deaminase deficiency	EU, UK	Orchard Therapeutics
Kymriah	tisagenlecleucel-t	2017	Acute lymphocytic leukemia; diffuse large B-cell lymphoma; follicular lymphoma	US, EU, UK, Japan, Australia, Canada, South Korea, Switzerland, Brazil	Novartis
Luxturna	voretigene neparvovec	2017	Leber's congenital amaurosis; retinitis pigmentosa	US, EU, UK, Australia, Brazil, Canada, South Korea, Japan, Russian Federation	Spark Therapeutics (Roche)
Yescarta	axicabtagene ciloleucel	2017	Diffuse large B-cell lymphoma; non-Hodgkin's lymphoma; follicular lymphoma	US, EU, UK, Japan, Canada, China, Australia, Brazil, Israel, Singapore, South Korea	Kite Pharma (Gilead)
Zolgensma	onasemnogene abeparvovec	2019	Spinal muscular atrophy	US, Japan, EU, UK, Brazil, Canada, Israel, Taiwan, UK, Australia, South Korea, China, UAE	Novartis
Zynteglo	betibeglogene autotemcel	2019	Transfusion-dependent beta thalassemia	US	Genetix Biotherapeutics (formerly bluebird bio)
Tecartus	brexucabtagene autoleucel	2020	Mantle cell lymphoma; acute lymphocytic leukemia	US, EU, UK, Australia, Canada, Brazil	Kite Pharma (Gilead)
Libmeldy	atidarsagene autotemcel	2020	Metachromatic leukodystrophy	EU, UK, Switzerland, US	Orchard Therapeutics
Breyanzi	lisocabtagene maraleucel	2021	Diffuse large B-cell lymphoma; follicular lymphoma; chronic lymphocytic leukemia; mantle cell lymphoma; marginal zone lymphoma	US, Japan, EU, Canada, Switzerland, UK	Celgene (Bristol Myers Squibb)

Highlighted text represents new approvals during Q2 2026

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Approved gene therapies as of Q2 2026 *continued*

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

Highlighted text represents new approvals during Q2 2026

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Approved gene therapies as of Q2 2026 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Zevor-cel	zevorcabtagene autoleucel	2024	Relapsed or refractory multiple myeloma	China	CARsgen Therapeutics
Tecelra	afamitresgene autoleucel	2024	Synovial sarcoma	US	Adaptimmune
Aucatzyl	obecabtagene autoleucel	2024	Acute lymphocytic leukemia	US, UK, EU	Autolus
Qartemi	varnimcabtagene autoleucel	2025	B-cell Non-Hodgkin's Lymphoma (B-NHL)	India, Spain	Immuneel Therapeutics
Encelto	revakinagene taroretcel	2025	Macular telangiectasia type 2 (MacTel)	US	Neurotech
BBM-H901	dalnacogene ponparvovec	2025	Hemophilia B	China	Belief BioMed
Zevaskyn	prademagene zamikeracel	2025	Recessive dystrophic epidermolysis bullosa (RDEB)	US	Abeona Therapeutics
Hicara	renikeolunsai	2025	Relapsed or refractory large B-cell lymphoma	China	Hrain Biotechnology
Papzimeos	zopapogene imadenovec	2025	Recurrent respiratory papillomatosis (RRP)	US	Precigen
Pulidekai	pulkilumab	2025	Acute lymphocytic leukemia	China	Chongqing Precision Biotech
Waskyra	etuvetidigene autotemcel	2025	Wiskott-Aldrich syndrome	US	GSK
Kresladi	marnetegragene autotemcel	2026	Leukocyte adhesion deficiency	US	Rocket Pharmaceuticals
Otarmeni	lunsotogene parvec	2026	Hearing loss	US	Regeneron
Rimqarto	anbalcabtagene autoleucel	2026	Diffuse large B-cell lymphoma; Primary mediastinal large B-cell lymphoma	South Korea	Curocell
Huasuoling	donaperminogene seltoplasmid	2026	Limb ischemia	China	Helixmith
Telomelysin	suratadenoturev	2026	Esophageal cancer	Japan	Oncolys BioPharma
Satri-cel	satricabtagene autoleucel	2026	Gastric/gastroesophageal junction adenocarcinoma	China	CARsgen Therapeutics

Highlighted text represents new approvals during Q2 2026

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Approved RNA therapies as of Q2 2026

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

Highlighted text represents new approvals during Q2 2026

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Approved RNA therapies as of Q2 2026 *continued*

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

Highlighted text represents new approvals during Q2 2026

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Approved RNA therapies as of Q2 2026 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Rivfloza	nedosiran	2023	Hyperoxaluria	US	Dicerna Pharmaceuticals (Novo Nordisk)
SYS-6006.32	Bivalent COVID-19 mRNA vaccine, CSPC Pharmaceutical	2023	Infection, coronavirus, novel coronavirus prophylaxis	China	CSPC Pharmaceutical
RQ-3033	COVID-19 mRNA vaccine, Walvax Biotechnology	2023	Infection, coronavirus, novel coronavirus prophylaxis	China	Walvax Biotechnology
Rytelo	imetelstat	2024	Myelodysplastic syndrome	US, EU	Geron
mRESVIA	respiratory syncytial virus vaccine, Moderna Therapeutics	2024	Respiratory syncytial virus prophylaxis	US, EU, Canada, Qatar, Taiwan, UAE, UK, Australia, Switzerland, Japan, South Korea	Moderna Therapeutics
Tryngolza	olezarsen	2024	Lipoprotein lipase deficiency; Hypertriglyceridemia	US, EU, Canada	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, Canada, Australia, EU, Japan	Moderna Therapeutics
Dawnzera	donidalorsen	2025	Angioedema, hereditary	US, EU, UK	Ionis Pharmaceuticals
Redemplo	plozasiran	2025	Lipoprotein lipase deficiency	US, Canada, China, Australia, EU	Arrowhead Pharmaceuticals
mCombriax	COVID-19 + influenza vaccine	2026	Infection, coronavirus, novel coronavirus prophylaxis; Infection, influenza virus prophylaxis	EU	Moderna

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Key highlights in Q2 2026 (Noteworthy events that happened in Q2 2026)

Drug	Event Type	Indication	Molecule	Event Date
A2B543	Regulatory - Fast Track Status	Solid Tumors	Cellular	Apr 1, 2026
RTx015	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Retinitis Pigmentosa (RP)	Viral Gene Therapy	Apr 1, 2026
Tecartus	Regulatory - Accelerated/Conditional Approval to Full Approval (U.S.)	Mantle Cell Lymphoma (MCL)	Cellular	Apr 1, 2026
rebisufligene etisparvovec	Regulatory - Response to Complete Response Letter (CRL) to NDA/BLA Accepted	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A Syndrome)	Viral Gene Therapy	Apr 2, 2026
Allostem	Regulatory - Approval (Japan)	Epidermolysis Bullosa	Cellular	Apr 3, 2026
Itvisma	Regulatory - Approval (Japan)	Spinal Muscular Atrophy	Viral Gene Therapy	Apr 3, 2026
Telomelysin	Regulatory - Manufacturing Application Approval	Esophageal Cancer	Viral Gene Therapy	Apr 3, 2026
miroliverELAP	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Liver Failure / Cirrhosis	Cellular	Apr 8, 2026
vusolimogene oderparepvec	Regulatory - Complete Response Letter (CRL)	Melanoma	Viral Gene Therapy	Apr 10, 2026
ET3	Regulatory - Orphan Drug Designation (U.S.)	Hemophilia A	Cellular	Apr 12, 2026
TARA-002	Regulatory - Orphan Drug Designation (U.S.)	Lymphatic Malformation (LM)	Cellular	Apr 12, 2026
GS-100	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Neurology - Other	Viral Gene Therapy	Apr 14, 2026
AB-729	Regulatory - Fast Track Status	Hepatitis B (HBV) Treatment	siRNA/RNAi	Apr 15, 2026
HM2003	Regulatory - Fast Track Status	Cardiovascular Disease	Other Nucleic Acid	Apr 15, 2026
haematopoietic stem cell	Regulatory - Orphan Drug Designation (U.S.)	Acute Lymphoblastic Leukemia (ALL)	Cellular	Apr 16, 2026
Mydicar	Regulatory - Fast Track Status	Cardiomyopathy - Dilated	Viral Gene Therapy	Apr 16, 2026
Mydicar	Regulatory - Fast Track Status	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	Apr 16, 2026
CMP-002	Regulatory - Orphan Drug Designation (Europe)	Neurology - Other	siRNA/RNAi	Apr 20, 2026
mCombriax	Regulatory - Approval (Europe)	Seasonal Influenza Vaccines	mRNA (messenger RNA)	Apr 20, 2026

Source: Biomedtracker | Evaluate, July 2026

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Key highlights in Q2 2026 (Noteworthy events that happened in Q2 2026) *continued*

Drug	Event Type	Indication	Molecule	Event Date
mCombriax	Regulatory - Approval (Europe)	COVID-19 Prevention	mRNA (messenger RNA)	Apr 20, 2026
PBFT02	Regulatory - FDA Response	Frontotemporal Dementia (FTD)	Viral Gene Therapy	Apr 20, 2026
SGT-003	Regulatory - Orphan Drug Designation (Europe)	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	Apr 20, 2026
CartiLife	Regulatory - Approval (China)	Osteoarthritis	Cellular	Apr 23, 2026
Otarmeni	Regulatory - Accelerated/Conditional Approval (U.S.)	Otoferlin Gene-Mediated Hearing Loss	Viral Gene Therapy	Apr 23, 2026
Telomelysin	Regulatory - Fast Track Status	Esophageal Cancer	Viral Gene Therapy	Apr 23, 2026
lonvoguran ziclumeran	Regulatory - Rolling NDA/BLA Initiated	Hereditary Angioedema (HAE)	Cellular	Apr 27, 2026
Bepirovirsen	Regulatory - Breakthrough Therapy Designation (U.S.)	Hepatitis B (HBV) Treatment	Antisense	Apr 28, 2026
Bepirovirsen	Regulatory - Priority Review	Hepatitis B (HBV) Treatment	Antisense	Apr 28, 2026
iSCIB-1+	Regulatory - Fast Track Status	Melanoma	Other Nucleic Acid	Apr 28, 2026
Orca-Q	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Hematologic Cancer	Cellular	Apr 28, 2026
ReNu	Regulatory - Rolling NDA/BLA Completed	Osteoarthritis	Cellular	Apr 28, 2026
ET3	Regulatory - Fast Track Status	Hemophilia A	Cellular	Apr 29, 2026
ET3	Regulatory - Rare Pediatric Disease (RPD) Designation	Hemophilia A	Cellular	Apr 29, 2026
GT719	Regulatory - Orphan Drug Designation (U.S.)	Acute Lymphoblastic Leukemia (ALL)	Cellular	Apr 29, 2026
NP001	Regulatory - Orphan Drug Designation (U.S.)	Amyotrophic Lateral Sclerosis (ALS)	Cellular	Apr 29, 2026
Rimqarto	Regulatory - Approval (Emerging Markets)	Diffuse Large B-Cell Lymphoma (DLBCL)	Cellular	Apr 29, 2026
RTx015	Regulatory - PRIME Designation (Europe)	Retinitis Pigmentosa (RP)	Viral Gene Therapy	Apr 29, 2026
Casgevy	Regulatory - sNDA/sBLA Filing	Sickle Cell Disease	Cellular	May 4, 2026

