

Gene, Cell, & RNA Therapy Landscape Report

Q3 2026 Quarterly Data Report

October 2026

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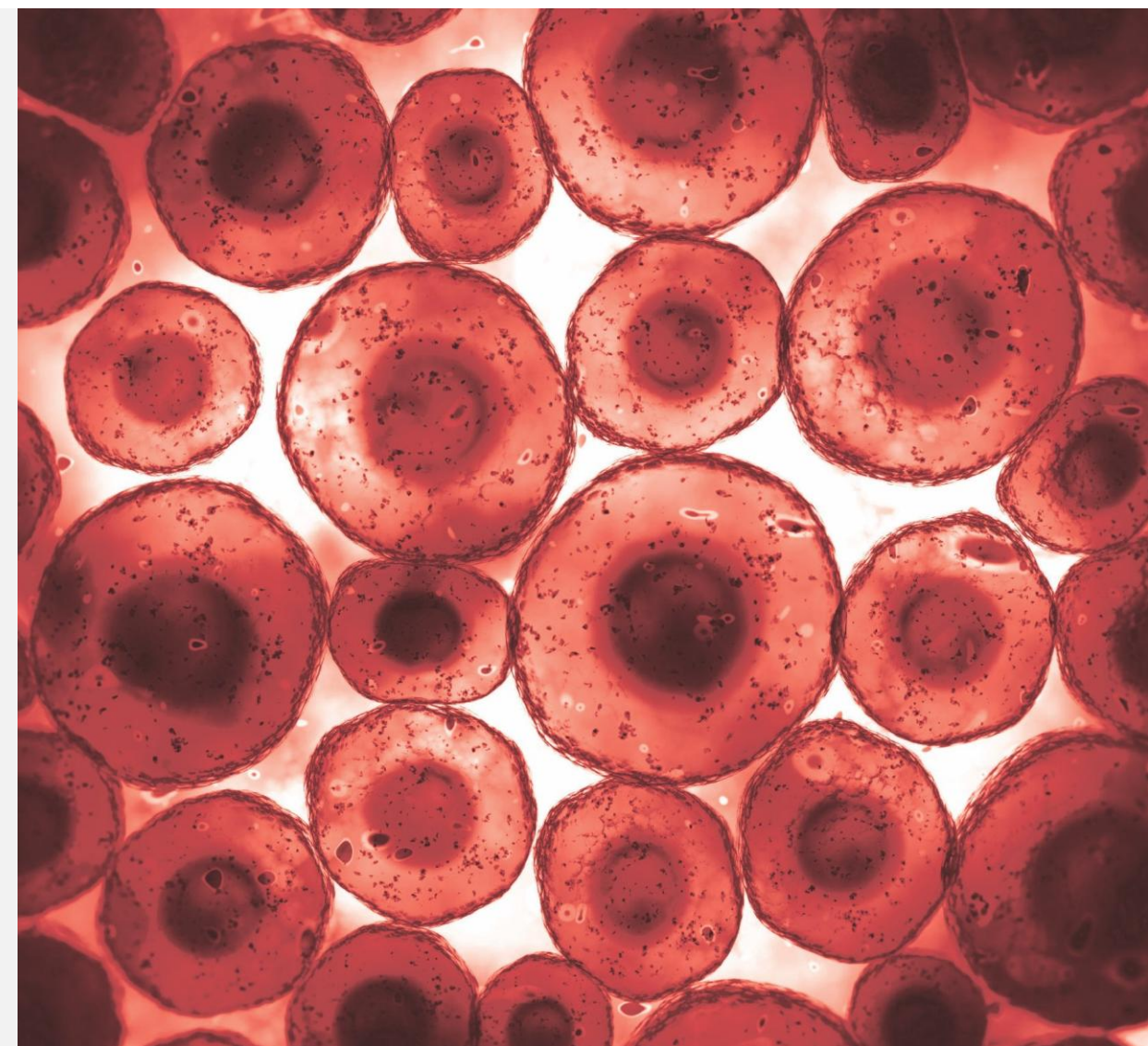
Introduction

The third quarter of 2026 reflected both the continued progress and increasing maturity of the gene, cell, and RNA landscape. Seven new approvals across the US and Japan expanded treatment options for patients with rare diseases, cancer, infectious diseases, and transplant-related conditions. Notable approvals included Fayuvi for mucopolysaccharidosis IIIA (Sanfilippo A syndrome), Genglycos for glycogen storage disease type Ia, Tregzi for hematopoietic and immunologic reconstitution, and Zenvastro for Alexander disease. Together, these milestones underscore the growing breadth of advanced therapeutic modalities reaching the market.

Clinical development remained active, with four gene therapies advancing into Phase III trials and the overall pipeline expanding to 4,594 programs. At the same time, mixed late-stage results among several Angelman syndrome programs highlighted both the promise and uncertainty that accompany the development of first-in-class approvals. Regulatory momentum remained strong, with 11 gene therapies currently awaiting approval decisions.

In contrast to Q2's surge in investment activity, dealmaking declined 33% quarter over quarter, driven by fewer financings and alliances. Despite this pullback, acquisition activity remained steady and early-stage funding continued to outpace levels seen in the same period last year. Together, these trends point to a sector entering a more disciplined phase of growth, supported by a deep development pipeline, sustained innovation, and continued progress toward delivering transformative therapies to patients.

David Barrett, JD
CEO, ASGCT



Key takeaways from Q3 2026

Seven new approvals across the gene, cell, and RNA categories took place in Q3 2026

- 3 gene therapies were approved:
 - Abeona Therapeutics' Fayuvi (rebisufligene etisparvovec-hopf) in the US for mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A syndrome), in collaboration with Ultragenyx
 - Ultragenyx Pharmaceutical's Genglycos (pariglasgene brecaparvovec-opnr) in the US for glycogen storage disease type Ia
 - Replimune's Tudriqev (vusolimogene oderparepvec-wtpg) in the US for melanoma
- 1 non-genetically modified cell therapy was approved:
 - Orca Bio's Tregzi in the US for hematopoietic and immunologic reconstitution and to improve chronic graft-versus-host disease-free survival
- 3 RNA therapies were approved:
 - Ionis Pharmaceuticals' Hibsago (bepirovirsen) in Japan for hepatitis B (HBV) treatment, in collaboration with GSK
 - Moderna's mFlusiva in the US for influenza prophylaxis
 - Ionis Pharmaceuticals' Zenvastro (zilganersen) in the US for Alexander disease

Three Angelman syndrome antisense therapies reported Phase III updates in Q3, with a mixed picture

- Three therapies in development for Angelman syndrome reported Phase III updates
- Outcomes were mixed, with one program facing setbacks and others only now entering late-stage development
 - Ultragenyx Pharmaceutical's apazunersen did not meet its primary endpoint of change from baseline in Bayley-4 cognitive raw score, as reported in September
 - Ionis Pharmaceuticals' obudanersen completed patient enrollment in July
 - Oak Hill Bio's rugonersen announced first dosing in July; Oak Hill Bio obtained global rights for rugonersen for Angelman syndrome from Roche in 2025
- With no approved therapy for Angelman syndrome in any market, the race to first approval is highly competitive

Dealmaking by advanced molecular therapeutics companies was down in Q3

- Q3 2026 saw transaction volume decline by 33% from the previous quarter, mainly due to lower totals in alliances and financings, while acquisition activity remained steady with eight M&As during the quarter
- Repligen bought cell bioprocessing company BioLife Solutions in the industry's largest acquisition of the quarter, worth \$1.5 billion
- Series A and seed financing activity also slowed in Q3 in both volume and value, totaling \$387.9 million from 14 rounds, but surpassed figures seen in the same quarter in 2025

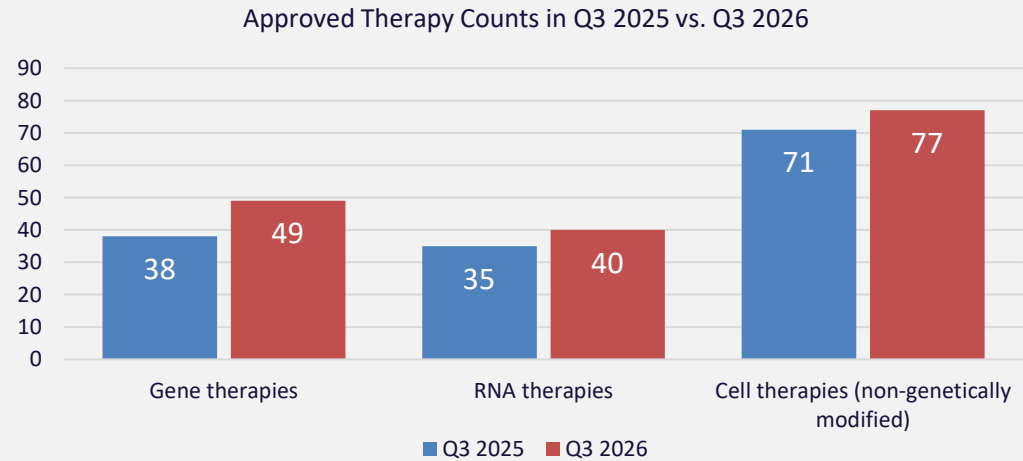
Key highlights in Q3 2026

Approved gene, cell, and RNA therapies

Globally, for clinical use:

- 49 gene therapies have been approved (including genetically modified cell therapies)
- 77 non-genetically modified cell therapies have been approved
- 40 RNA therapies have been approved

30%, 46%, and 24% of the approved advanced therapies have a gene, non-genetically modified cell, and RNA component, respectively



Note: Only the gene and cell classifications are mutually exclusive

This quarter’s approvals are:

Classification	Product name	Generic name	Disease(s)	Locations approved	Originator company
Gene	Fayuvi	rebisufligene etisparvovec-hopf	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A syndrome)	US	Abeona Therapeutics
Gene	Genglycos	pariglasgene breca-parvovec-opnr	Glycogen storage disease type Ia	US	Ultragenyx Pharmaceutical
Gene	Tudriqev	vusolimogene oderparepvec-wtpg	Melanoma	US	Replimune
Non-genetically modified cell	Tregzi	allogeneic regulatory T cell immunotherapy with HSPC and T cells-vldq	For hematopoietic and immunologic reconstitution and to improve chronic graft-versus-host disease-free survival	US	Orca Bio
RNA	Hibsago	bepirovirsen	Hepatitis B (HBV) treatment	Japan	Ionis Pharmaceuticals
RNA	mFlusiva	influenza vaccine, mRNA	Infection, influenza virus prophylaxis	US	Moderna
RNA	Zanvastro	zilganersen	Alexander disease	US	Ionis Pharmaceuticals

Source: Pharmaprojects | Citeline, October 2026

Approved gene therapies as of Q3 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, 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)

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Approved gene therapies as of Q3 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 exuparovec	2022	Aromatic L-amino acid decarboxylase (AADC) deficiency	EU, UK, Israel, US, Brazil	PTC Therapeutics
Roctavian	valoctocogene roxaparovec	2022	Hemophilia A	EU, US, Brazil	BioMarin
Hemgenix	etranacogene dezaparovec	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 moxeparovec	2023	Duchenne muscular dystrophy	US, Brazil, UAE, 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, UAE, Bahrain, Saudi Arabia, EU, Canada, Switzerland, Qatar	CRISPR Therapeutics
Yorwida	inaticabtagene autoleucel	2023	Acute lymphocytic leukemia; Large B cell lymphoma	China	Juventas Cell Therapy

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Approved gene therapies as of Q3 2026*continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Lyfgenia	lovotibeglogene autotemcel	2023	Sickle cell anemia	US	Genetix Biotherapeutics (formerly bluebird bio)
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
Huasueling	donaperminogene seltoplasmid	2026	Limb ischemia	China	Helixmith
Telomelysin	suratadenoturev	2026	Esophageal cancer	Japan	Oncolys BioPharma

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Approved gene therapies as of Q3 2026 *continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Satri-cel	satricabtagene autoleucel	2026	Gastric/gastroesophageal junction adenocarcinoma	China	CARsgen Therapeutics
Fayuvi NEW	rebisufligene etisparvovec-hopf	2026	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A syndrome)	US	Abeona Therapeutics
Genglycos NEW	pariglasgene brecaaparvovec-opnr	2026	Glycogen storage disease type Ia	US	Ultragenyx Pharmaceutical
Tudriqev NEW	vusolimogene oderparepvec-wtpg	2026	Melanoma	US	Replimune

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026
NEW = Approved during Q3 2026

Source: Pharamprojects | Citeline, October 2026

Approved RNA therapies as of Q3 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	Duchenne muscular dystrophy	US	Sarepta Therapeutics
Spinraza	nusinersen	2016	Spinal muscular atrophy	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	Duchenne muscular dystrophy	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, UAE, 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	andusomeran	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
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

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Approved RNA therapies as of Q3 2026*continued*

Product name	Generic name	Year first approved	Disease(s)	Locations approved	Originator company
Viltepso	viltolarsen	2020	Duchenne muscular dystrophy	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	Duchenne muscular dystrophy	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
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
Kostaive	zapomeran	2023	Infection, coronavirus, novel coronavirus prophylaxis	Japan, EU, UK	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, Singapore, China, South Korea*	Ionis Pharmaceuticals

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Approved RNA therapies as of Q3 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	2024	Respiratory syncytial virus prophylaxis	US, EU, Canada, Qatar, Taiwan, UAE, UK, Australia, Switzerland, Japan, South Korea	Moderna
Tryngolza	olezarsen	2024	Lipoprotein lipase deficiency; Hypertriglyceridemia	US, EU, Canada, UK	Ionis Pharmaceuticals
Qfitlia	fitusiran	2025	Hemophilia A & B	US	Alnylam
mNexspike	COVID-19 next generation vaccine, Moderna	2025	Infection, coronavirus, novel coronavirus prophylaxis	US, Canada, Australia, EU, Japan	Moderna
Dawnzera	donidalorsen	2025	Angioedema, hereditary	US, EU, UK, Australia	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
Hibsago NEW	bepirovirsen	2026	Hepatitis B (HBV) treatment	Japan	Ionis Pharmaceuticals
mFlusiva NEW	influenza vaccine, mRNA	2026	Infection, influenza virus prophylaxis	US	Moderna
Zanvastro NEW	zilganersen	2026	Alexander disease	US	Ionis Pharmaceuticals

*Asterisks in the Locations and Disease(s) columns represents supplemental approvals during Q3 2026
NEW = Approved during Q3 2026

Source: Pharmaprojects | Citeline, October 2026

Key highlights in Q3 2026

(Noteworthy events that happened in Q3 2026)

Drug	Event Type	Indication	Molecule	Event Date
Purified Exosome Product	Regulatory - Breakthrough Therapy Designation (US)	Diabetic Foot and Other Ulcers	Cellular	Jul 1, 2026
SkinTE	Regulatory - NDA/BLA Filing	Diabetic Foot and Other Ulcers	Cellular	Jul 1, 2026
Tregzi	Regulatory - Approval (US)	Acute Myelogenous Leukemia (AML)	Cellular	Jul 1, 2026
Tregzi	Regulatory - Approval (US)	Myelodysplastic Syndrome (MDS)	Cellular	Jul 1, 2026
ReNu	Regulatory - NDA/BLA Accepted	Osteoarthritis	Cellular	Jul 6, 2026
EO2002	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Other Ophthalmological Indications	Cellular	Jul 7, 2026
VCA-894A	Regulatory - Rare Pediatric Disease (RPD) Designation	Charcot-Marie-Tooth Disease	Antisense	Jul 7, 2026
EA0010	Regulatory - Orphan Drug Designation (US)	Hearing Loss - General	Viral Gene Therapy	Jul 9, 2026
CD3+ T-lymphocytes therapy	Regulatory - Orphan Drug Designation (US)	Brain Cancer (Malignant Glioma; AA and glioblastoma)	Cellular	Jul 10, 2026
Allocetra	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Osteoarthritis	Cellular	Jul 13, 2026
ION337	Regulatory - Fast Track Status	Dravet Syndrome	Antisense	Jul 13, 2026
Wainua	Regulatory - Approval (Emerging Markets)	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy	Antisense	Jul 13, 2026
SP-101	Regulatory - Orphan Drug Designation (Europe)	Cystic Fibrosis (CF)	Viral Gene Therapy	Jul 14, 2026
AFTX-201	Regulatory - Orphan Drug Designation (US)	Cardiomyopathy - Dilated	Viral Gene Therapy	Jul 16, 2026
VNX-202	Regulatory - Fast Track Status	Solid Tumors	Viral Gene Therapy	Jul 16, 2026
PLOW-101	Regulatory - Orphan Drug Designation (US)	Maple Syrup Urine Disease	Viral Gene Therapy	Jul 17, 2026
PLOW-101	Regulatory - Rare Pediatric Disease (RPD) Designation	Maple Syrup Urine Disease	Viral Gene Therapy	Jul 18, 2026
pelareorep	Regulatory - Fast Track Status	Anal Cancer	Other Nucleic Acid	Jul 20, 2026
C-CAR168	Regulatory - PRIME Designation (Europe)	Lupus Nephritis	Cellular	Jul 21, 2026