Source: Biomedtracker | Evaluate, July 2026

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Key highlights in Q2 2026 (Noteworthy events that happened in Q2 2026) *continued*

Drug	Event Type	Indication	Molecule	Event Date
Casgevy	Regulatory - sNDA/sBLA Filing	Thalassemia	Cellular	May 4, 2026
SB-101	Regulatory - Rare Pediatric Disease (RPD) Designation	Urea Cycle Disorders and Derangements (UCD)	Cellular	May 4, 2026
AP-SA02	Regulatory - Fast Track Status	Staphylococcal Vaccines and Other Staphylococcus-Specific Agents	Cellular	May 7, 2026
Izervay	Regulatory - Filing for Approval (China)	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy	Oligonucleotide	May 7, 2026
RPTR-1-201	Regulatory - Fast Track Status	Triple-Negative Breast Cancer (TNBC)	Cellular	May 7, 2026
VY-FXN01	Regulatory - Orphan Drug Designation (U.S.)	Friedreich's Ataxia	Viral Gene Therapy	May 7, 2026
Adstiladrin	Regulatory - Approval (Japan)	Bladder Cancer	Viral Gene Therapy	May 8, 2026
Lomecel-B	Regulatory - FDA Response	Cardiovascular Disease	Cellular	May 8, 2026
Savyscus	Regulatory - Approval (Japan)	Cartilage and Joint Repair	Cellular	May 8, 2026
Surv.m-CRA-1	Regulatory - Orphan Drug Designation (Japan)	Osteosarcoma	Other Nucleic Acid	May 8, 2026
taspitimagene advec	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	Cellular	May 8, 2026
NN9001	Regulatory - Fast Track Status	Parkinson's Disease (PD)	Cellular	May 11, 2026
KYV-101	Regulatory - Rolling NDA/BLA Initiated	Neurology - Other	Cellular	May 13, 2026
SUPLEXA	Regulatory - Fast Track Status	Colorectal Cancer (CRC)	Cellular	May 13, 2026
YOLT-202	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Alpha-1 Antitrypsin Deficiency (A1AD or AATD)	Cellular	May 14, 2026
TN-401	Regulatory - PRIME Designation (Europe)	Chronic Heart Failure and Cardiomyopathies - Unspecified	Viral Gene Therapy	May 15, 2026
89Zr-Oxine-RY_SW01	Regulatory - Filing for Approval (China)	Scleroderma	Cellular	May 21, 2026
Otarmeni	Regulatory - European Filing Accepted	Otoferlin Gene-Mediated Hearing Loss	Viral Gene Therapy	May 22, 2026
Telomelysin	Regulatory - Approval (Emerging Markets)	Esophageal Cancer	Viral Gene Therapy	May 22, 2026

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Key highlights in Q2 2026 (Noteworthy events that happened in Q2 2026) *continued*

Drug	Event Type	Indication	Molecule	Event Date
ARM101	Regulatory - Orphan Drug Designation (U.S.)	Delayed Graft Function (DGF)	Cellular	May 27, 2026
Engensis	Regulatory - Approval (China)	Cardiovascular Disease	Viral Gene Therapy	May 28, 2026
Dermacyte Liquid	Regulatory - Fast Track Status	Chronic Venous Ulcers	Cellular	Jun 2, 2026
KLN-1010	Regulatory - Fast Track Status	Multiple Myeloma (MM)	Cellular	Jun 2, 2026
hNPC-01	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Ischemic Stroke	Cellular	Jun 3, 2026
TN-201	Regulatory - PRIME Designation (Europe)	Cardiomyopathy - Hypertrophic	Viral Gene Therapy	Jun 3, 2026
salanersen	Regulatory - Breakthrough Therapy Designation (U.S.)	Spinal Muscular Atrophy	Antisense	Jun 4, 2026
AlloNK	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Rheumatoid Arthritis (RA)	Cellular	Jun 8, 2026
GT0003X	Regulatory - Sakigake Designation (Japan)	Parkinson's Disease (PD)	Viral Gene Therapy	Jun 8, 2026
Lasme-cel	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Acute Lymphoblastic Leukemia (ALL)	Cellular	Jun 9, 2026
CLD-401	Regulatory - FDA Response	Head and Neck Cancer	Viral Gene Therapy	Jun 16, 2026
CLD-401	Regulatory - FDA Response	Non-Small Cell Lung Cancer (NSCLC)	Viral Gene Therapy	Jun 16, 2026
CLD-401	Regulatory - FDA Response	Ovarian Cancer	Viral Gene Therapy	Jun 16, 2026
CLD-401	Regulatory - FDA Response	Solid Tumors	Viral Gene Therapy	Jun 16, 2026
QT-019B	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Immune Thrombocytopenic Purpura (ITP)	Cellular	Jun 16, 2026
QT-019B	Regulatory - Breakthrough Therapy Designation (U.S.)	Immune Thrombocytopenic Purpura (ITP)	Cellular	Jun 16, 2026
AST-301	Regulatory - Orphan Drug Designation (U.S.)	Gastric Cancer	Other Nucleic Acid	Jun 17, 2026
Tecelra	Regulatory - Accelerated/Conditional Approval to Full Approval (U.S.)	Synovial Sarcoma	Cellular	Jun 17, 2026
cemdisiran	Regulatory - MAA Submission (Europe)	Myasthenia Gravis (MG)	siRNA/RNAi	Jun 22, 2026

Source: Biomedtracker | Evaluate, July 2026

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Key highlights in Q2 2026 (Noteworthy events that happened in Q2 2026) *continued*

Drug	Event Type	Indication	Molecule	Event Date
cemdisiran	Regulatory - Priority Review	Myasthenia Gravis (MG)	siRNA/RNAi	Jun 22, 2026
Navsunli	Regulatory - FDA Response	Mucopolysaccharidosis II (MPS II; Hunter Syndrome)	Viral Gene Therapy	Jun 22, 2026
PM359	Regulatory - Fast Track Status	Chronic Granulomatous Disease	Cellular	Jun 22, 2026
PM359	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Chronic Granulomatous Disease	Cellular	Jun 22, 2026
PRAX-222	Regulatory - Breakthrough Therapy Designation (U.S.)	Seizure Disorders (Epilepsy)	Antisense	Jun 22, 2026
RGX-181	Regulatory - Fast Track Status	Neuronal Ceroid Lipofuscinosis (NCL)	Viral Gene Therapy	Jun 22, 2026
Satri-cel	Regulatory - Approval (China)	Gastric Cancer	Cellular	Jun 22, 2026
NS-035	Regulatory - Orphan Drug Designation (Japan)	Muscular Dystrophy - Unspecified	Antisense	Jun 23, 2026
VGR R01	Regulatory - Filing for Approval (China)	Other Ophthalmological Indications	Viral Gene Therapy	Jun 23, 2026
Stemchymal	Regulatory - J-NDA Filing (Japan)	Spinocerebellar Ataxia	Cellular	Jun 24, 2026
Stemchymal	Regulatory - Priority Review (Japan)	Spinocerebellar Ataxia	Cellular	Jun 24, 2026
ABO-101	Regulatory - Orphan Drug Designation (Europe)	Hyperoxaluria	Cellular	Jun 25, 2026
Del-zota	Regulatory - NDA/BLA Filing	Duchenne Muscular Dystrophy (DMD)	siRNA/RNAi	Jun 25, 2026
Nuvaxovid	Regulatory - Supplemental Filing (Japan)	COVID-19 Prevention	Other Nucleic Acid	Jun 26, 2026
vusolimogene oderparepvec	Regulatory - Response to Complete Response Letter (CRL) to NDA/BLA Accepted	Melanoma	Viral Gene Therapy	Jun 26, 2026

Source: Biomedtracker | Evaluate, July 2026

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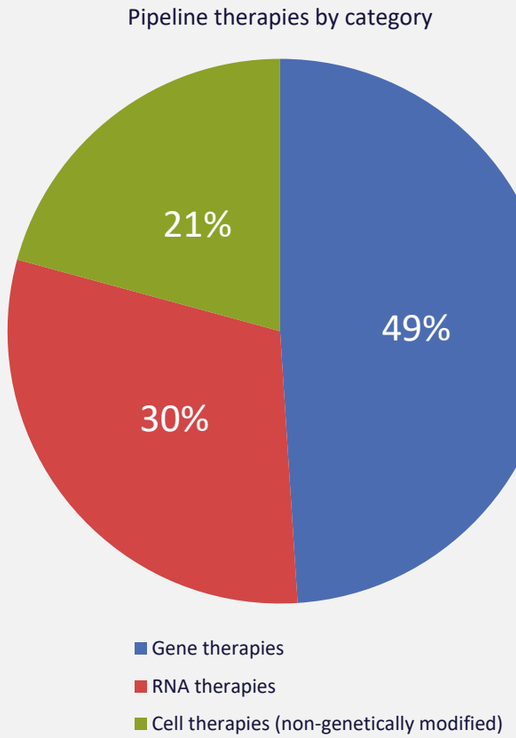
Pipeline overview

Pipeline of gene, cell, and RNA therapies

4,487 therapies are in development, ranging from preclinical through pre-registration

- 2,217 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
- 940 non-genetically modified cell therapies are in development, accounting for 21% of gene, cell, and RNA therapies



Source: Pharmaprojects | Citeline, July 2026

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Gene therapy pipeline

Gene therapy and genetically modified cell therapies

Gene therapy pipeline: quarterly comparison

- An increase in the number of gene therapy programs was seen at all stages of pipeline development except for pre-registration
- In Q2 2026, three gene therapies advanced to Phase III trials: avigbagene parvec for Gaucher's disease, KITE-753 for B-cell lymphoma, and TAEST-16001 for soft tissue sarcoma
- Therapies currently in pre-registration:

In the US

- vusolimogene oderparepvec (Replimune)
- sonpiretigene isteparvovec (Nanoscope Therapeutics)
- pariglasgene brecaparvovec (Ultragenyx)
- cretostimogene grenadenorepvec (CG Oncology)
- isaralgagene civaparvovec (Sangamo Therapeutics)
- rebisufligene etisparvovec (Abeona Therapeutics)
- anitocabtagene autoleucel (Arcellx)
- mivocabtagene autoleucel (Kyverna Therapeutics)
- lonvoguran ziclumeran (Intellia Therapeutics)

In China

- IM-19 (Imunopharm)
- Wanji Aolunsai (Huadao (Shanghai) Biopharma)
- VGR-R01 (Vitalgen BioPharma)

Global Status	Q2 2025	Q3 2025	Q4 2025	Q1 2026	Q2 2026
Preclinical	1,461	1,346	1,227	1,269	1,332
Phase I	361	377	397	423	427
Phase II	330	347	355	368	387
Phase III	45	47	49	57	59
Pre-registration	13	11	12	15	12
Total	2,155	2,129	2,040	2,132	2,217

Highlighted text represents new filings during Q2 2026

Source: Pharmaprojects | Citeline, July 2026

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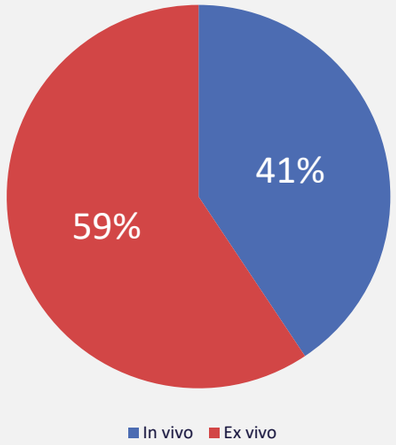
Genetic modification: *In vivo* vs. *Ex vivo*

- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q2 2026, *In vivo* delivery techniques were used in 41% of gene therapies

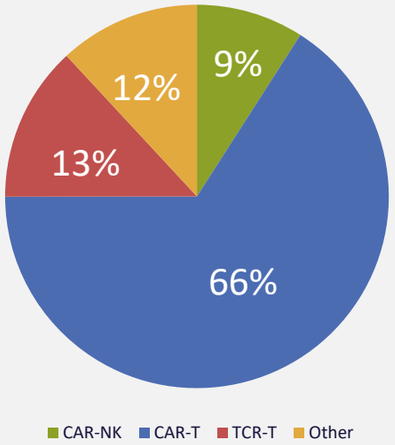
Gene therapy breakdown: CAR-Ts continue to dominate the pipeline

- CAR-T cell therapies remained the most common technology used in the pipeline of genetically modified cell therapies (preclinical through pre-registration), representing 66%, followed by the TCR-T therapies at 13%
- 81% of CAR-T cell therapies are in development for cancer indications
- The most common non-oncology diseases for CAR-T therapies are lupus, scleroderma, and myasthenia gravis

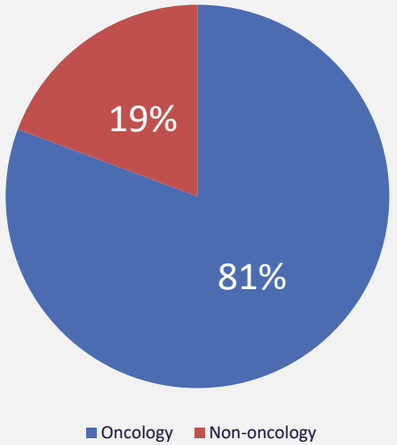
In vivo vs. *Ex vivo* genetic modification



Genetically modified cell therapy breakdown



CAR-T breakdown



Source: Cell and Gene Therapy dashboard | Citeline, July 2026

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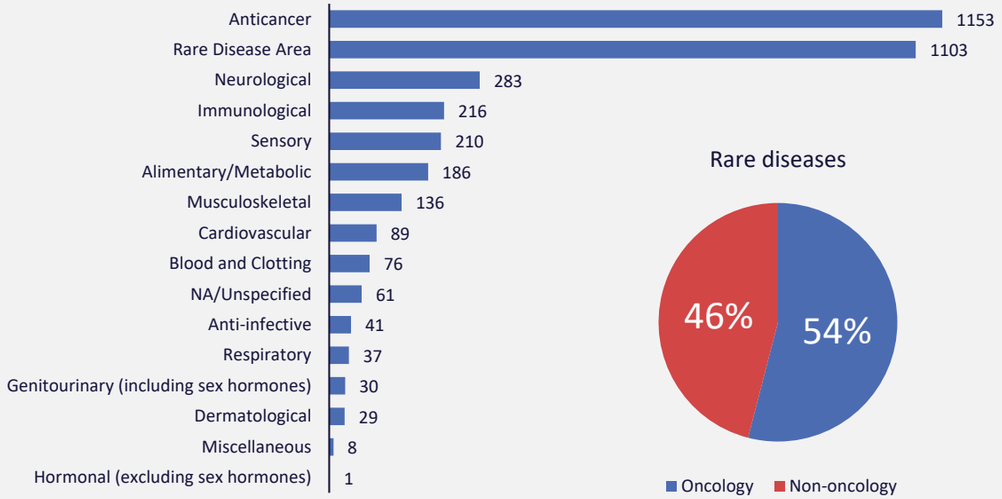
Gene therapy pipeline: most commonly targeted therapeutic areas

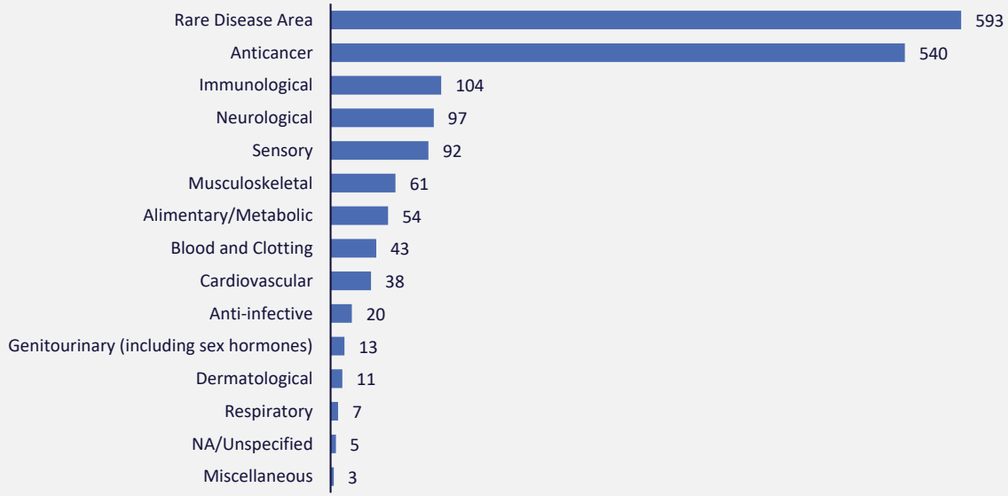
- Rare diseases and oncology remained the top areas of gene therapy development in both the overall pipeline (preclinical to pre-registration) and in the clinic (Phase I to pre-registration)
- Oncology still accounts for more than half of all rare gene therapies from preclinical through pre-registration (54%), despite dropping one percentage point last quarter
  - The most commonly targeted rare oncology diseases are myeloma, non-Hodgkin's lymphoma, and B cell lymphoma

Number of therapies from preclinical through pre-registration



Rare diseases

Number of therapies in the clinic (excludes preclinical development)



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple disease areas

Source: Pharmaprojects | Citeline, July 2026

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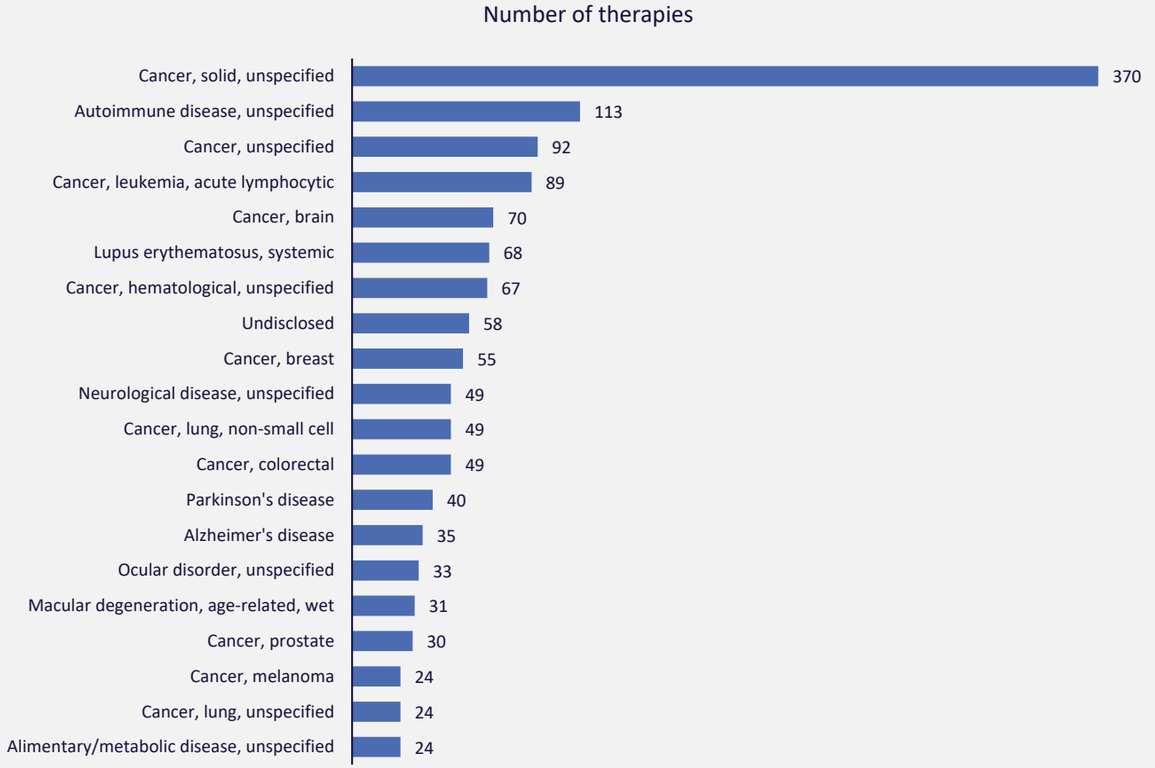
Gene therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q2 2026 were:

- Acute lymphocytic leukemia
- Brain cancer
- Systemic lupus erythematosus



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

Source: Pharmaprojects | Citeline, July 2026

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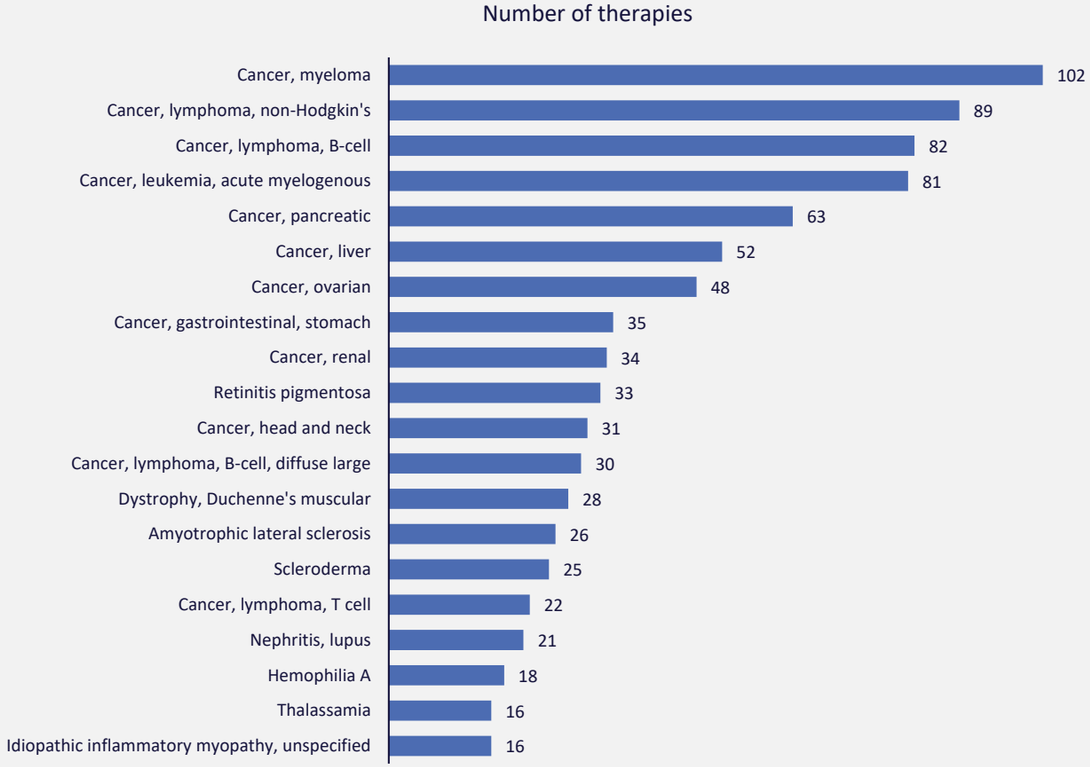
Gene therapy pipeline: most common rare diseases targeted