Source: Biomedtracker | Evaluate, October 2026

Key highlights in Q3 2026 (Noteworthy events that happened in Q3 2026) *continued*

Drug	Event Type	Indication	Molecule	Event Date
C-CAR168	Regulatory - PRIME Designation (Europe)	Systemic Lupus Erythematosus (SLE)	Cellular	Jul 21, 2026
Wiskott-Aldrich syndrome therapy	Regulatory - Orphan Drug Designation (US)	Wiskott-Aldrich Syndrome	Cellular	Jul 22, 2026
ATSN-101	Regulatory - Fast Track Status	Leber's Congenital Amaurosis	Viral Gene Therapy	Jul 23, 2026
ATSN-101	Regulatory - Orphan Drug Designation (Europe)	Leber's Congenital Amaurosis	Viral Gene Therapy	Jul 23, 2026
ATSN-201	Regulatory - Orphan Drug Designation (Europe)	X-Linked Retinoschisis	Viral Gene Therapy	Jul 23, 2026
KRRO-121	Regulatory - Orphan Drug Designation (Europe)	Urea Cycle Disorders and Derangements (UCD)	Oligonucleotide	Jul 24, 2026
VGN-R09b	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Parkinson's Disease (PD)	Viral Gene Therapy	Jul 27, 2026
HTX-001	Regulatory - Fast Track Status	Cardiomyopathy - Hypertrophic	Antisense	Jul 28, 2026
XS-001	Regulatory - Fast Track Status	Parkinson's Disease (PD)	Cellular	Jul 28, 2026
cemacabtagene ansegedleucel	Regulatory - Fast Track Status	Non-Hodgkin's Lymphoma (NHL)	Cellular	Jul 29, 2026
cemacabtagene ansegedleucel	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Non-Hodgkin's Lymphoma (NHL)	Cellular	Jul 29, 2026
hNPC-01	Regulatory - Fast Track Status	Hemorrhagic Stroke / Intracerebral Hemorrhage (ICH)	Cellular	Jul 29, 2026
hNPC-01	Regulatory - Fast Track Status	Traumatic Brain Injury (TBI)	Cellular	Jul 29, 2026
ICEF16	Regulatory - Advanced Therapy Medicinal Product (ATMP) Classification	Fecal Incontinence Treatment	Cellular	Jul 29, 2026
OCU-410	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy	Viral Gene Therapy	Jul 29, 2026
FT-003	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Neovascular (Wet) Age-Related Macular Degeneration (nAMD)	Viral Gene Therapy	Jul 30, 2026
sasineprocel	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Parkinson's Disease (PD)	Cellular	Jul 30, 2026
VIPER-101	Regulatory - Orphan Drug Designation (US)	Adult T cell Leukemia/Lymphoma (ATL)	Cellular	Jul 31, 2026

Source: Biomedtracker | Evaluate, October 2026

Key highlights in Q3 2026 (Noteworthy events that happened in Q3 2026) continued

Drug	Event Type	Indication	Molecule	Event Date
LX-2020	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Dysrhythmia (Arrhythmia)	Viral Gene Therapy	Aug 5, 2026
mFlusiva	Regulatory - Approval (US)	Seasonal Influenza Vaccines	mRNA (messenger RNA)	Aug 5, 2026
DISP-10	Regulatory - Fast Track Status	Colorectal Cancer (CRC)	Cellular	Aug 6, 2026
DISP-10	Regulatory - Fast Track Status	Esophageal Cancer	Cellular	Aug 6, 2026
DISP-10	Regulatory - Fast Track Status	Gastric Cancer	Cellular	Aug 6, 2026
ISX9-CPC	Regulatory - Rare Pediatric Disease (RPD) Designation	Cardiomyopathy - Hypertrophic	Cellular	Aug 6, 2026
Tudriqev	Regulatory - Accelerated/Conditional Approval (US)	Melanoma	Viral Gene Therapy	Aug 6, 2026
Miv-cel	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Multiple Sclerosis (MS)	Cellular	Aug 11, 2026
BBM-D101	Regulatory - Fast Track Status	Duchenne Muscular Dystrophy (DMD)	Viral Gene Therapy	Aug 14, 2026
Genglycos	Regulatory - Accelerated/Conditional Approval (US)	Glycogen Storage Disease (GSD)	Viral Gene Therapy	Aug 19, 2026
ALN-6400	Regulatory - Orphan Drug Designation (Europe)	Von Willebrand Disease	siRNA/RNAi	Aug 20, 2026
Zola-Cel	Regulatory - Orphan Drug Designation (Europe)	Systemic Sclerosis	Cellular	Aug 20, 2026
CK0803	Regulatory - Fast Track Status	Amyotrophic Lateral Sclerosis (ALS)	Cellular	Aug 21, 2026
Tecartus	Regulatory - J-NDA Filing (Japan)	Acute Lymphoblastic Leukemia (ALL)	Cellular	Aug 21, 2026
Tecartus	Regulatory - J-NDA Filing (Japan)	Mantle Cell Lymphoma (MCL)	Cellular	Aug 21, 2026
Hibsago	Regulatory - Approval (Japan)	Hepatitis B (HBV) Treatment	Antisense	Aug 24, 2026
onvuzosiran	Regulatory - Fast Track Status	Hereditary Angioedema (HAE)	siRNA/RNAi	Aug 24, 2026
MZ-1866	Regulatory - Rare Pediatric Disease (RPD) Designation	Neurology - Other	Viral Gene Therapy	Aug 25, 2026
NS-035	Regulatory - Sakigake Designation (Japan)	Muscular Dystrophy - Unspecified	Antisense	Aug 25, 2026