- For the 1,074 pipeline (preclinical to pre-registration) gene therapies being developed for rare diseases, nine out of the top 10 rare diseases were oncological
- The top five rare diseases for which gene therapies are being developed:
  - Myeloma
  - Non-Hodgkin’s lymphoma
  - B-cell lymphoma
  - Acute myelogenous leukemia
  - Pancreatic cancer



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

Source: Pharmaprojects | Citeline, July 2026

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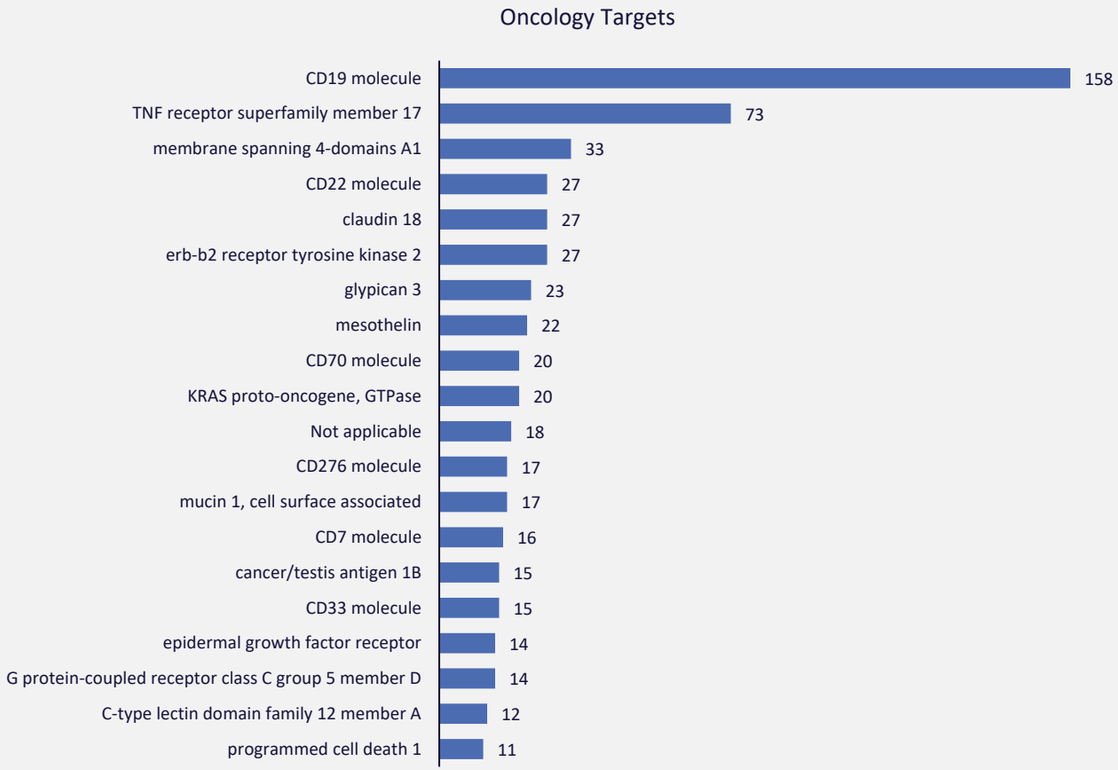
Gene therapy pipeline: most common targets

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

- CD19 molecule and B-cell maturation antigen (BCMA), also known as TNF receptor superfamily member 17, remained the top two most common targets for oncology indications



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Gene therapy pipeline: most common targets *continued*

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

- CD19 molecule, TNF receptor superfamily member 17, and vascular endothelial growth factor A continued to be the top three most common targets for non-oncology indications

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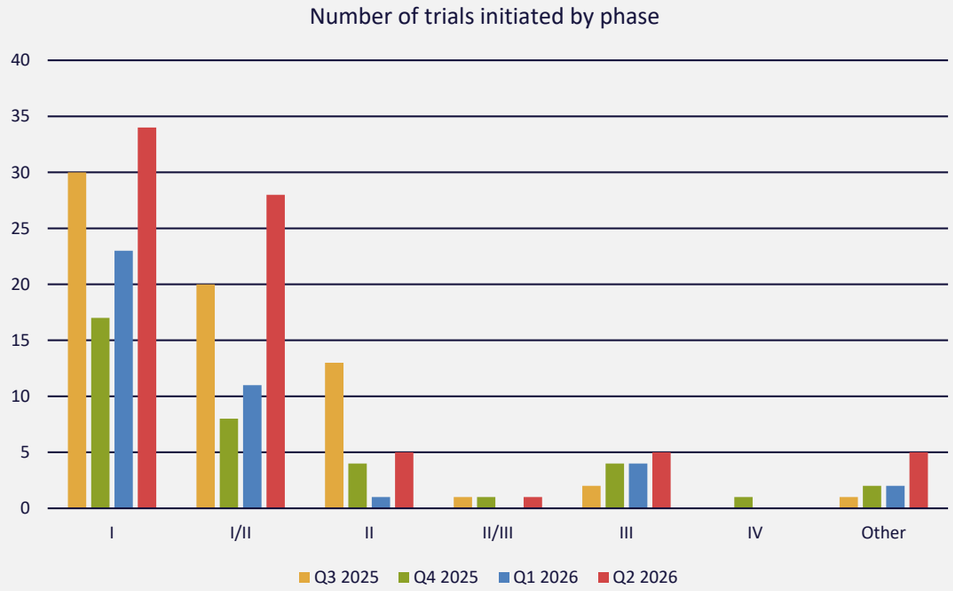
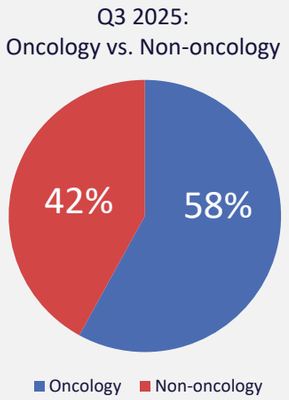
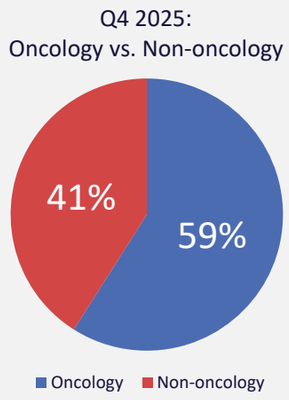
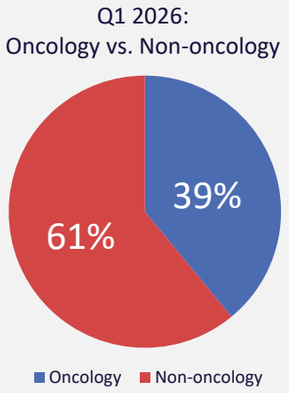
Gene therapy clinical trial activity

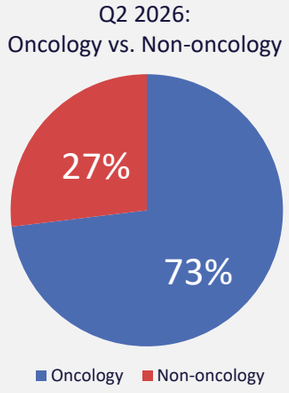
- The proportion of gene therapy trials for non-oncology indications decreased to 27%, 34 percentage points lower than last quarter
- 78 gene therapy trials were initiated in Q2 2026, 37 more than the previous quarter
- There are currently 1,896 open gene therapy clinical trials











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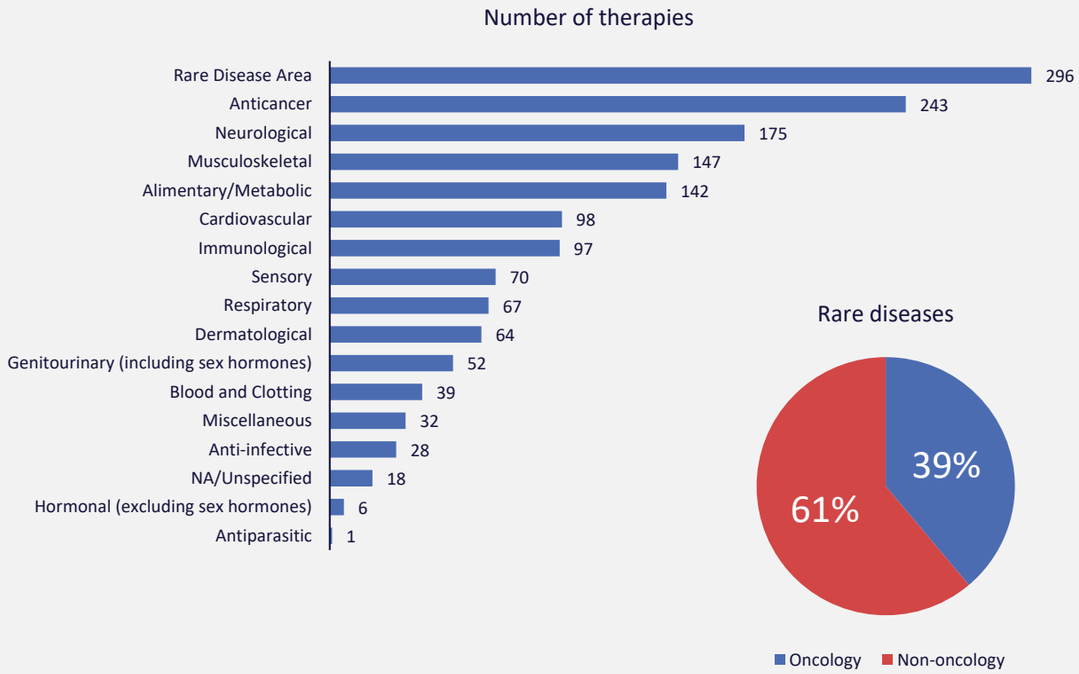
Non-genetically modified cell therapy pipeline