Source: Biomedtracker | Evaluate, October 2026

Key highlights in Q3 2026 (Noteworthy events that happened in Q3 2026) continued

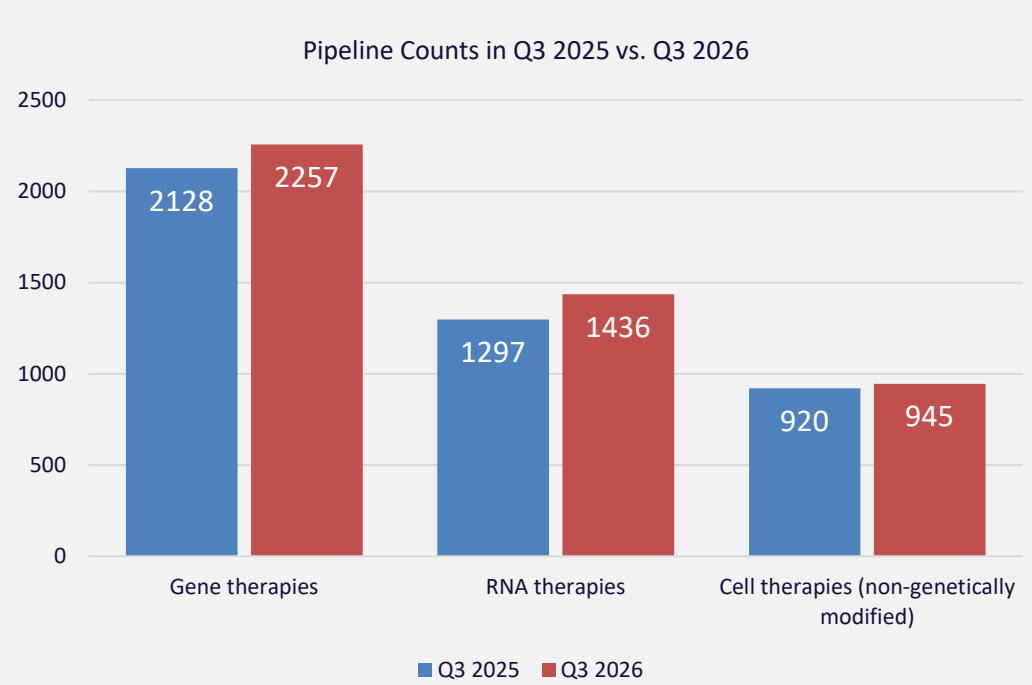
Drug	Event Type	Indication	Molecule	Event Date
puliretgene parvec	Regulatory - Filing for Approval (China)	Other Ophthalmological Indications	Viral Gene Therapy	Aug 25, 2026
puliretgene parvec	Regulatory - PRIME Designation (Europe)	Other Ophthalmological Indications	Viral Gene Therapy	Aug 25, 2026
AAV-TERT-01	Regulatory - Orphan Drug Designation (US)	Idiopathic Pulmonary Fibrosis (IPF)	Viral Gene Therapy	Aug 31, 2026
MT027	Regulatory - Fast Track Status	Brain Cancer (Malignant Glioma; AA and glioblastoma)	Cellular	Aug 31, 2026
ifezuntirgene inilparvovec	Regulatory - NDA/BLA Filing	Huntington's Disease	Viral Gene Therapy	Sep 2, 2026
Zanvastro	Regulatory - Approval (US)	Alexander Disease	Antisense	Sep 3, 2026
AZD-7003	Regulatory - Orphan Drug Designation (US)	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	Cellular	Sep 4, 2026
rAAV9-MECP2	Regulatory - Orphan Drug Designation (US)	Rett Syndrome	Viral Gene Therapy	Sep 4, 2026
BCB-276	Regulatory - Fast Track Status	Brain Cancer (Malignant Glioma; AA and glioblastoma)	Cellular	Sep 8, 2026
Del-zota	Regulatory - Priority Review	Duchenne Muscular Dystrophy (DMD)	Antisense	Sep 8, 2026
RXRG001	Regulatory - Fast Track Status	Xerostomia (Dry Mouth)	Other Nucleic Acid	Sep 8, 2026
RXRG001	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Xerostomia (Dry Mouth)	Other Nucleic Acid	Sep 8, 2026
Mogenry	Regulatory - NDA/BLA Accepted	Retinitis Pigmentosa (RP)	Viral Gene Therapy	Sep 9, 2026
VERVE-201	Regulatory - Orphan Drug Designation (US)	Homozygous Familial Hypercholesterolemia (HoFH)	Viral Gene Therapy	Sep 11, 2026
AP-SA02	Regulatory - Breakthrough Therapy Designation (US)	Staphylococcal Vaccines and Other Staphylococcus-Specific Agents	Cellular	Sep 14, 2026
EG110A	Regulatory - Regenerative Medicine Advanced Therapy (RMAT) Designation	Neurogenic Bladder	Viral Gene Therapy	Sep 14, 2026
SkinTE	Regulatory - Priority Review	Diabetic Foot and Other Ulcers	Cellular	Sep 15, 2026
GEB-101	Regulatory - Fast Track Status	Other Ophthalmological Indications	Cellular	Sep 16, 2026
Fayuvi	Regulatory - Approval (US)	Mucopolysaccharidosis IIIA (MPS IIIA; Sanfilippo A syndrome)	Viral Gene Therapy	Sep 17, 2026

Source: Biomedtracker | Evaluate, October 2026

Pipeline overview

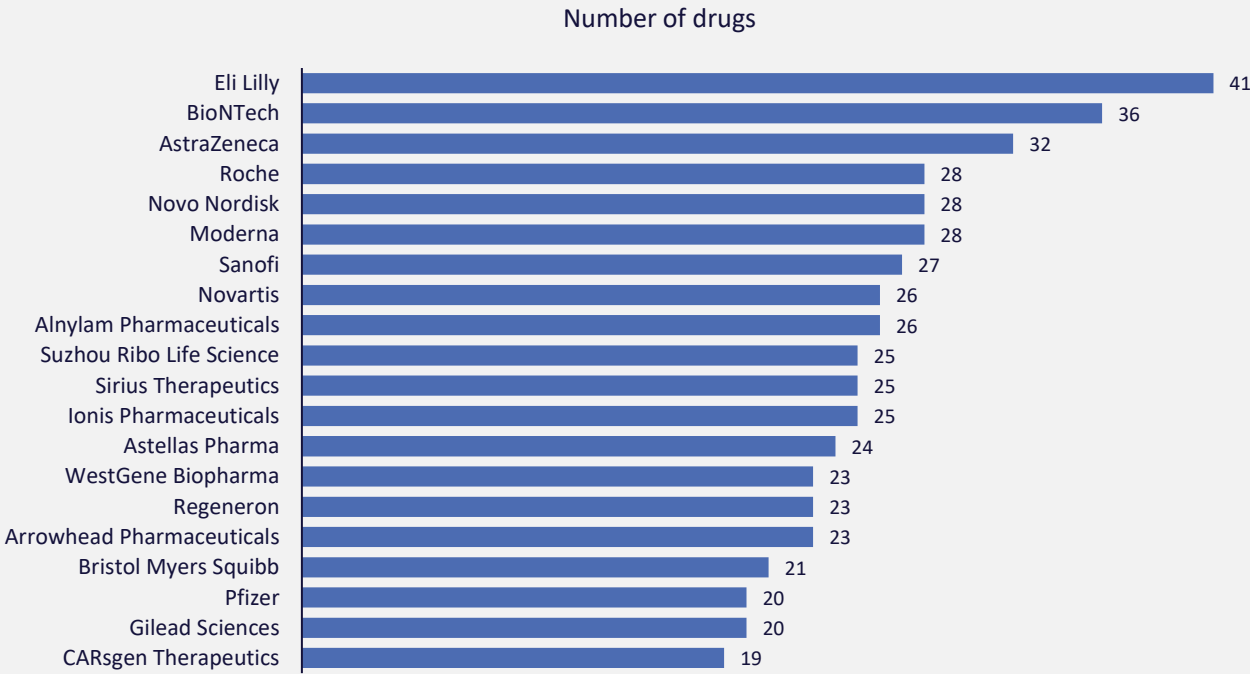
Pipeline of gene, cell, and RNA therapies

4,594 therapies are in development, ranging from preclinical through pre-registration:



Note: Only the gene and cell classifications are mutually exclusive

The top 20 companies by count of therapies in preclinical through pre-registration status are:



Source: Pharmaprojects | Citeline, October 2026

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 pre-registration
- In Q3 2026, four gene therapies advanced to Phase III trials:
 - LX-2006 for Friedreich ataxia
 - OCU-410 for dry age-related macular degeneration
 - OC-1 for T cell acute lymphoblastic leukemia
 - TSC-101 for acute myeloid leukemia or myelodysplastic syndromes

Global Status	Q3 2025	Q4 2025	Q1 2026	Q2 2026	Q3 2026
Preclinical	1,346	1,227	1,269	1,332	1,355
Phase I	377	397	423	427	437
Phase II	347	355	368	387	392
Phase III	47	49	57	59	62
Pre-registration	11	12	15	12	11
Total	2,128	2,040	2,132	2,217	2,257

NEW = Filed during Q3 2026

Gene therapies awaiting approval decisions

- 11 gene therapies are filed and currently awaiting regulatory approval:

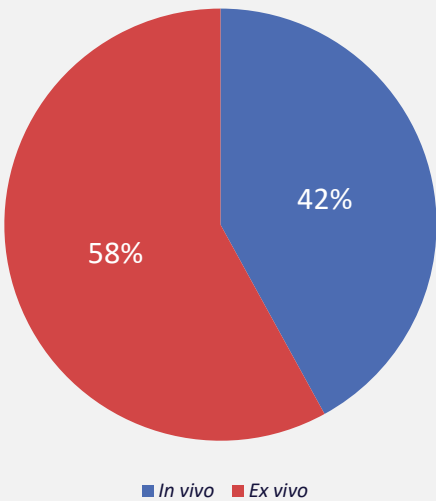
Drug	First filing date	Locations filed	Originator company
IM-19	Mar 13, 2025	China	Imunopharm
sonpirtigene isteparvovec	Jul 14, 2025	US	Nanoscope Therapeutics
cretostimogene grenadenorepvec	Nov 15, 2025	US	CG Oncology
isarlalagene civaparpvovec	Dec 18, 2025	US	Sangamo Therapeutics
anitocabtagene autoleucel	Jan 12, 2026	US	Arcellx
lonvoguran ziclumeran	Apr 27, 2026	US	Intellia Therapeutics
Wanji Aolunsai	Apr 30, 2026	China	Huadao (Shanghai) Biopharma
mivocabtagene autoleucel	May 13, 2026	US	Kyverna Therapeutics
VGR-R01	Jun 23, 2026	China	Vitalgen BioPharma
puliretgene parvec	Aug 25, 2026	China	Chigenovo
ifezuntirgene inilparvovec	Sep 2, 2026	UK	uniQure

Source: Pharamaprojects | Citeline, October 2026

Genetic modification:
In vivo vs. *Ex vivo*

- *Ex vivo* genetic modification is more widely used for gene therapies in pipeline development
- In Q3 2026, *in vivo* delivery techniques were used in 42% of gene therapies

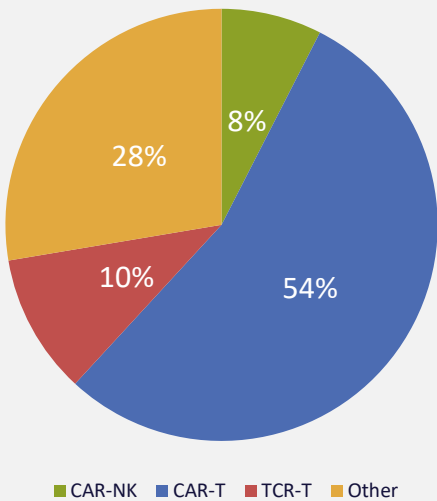
In vivo vs. *Ex vivo* genetic modification



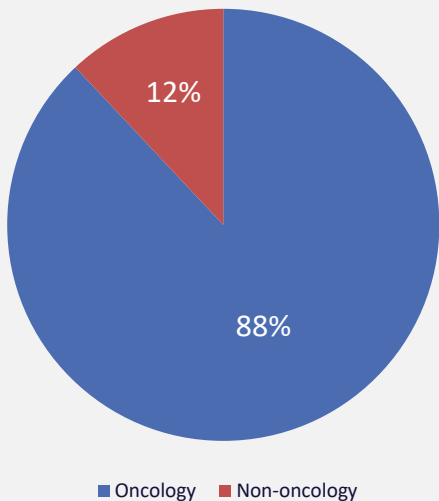
Gene therapy breakdown:
CAR-Ts continue to dominate the pipeline

- CAR-T cell therapies remained the most common technology used in the pipeline of genetically modified cell therapies (preclinical through pre-registration), representing 54%
- There are 729 CAR-T cell therapies in development, with 641 (88%) in development for cancer indications
- The most common non-oncology diseases for CAR-T therapies are lupus, scleroderma, and myasthenia gravis

Genetically modified cell therapy breakdown



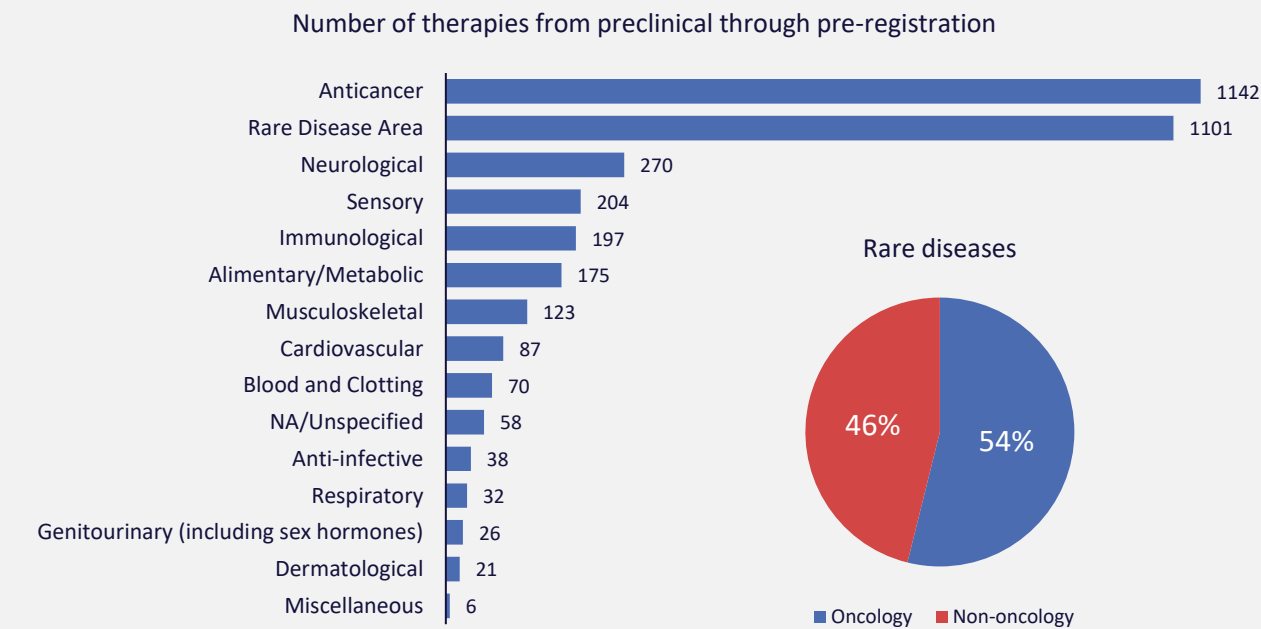
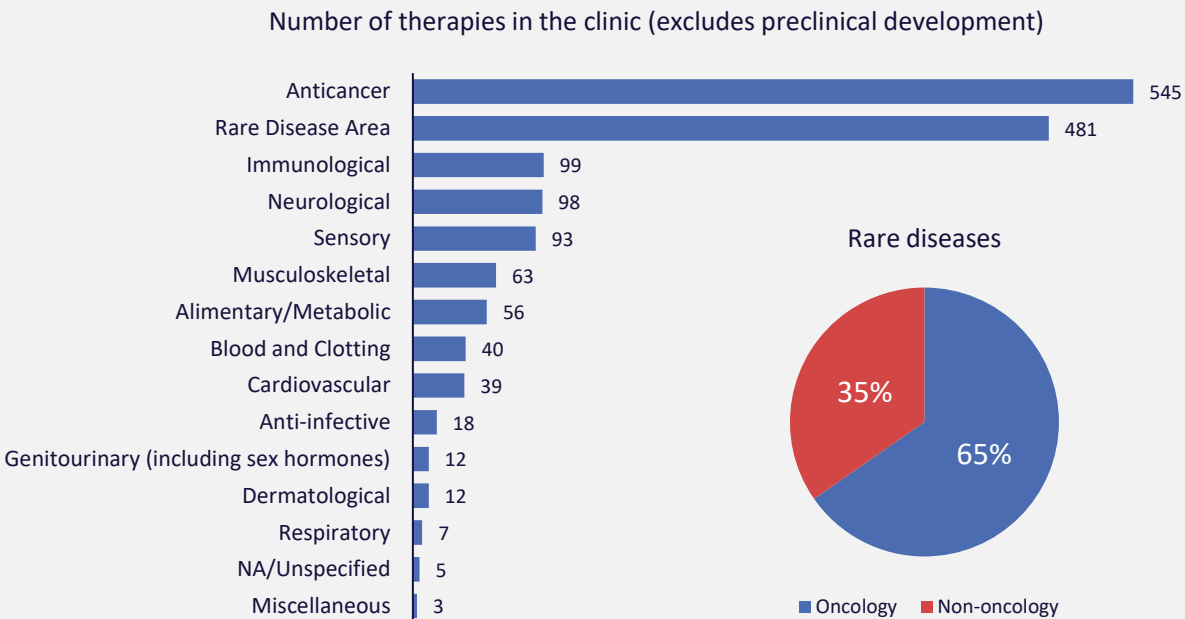
CAR-T breakdown