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

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

- Rare diseases and oncology remained the top areas of non-genetically modified cell therapy development
- Non-oncology diseases still account for more than half of all non-genetically modified cell therapies in development (61%), despite dropping one percentage point last quarter
    - The most commonly targeted rare non-oncology disease areas are Neurological, Immunological, and Respiratory



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple disease areas

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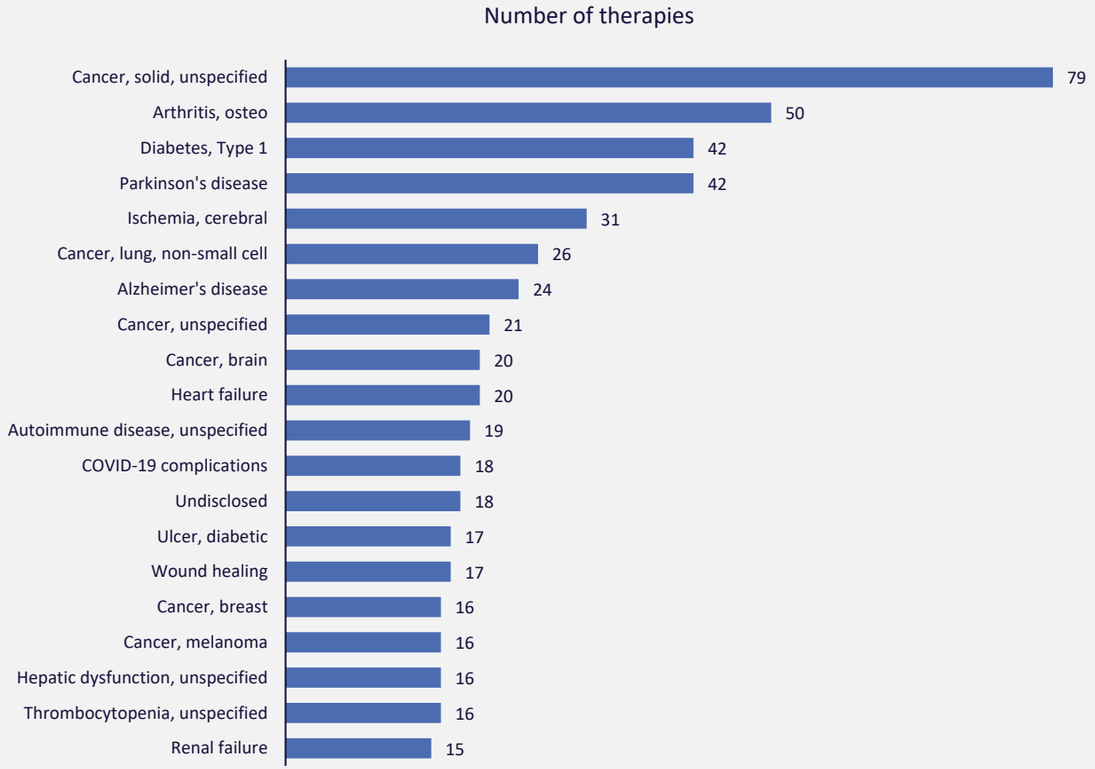
Non-genetically modified cell therapy pipeline: most common diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q2 2026 were:

- Osteoarthritis
- Type 1 diabetes
- Parkinson’s disease
- Cerebral ischemia



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

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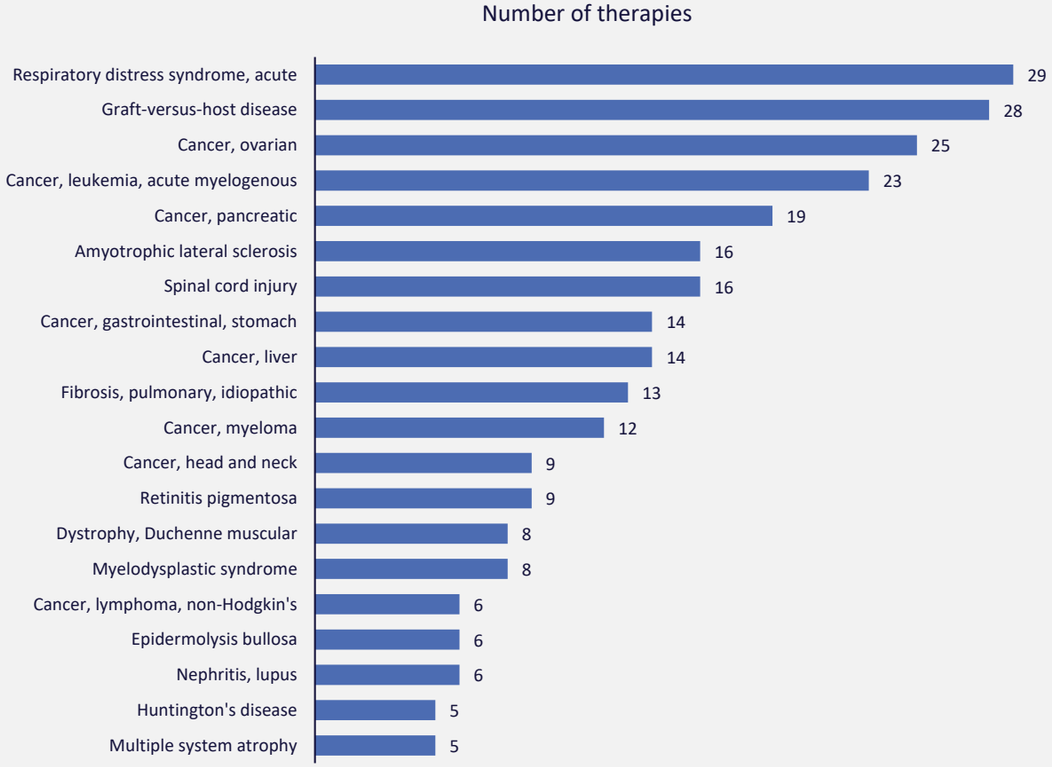
Non-genetically modified cell therapy pipeline: most common rare diseases targeted

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

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



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

Source: Pharmaprojects | Citeline, July 2026

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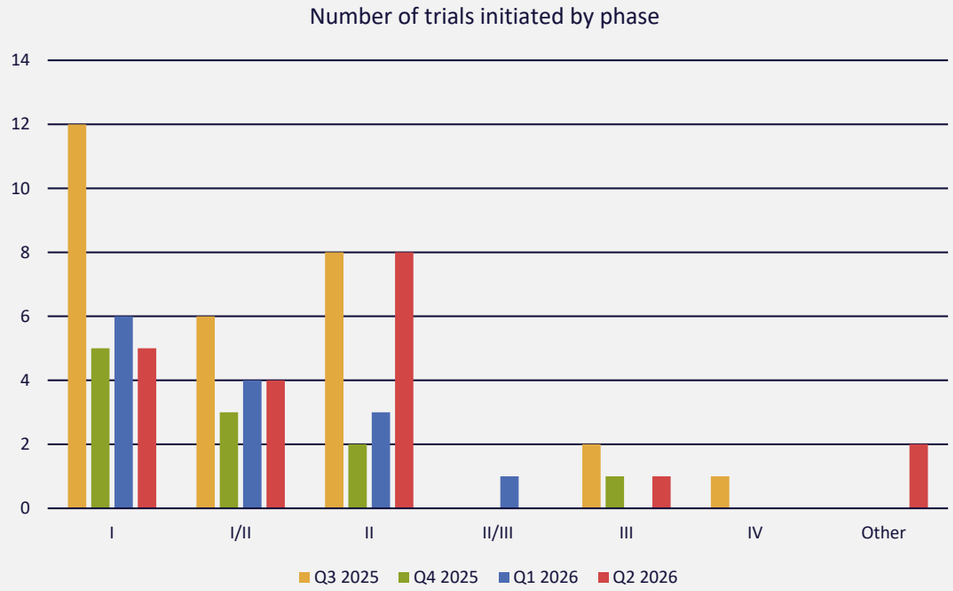
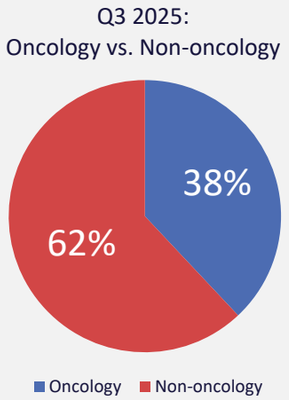
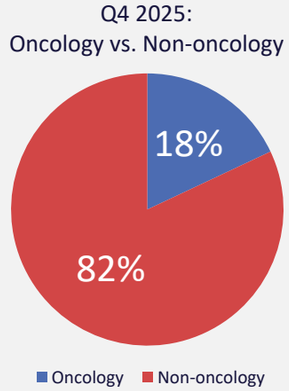
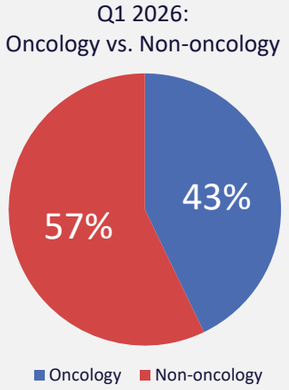
Non-genetically modified cell therapy trial activity

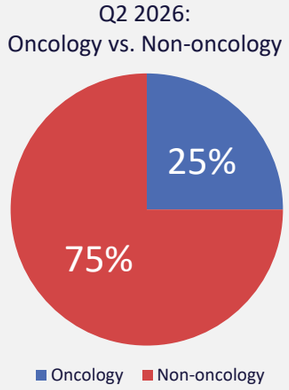
- 20 trials were initiated for non-genetically modified cell therapies in Q2 2026, six more than in Q1 2026
- Of these 20, 75% were for non-oncology indications, 18 percentage points higher than the previous quarter
- There are currently 705 open non-genetically modified cell therapy clinical trials











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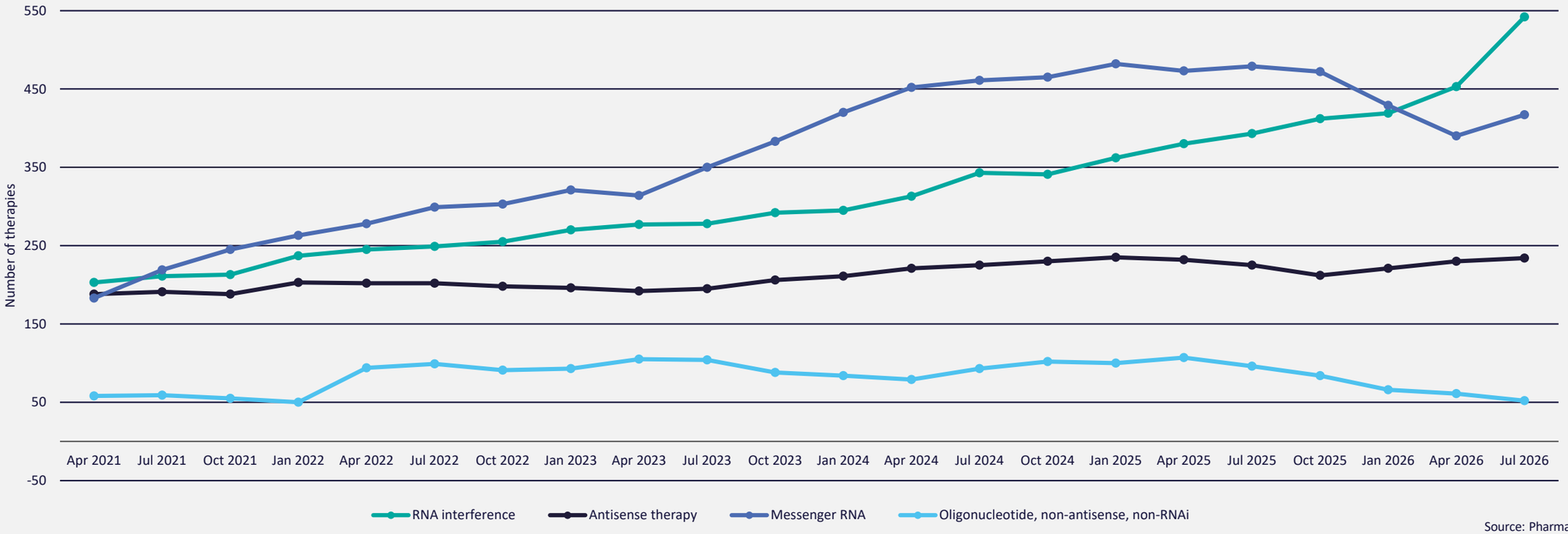
RNA therapy pipeline

RNA therapy pipeline: most common modalities

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



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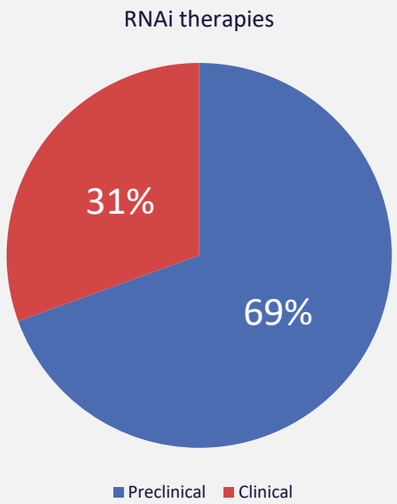
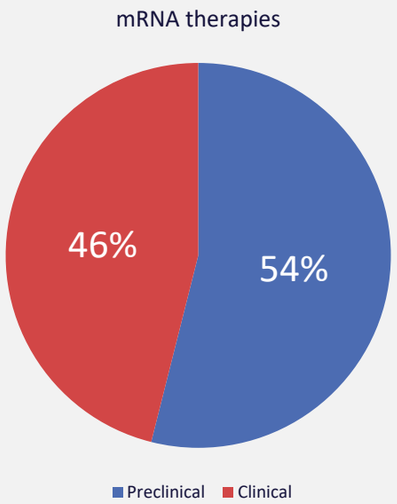
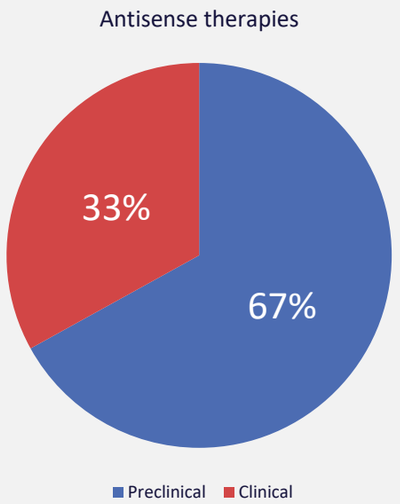
RNAi, mRNA, and antisense oligonucleotides: preclinical vs. clinical

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 69%, 54%, and 67% of their respective pipelines







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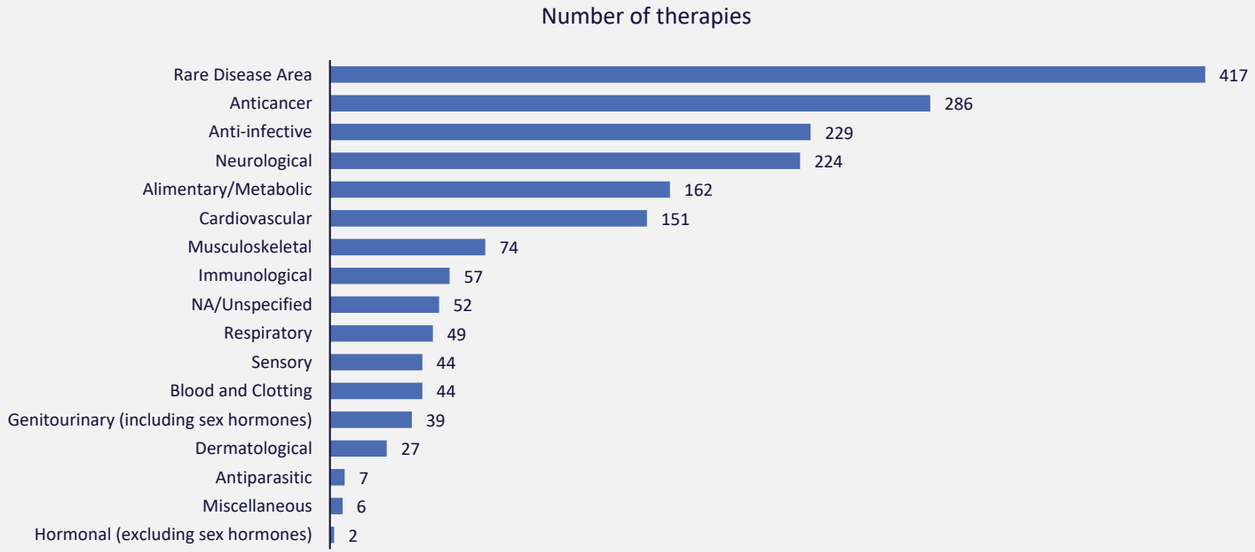
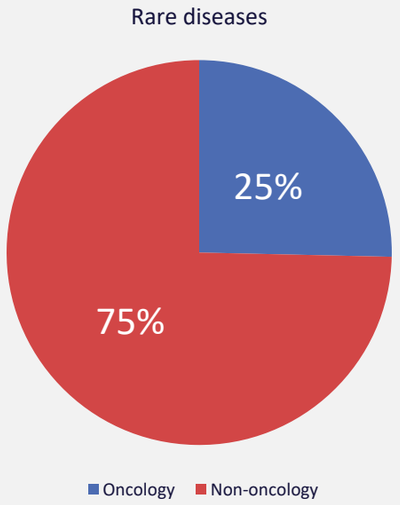
RNA therapies: most commonly targeted therapeutic areas

Of the 1,370 RNA therapies currently in the pipeline (from preclinical through pre-registration):

- Rare diseases remained the top targeted therapeutic area by RNA therapies; Oncology and anti-infective therapies rank second and third, respectively
- 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; Each therapy can be in development across multiple disease areas

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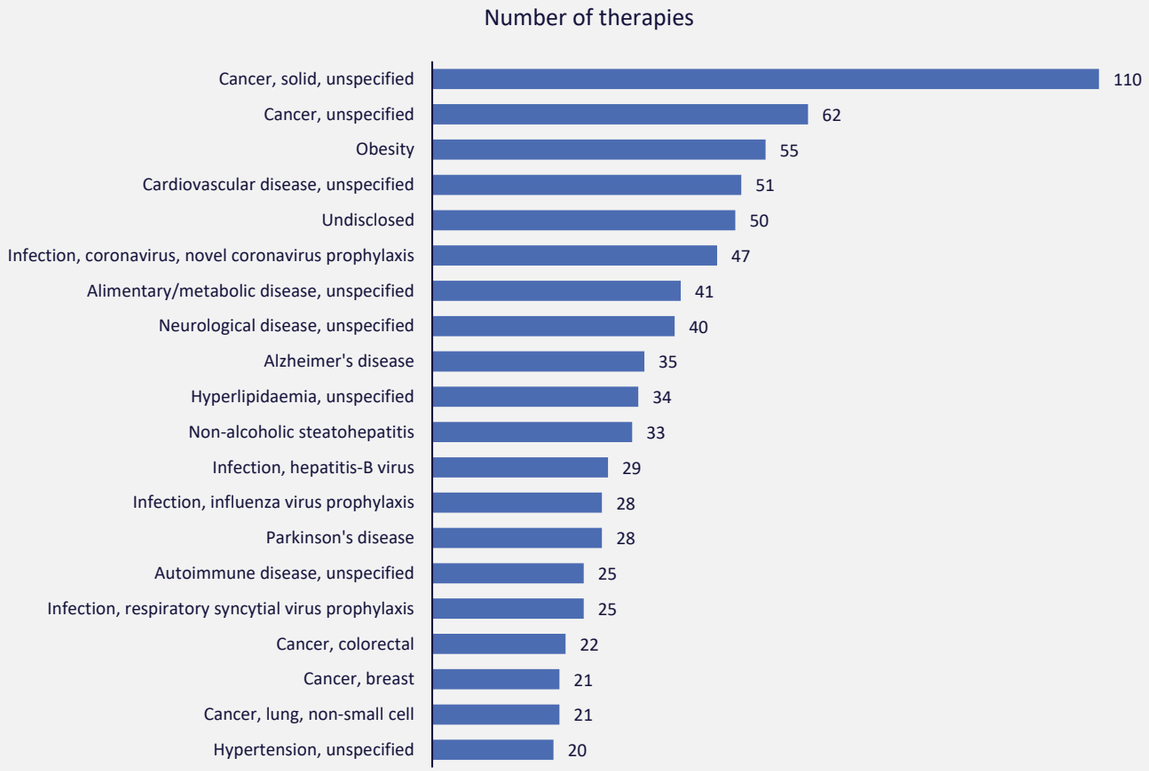
RNA therapies: most common non-rare diseases targeted

Of the therapies for which indications are specified, the most targeted indications in Q2 2026 were:

- Obesity
- Coronavirus prophylaxis
- Alzheimer’s disease



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

Source: Pharmaprojects | Citeline, July 2026

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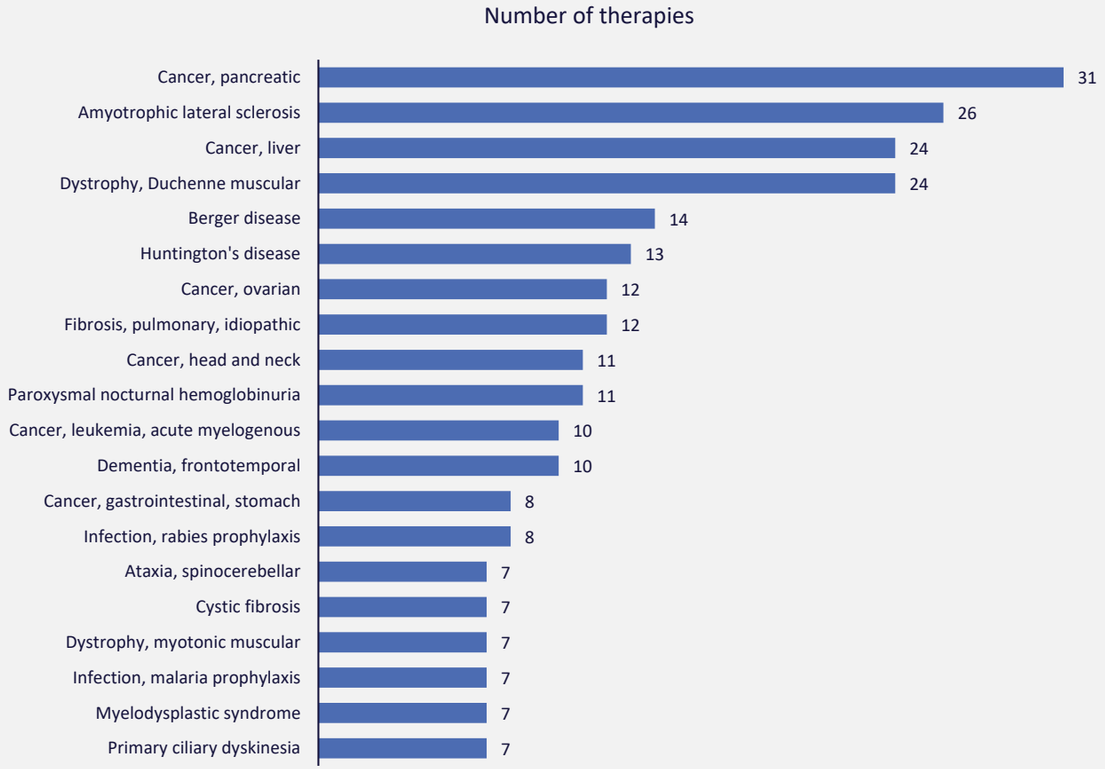
RNA therapies: most common rare diseases targeted

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

- The top specified rare oncology indications were pancreatic, liver, and ovarian
- For non-oncology rare diseases, amyotrophic lateral sclerosis, Duchenne muscular dystrophy, and Berger disease were the most targeted indications



Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple diseases

Source: Pharmaprojects | Citeline, July 2026

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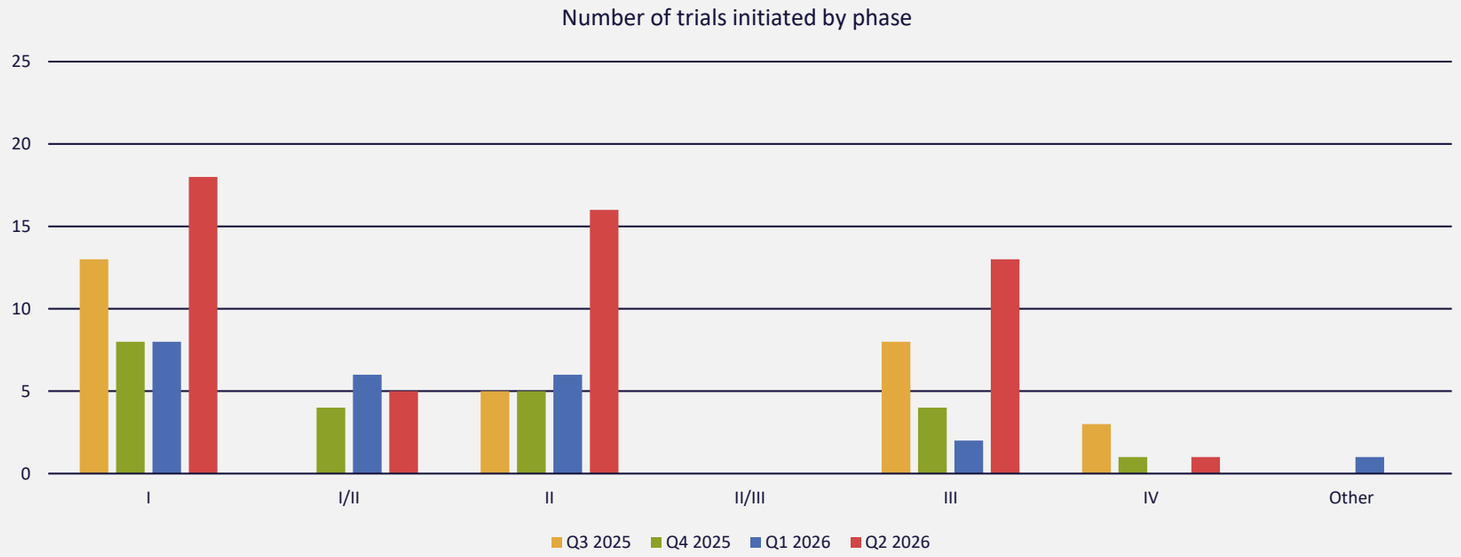
RNA therapy pipeline: clinical trial activity

- 53 RNA trials were initiated in Q2 2026, compared to 22 in Q1 2026, 92% of which were for non-oncology indications
- There are currently 530 open RNA therapy clinical trials



Source: Trialtrove | Citeline, July 2026

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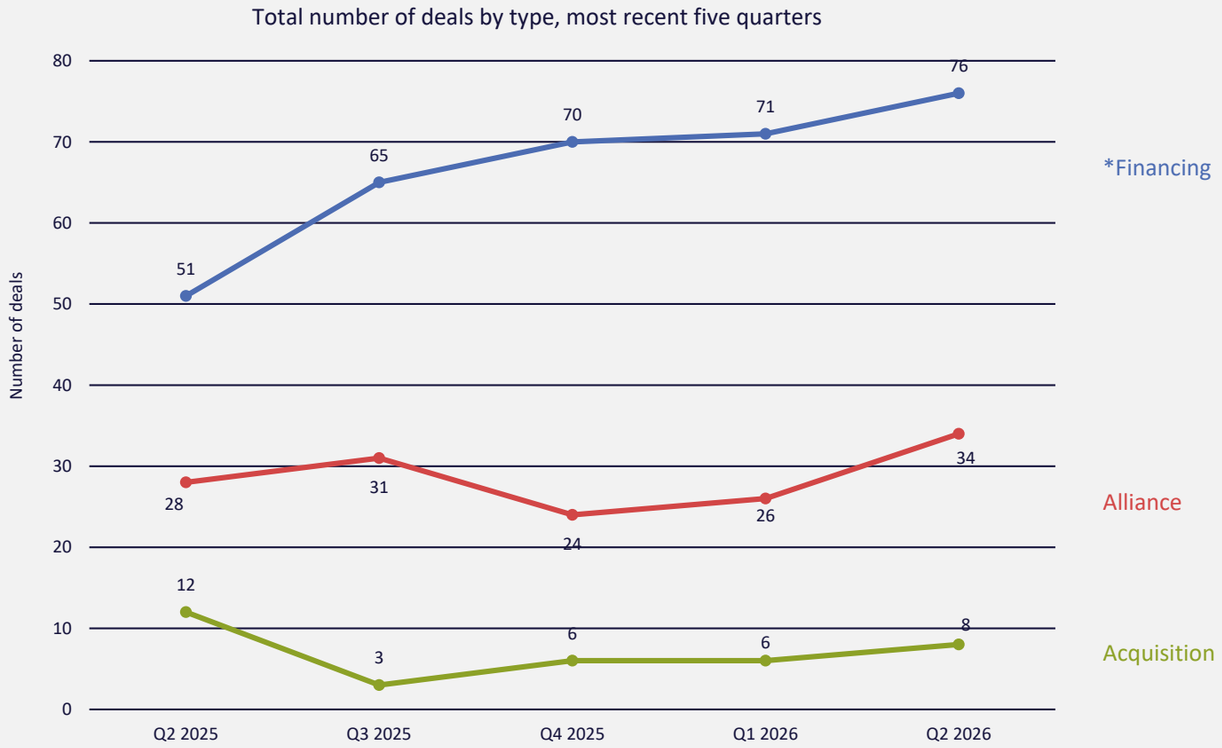
Overview of dealmaking for gene, cell, and RNA therapy companies

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

- Advanced molecular therapy dealmaking rebounded in Q2 2026, with all deal types increasing over the previous quarter
- Q2 2026's total of 118 transactions represented a quarterly high within the last year, jumping 15% from Q1's 103 deals, and 30% over the same quarter in 2025
- Alliances saw the biggest net increase in Q2 2026, with eight more partnerships than 2026's opening quarter



*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 2026

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Q2 2026 acquisitions in gene, cell, and RNA therapy

- Acquisitions of advanced molecular therapy companies increased by two transactions to reach eight in Q2 2026
- In addition to its RNAi partnership with Ascidian during Q2, Eli Lilly also acquired Kelonia Therapeutics for $3.25 billion up front plus up to $3.75 billion in earn-outs, for a total of $7 billion
  - Kelonia's *in vivo* gene placement system allows a patient to generate their own CAR-T cells
- Notably, three of the eight deals were reverse mergers, in which private advanced molecular therapy companies Obsidian, Remix Therapeutics, and Serapha Bio have taken on publicly traded status through these transactions

Deal date	Deal title	Potential deal value (USD $)
Apr 14, 2026	Obsidian Therapeutics and Galera Agree to Merge	Undisclosed
Apr 17, 2026	UCB to Acquire Neurona Therapeutics; Acquisition Completed	1,150,000,000
Apr 20, 2026	Eli Lilly to Buy Kelonia for Up To $7B	7,000,000,000
Apr 30, 2026	Leo Pharma Acquires Replay for $50M	50,000,000
May 21, 2026	Liminatus Pharma to Acquire InnocsAI	320,000,000
Jun 01, 2026	T-CURX Acquires Pantherna Therapeutics	Undisclosed
Jun 23, 2026	Serapha Bio to Reverse Merge with Boundless Bio	Undisclosed
Jun 24, 2026	Passage Bio and Remix Therapeutics Announce Merger Agreement	Undisclosed

Sources: Biomedtracker, BioSciDB | Evaluate, July 2026

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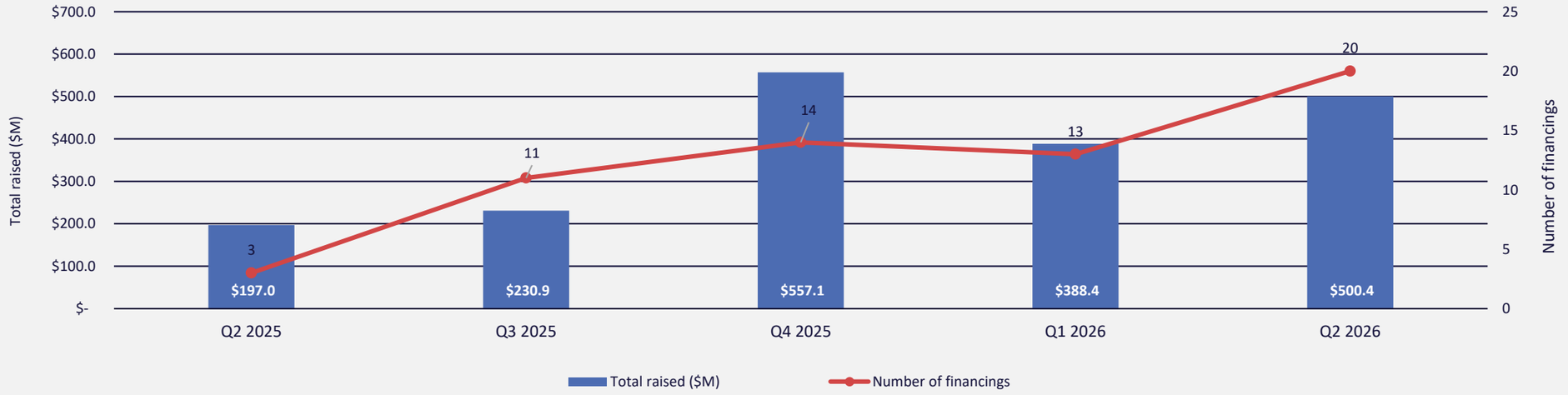
Start-up funding for gene, cell, and RNA therapy companies

- Funding for start-up advanced molecular therapy companies jumped in Q2 2026 to just over $500 million, with 20 seed or Series A financings completed
- Q2's figures increased by 54% in volume and 29% in value over Q1 2026, and represented a marked increase over the same quarter last year, which had three start-up financings totaling $197.0 million

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



Source: Biomedtracker | Evaluate, July 2026

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Q2 2026 start-up financing for gene, cell, and RNA therapy companies (1/2)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
Apr 07, 2026	HexemBio Raises $10.4M in Seed Funding	Cell therapy	United States/New York/New York City	University of Pittsburgh	10.4
Apr 09, 2026	Wiseheart Medval Biotechnology Secures Series A Funding	Cell therapy	China/Beijing	Founded by Chen Zhiguo	Undisclosed
Apr 09, 2026	Mercurna Closes Pre-Seed Financing	mRNA therapy	Netherlands/Oss	Radboud University Medical Center	Undisclosed
Apr 10, 2026	Vivatides Raises $54M in Oversubscribed Series A Round	siRNA therapy and ASOs	United States/Massachusetts/Woburn	Founded by former scientists from Arrowhead, Ionis, Alnylam, and Dicerna	54.0
Apr 14, 2026	Evolyra Completes $5M Seed Financing	Gene therapy	United States/Virginia/Richmond	Virginia Commonwealth University	5.0
Apr 21, 2026	Serif Biomedicines Launches with $50M from Flagship	Gene therapy	United States/Massachusetts/Cambridge	Undisclosed (founded by Flagship Pioneering)	50.0
Apr 22, 2026	UniXell Gets $7.33M in Series A+ Financing	Cell therapy	China/Shanghai City	Center for Excellence in Brain Science and Intelligence Technology (Chinese Academy of Sciences)	7.3
Apr 29, 2026	Vivacta Bio Closes $50M Series A and A+ Financing	CAR-T	China/Shanghai	Spun off from Grit Biotherapeutics	50.0
May 06, 2026	Signadori Closes Seed Round	Cell therapy	France/Paris	Gustave Roussy	13.0
May 07, 2026	Scarlet Therapeutics Raises £3.2M in Seed Round	Cell therapy	United Kingdom/Bristol	University of Bristol; NHS Blood and Transplant	4.4
May 08, 2026	ParcelBio Raises $13M in Seed Financing to Advance Next-Generation mRNA Medicines	CAR-T	United States/California/San Francisco	Founded by former scientists at Orbital Therapeutics (acquired by BMS)	13.0
May 21, 2026	NeuraGen Raises ¥100M RMB in Series A Financing	In vitro and *in vivo* cell reprograming	China/Shanghai	Undisclosed	14.7
May 27, 2026	Secretome Therapeutics Raises $30M in Series A Round	Neonatal cardiac progenitor cells	United States/Maryland/Baltimore	University of Maryland, Baltimore	30.0
May 27, 2026	Reprogram Biosciences Closes Seed Financing	mRNA therapy	United States/California/San Carlos	Scientific co-founders are Rustam Esanov and Eziz Kuliyev	Undisclosed
Jun 01, 2026	Waypoint Bio Raises $20M in Series A Financing	CAR-T	United States/New York/New York City	MIT	20.0
Jun 01, 2026	Oak Hill Bio Raises $32.5 Million in Series A Financing	ASOs	United States/Massachusetts/Cambridge	Lead candidate originally developed by Roche	32.5

Source: Biomedtracker | Evaluate, July 2026

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Q2 2026 start-up financing for gene, cell, and RNA therapy companies (2/2)

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value ($M)
Jun 16, 2026	Dioseve Closes JPY 1.45B Funding Round to Advance iPS Cell-Based IVF Support	Technology to induce oocytes and ovarian somatic cells from iPS cells	Japan/Koto-ku	Kyushu University	9.1
Jun 16, 2026	Kopra Bio Raises $9.1M in Seed Financing	Genetically engineered virus	United States/California/San Francisco	University of California, San Francisco	9.1
Jun 17, 2026	Spot Biosystems Launches With $40M	Gene therapy	United States/California/San Francisco	Stanford Health Care	40.0
Jun 23, 2026	Serapha Bio Gets $138M in Series A Round	Gene editing	United States/California/San Diego	Technology licensed from YolTech	138

Source: Biomedtracker | Evaluate, July 2026

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Notable Q2 2026 start-up gene, cell, and RNA therapy companies

Company details	Academic source	Financing type/amount raised	Lead investor(s)	Therapy areas of interest
In vivo base editing	Technology licensed from YolTech	Series A/$138M	RA Capital Management; RTW Investments	Alpha-1 antitrypsin deficiency
Advanced technologies to enable extrahepatic delivery of RNA therapeutics, including siRNA and antisense oligonucleotides	Founded by former scientists from Arrowhead, Ionis, Alnylam, and Dicerna	Series A/$54M	Qiming Venture Partners; undisclosed leading industry fund	Cardiovascular, metabolic, and oncology
DNA modification by altering structural and chemical form to produce non-viral DNA medicines that are programmable, scalable, durable, and redosable	Undisclosed (founded by Flagship Pioneering)	"Launch" funding/$50M	Flagship Pioneering	Rare genetically defined diseases
In vivo CAR-T therapy	Spun off from Grit Biotherapeutics	Series A and Series A+/$50M	Loyal Valley Capital; Decheng Capital	Hematologic malignancies and autoimmune conditions

Source: Biomedtracker | Evaluate, July 2026

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Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q3 2026

Therapy	Generic name	Disease	Catalyst	Catalyst date
Orca-T		Acute Lymphoblastic Leukemia (ALL)	PDUFA for BLA - First Review	Jul 6, 2026
Orca-T		Acute Myelogenous Leukemia (AML)	PDUFA for BLA - First Review	Jul 6, 2026
Orca-T		Hematologic Cancer	PDUFA for BLA - First Review	Jul 6, 2026
Orca-T		Myelodysplastic Syndrome (MDS)	PDUFA for BLA - First Review	Jul 6, 2026
deramiocel	deramiocel	Duchenne Muscular Dystrophy (DMD)	FDA Advisory Panel Brief	Jul 27, 2026
deramiocel	deramiocel	Duchenne Muscular Dystrophy (DMD)	FDA Advisory Panel Meeting	Jul 29, 2026
BBM-H901	dalnacogene ponparvovec	Hemophilia B	CHMP (European Panel) Results	Jan 1, 2026 - Jul 31, 2026
BBM-H901	dalnacogene ponparvovec	Hemophilia B	PDUFA for BLA - First Review	Apr 10, 2026 - Jul 31, 2026
isaralgagene civaparvovec	isaralgagene civaparvovec	Fabry's Disease	Rolling BLA Completed	Jun 1, 2026 - Jul 31, 2026
vusolimogene oderparepvec	vusolimogene oderparepvec	Melanoma	FDA Advisory Panel Meeting	Jul 15, 2026 - Jul 31, 2026
vusolimogene oderparepvec	vusolimogene oderparepvec	Melanoma	PDUFA for NDA - Third Review	Jul 31, 2026
deramiocel	deramiocel	Duchenne Muscular Dystrophy (DMD)	PDUFA for BLA - Second Review	Aug 21, 2026
pariglasgene brecaparvovec	pariglasgene brecaparvovec	Glycogen Storage Disease (GSD)	PDUFA for BLA - First Review	Aug 23, 2026
rebisufligene etisparvovec	rebisufligene etisparvovec	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A Syndrome)	PDUFA for BLA - Second Review	Sep 18, 2026
ION-373	zilganersen	Neurology - Other	PDUFA for NDA - First Review	Sep 22, 2026
BBM-H901	dalnacogene ponparvovec	Hemophilia B	Approval Decision (Europe)	Mar 1, 2026 - Sep 30, 2026

Source: Biomedtracker | Evaluate, July 2026

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Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q3 2026 *continued*

Therapy	Generic name	Disease	Catalyst	Catalyst date
INO-3107	human papillomavirus (HPV) vaccine	Respiratory Papillomatosis (RP)	Meeting with FDA	Jul 1, 2026 - Sep 30, 2026
JointStem	mesenchymal stem cells	Osteoarthritis	BLA Filing	Aug 1, 2026 - Sep 30, 2026
NEURONATA-R	lenzumestrocel	Amyotrophic Lateral Sclerosis (ALS)	PDUFA for BLA - First Review	Apr 1, 2026 - Sep 30, 2026
Bepirovirsen	bepirovirsen sodium	Hepatitis B (HBV) Treatment	Approval Decision (Japan)	Jul 1, 2026 - Dec 31, 2026
ENTR-601-50		Duchenne Muscular Dystrophy (DMD)	MAA Submission	Mar 26, 2026 - Dec 31, 2026
JB-101		Liver Transplant Rejection	J-NDA Filing	Oct 20, 2025 - Dec 31, 2026
MDL-101		Congenital Muscular Dystrophy (CMD)	PDUFA for BLA - First Review	Sep 1, 2026 - Dec 31, 2026
SGT-003		Duchenne Muscular Dystrophy (DMD)	Meeting with FDA	Jun 30, 2026 - Dec 31, 2026
Symvess		End-Stage Renal Disease (ESRD)	sNDA Filing	Jul 1, 2026 - Dec 31, 2026
Zemcelpro	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	Nov 25, 2025 - Mar 31, 2027
SA030		Obesity	Pre-IND meeting with FDA	Mar 2, 2026 - Sep 30, 2027
Cartistem		Osteoarthritis	Approval Decision (Japan)	Mar 3, 2026 - Dec 31, 2027

Source: Biomedtracker | Evaluate, July 2026

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Appendix

Methodology, sources, and glossary of key terms

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

Pipeline and trial data

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

Deal, financing, and catalyst data

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

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Glossary of Key Terms

Therapy type definitions

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

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

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

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

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

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

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

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

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