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

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)
- Oncology still accounts for more than half of all rare gene therapies in the overall pipeline (54%), the same percentage as last quarter
 - The most commonly targeted rare oncology diseases in the overall pipeline are myeloma, non-Hodgkin’s lymphoma, and B cell lymphoma
- Oncology accounts for 65% of all rare gene therapies in the clinic, 11 percentage points higher than in the overall pipeline
 - The most commonly targeted rare oncology diseases in the clinic are non-Hodgkin’s lymphoma, myeloma, and B cell lymphoma

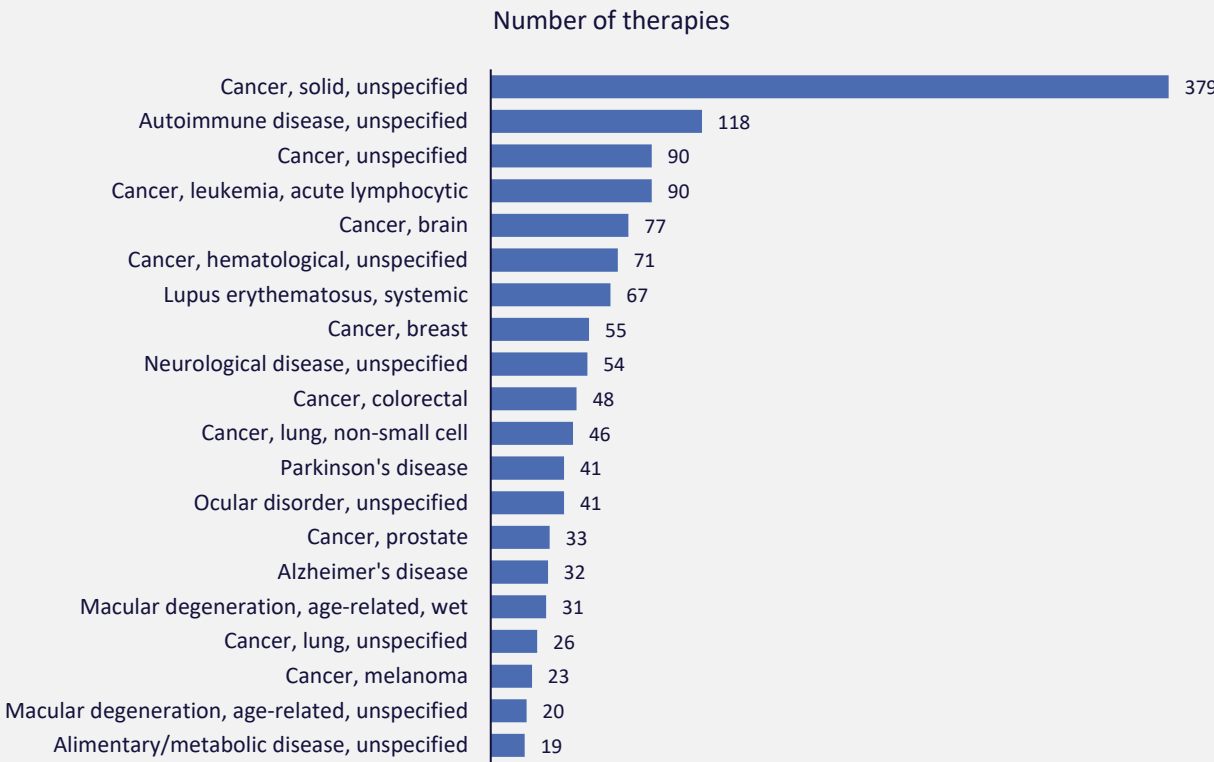


Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple disease areas

Source: Pharmaprojects | Citeline, October 2026

Gene therapy pipeline: most common non-rare diseases targeted

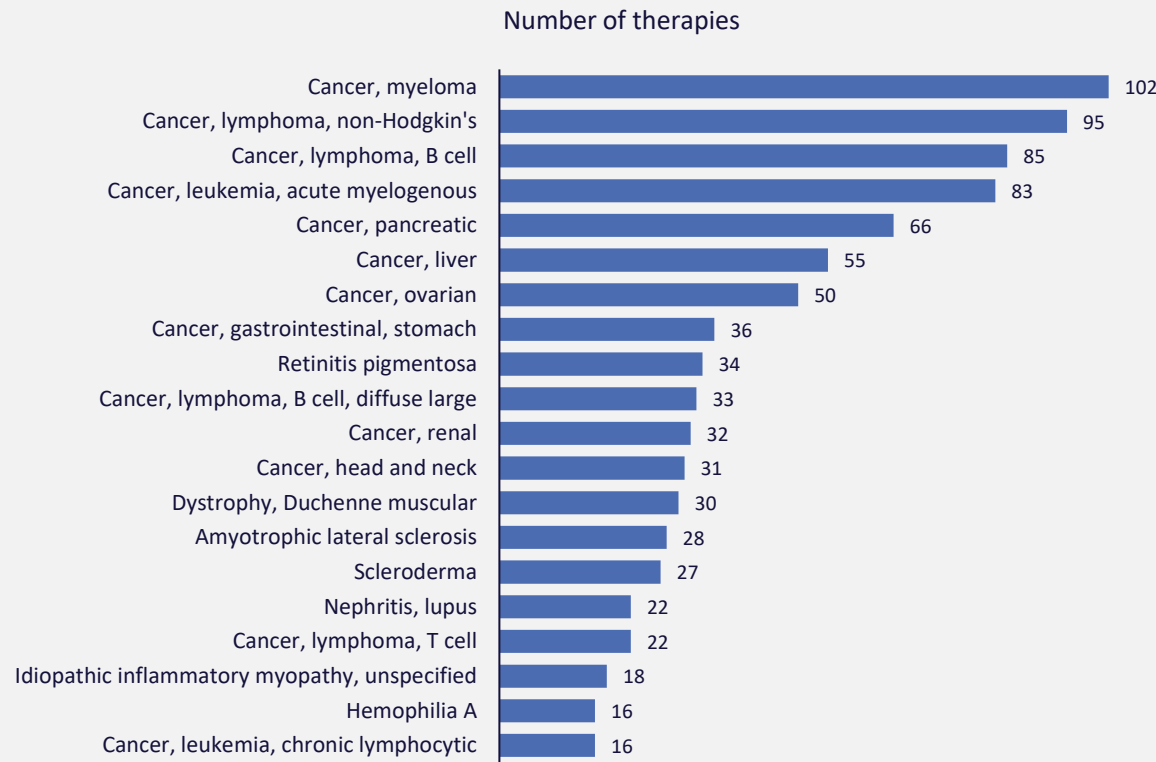
Of the gene therapies for which indications or disease areas are specified, the most targeted non-rare indications in Q3 2026 were:



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

Gene therapy pipeline: most common rare diseases targeted

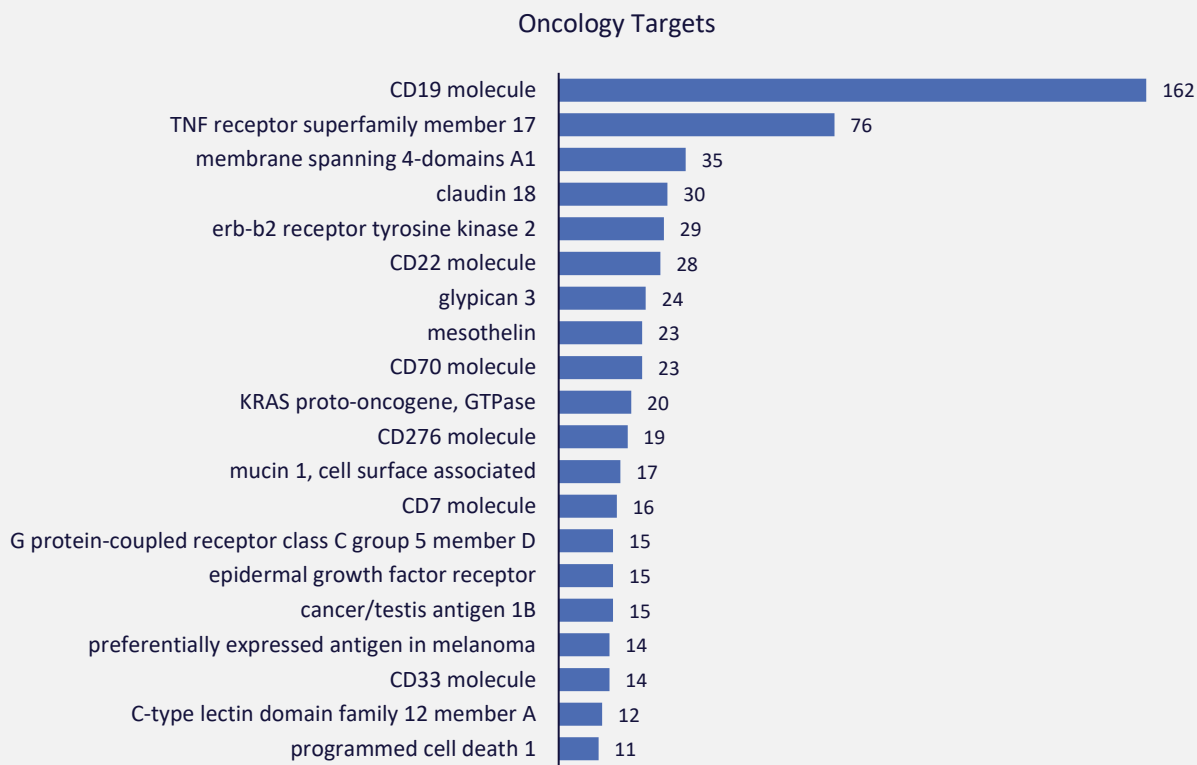
Of the gene therapies for which indications are specified, the most targeted rare indications in Q3 2026 were:



Source: Pharmaprojects | Citeline, October 2026

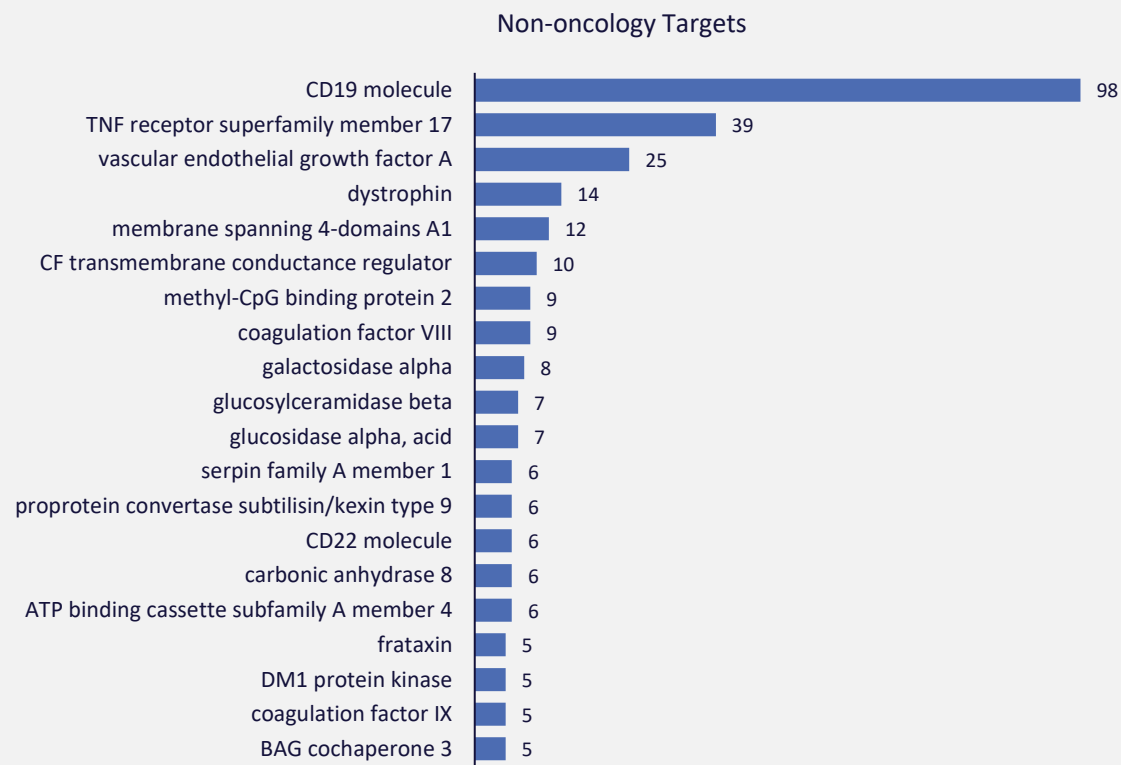
Gene therapy pipeline: most common targets for oncology indications

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



Gene therapy pipeline: most common targets for non-oncology indications

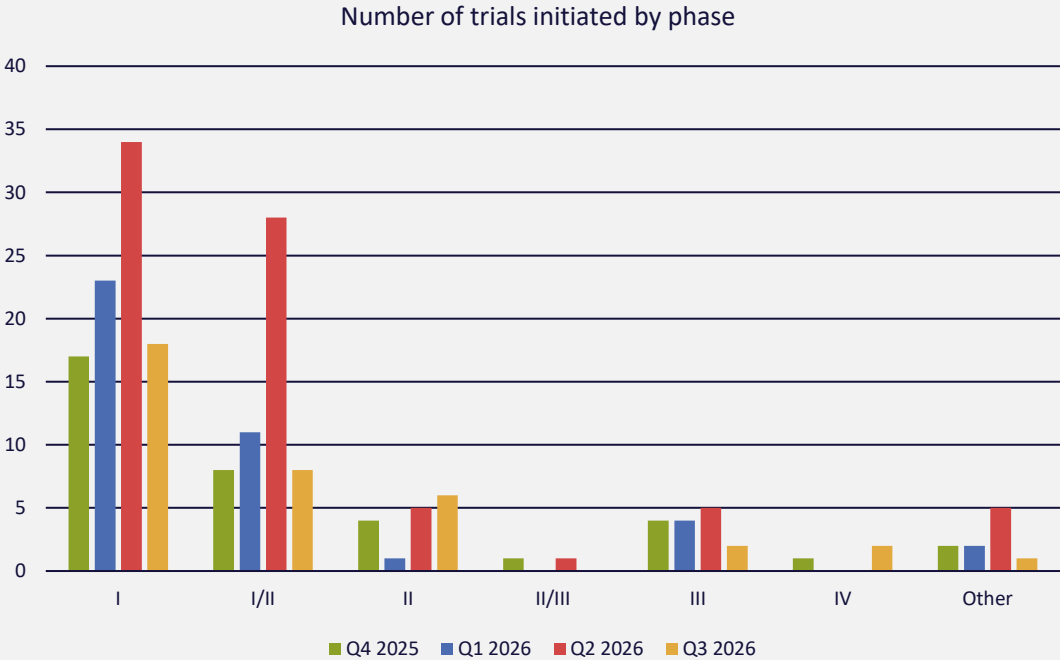
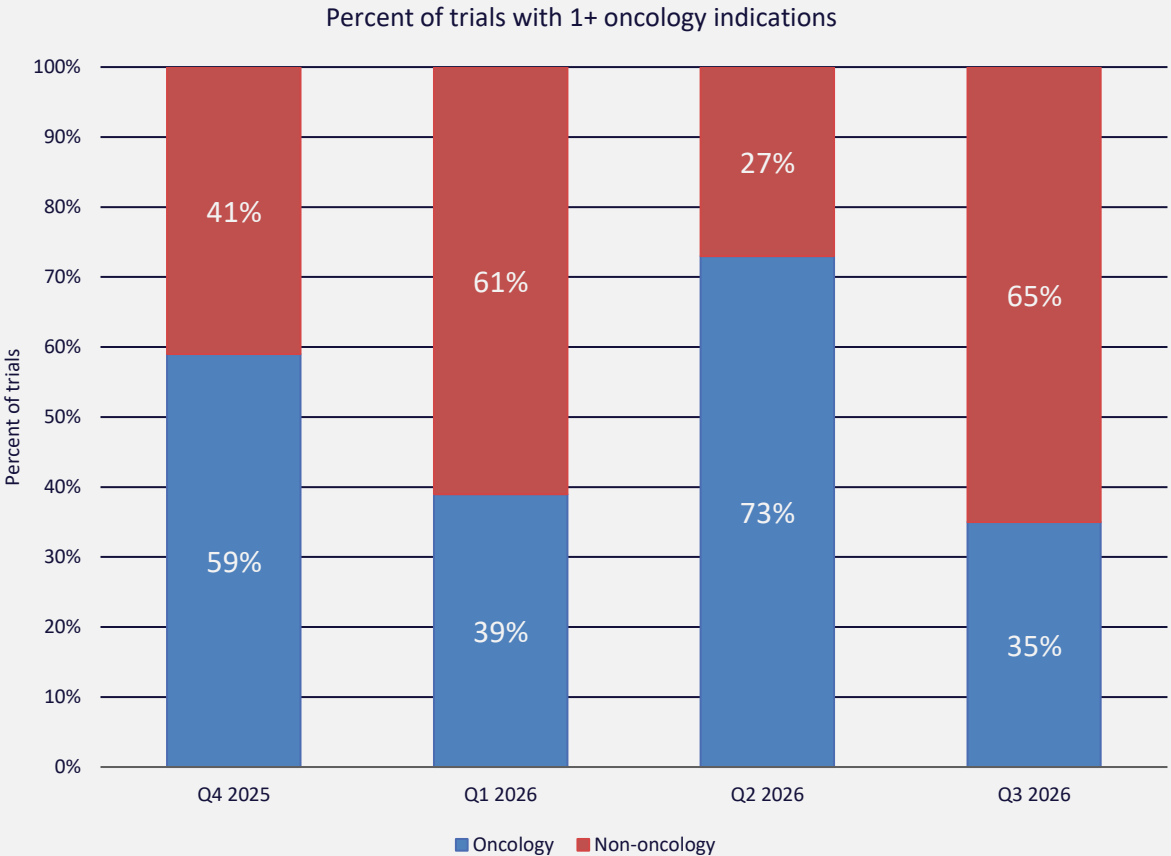
Of the gene therapies at preclinical through pre-registration for which targets were disclosed:



Source: Pharmaprojects | Citeline, October 2026

Gene therapy clinical trial activity

- 37 gene therapy trials were initiated in Q3 2026, 41 fewer than in Q2 2026
- Of these 37, 65% were for non-oncology indications, 38 percentage points higher than last quarter
- There are currently 1,750 open gene therapy clinical trials

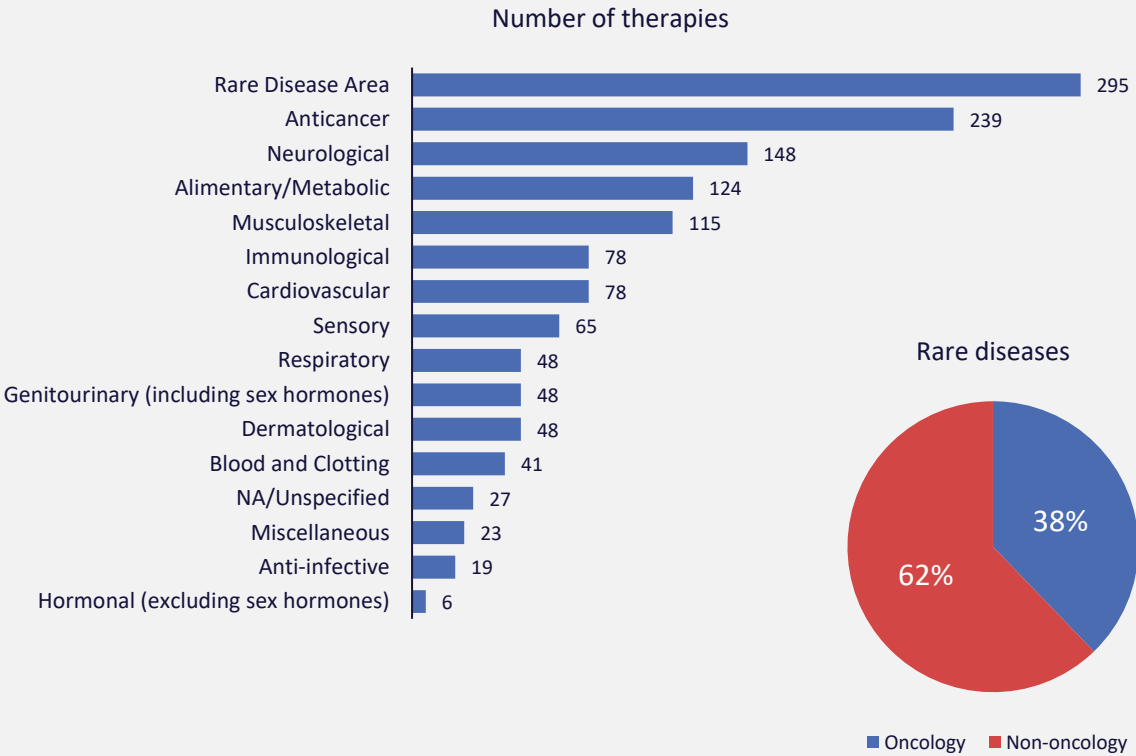


Source: Trialtrove | Citeline, October 2026

Non-genetically modified cell therapy pipeline

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

- 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 (62%) for rare diseases, growing one percentage point from last quarter
 - The most commonly targeted rare non-oncology disease areas are neurological, immunological, and respiratory

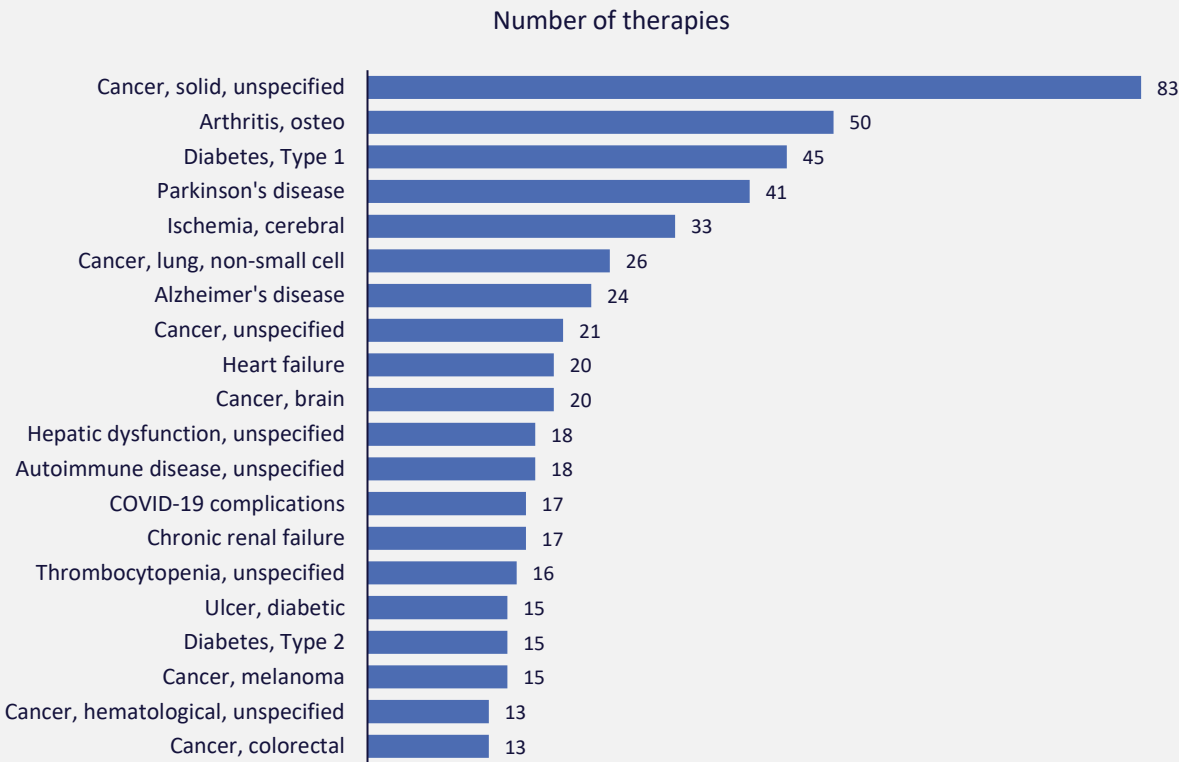


Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple disease areas

Source: Pharamaprojects | Citeline, October 2026

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

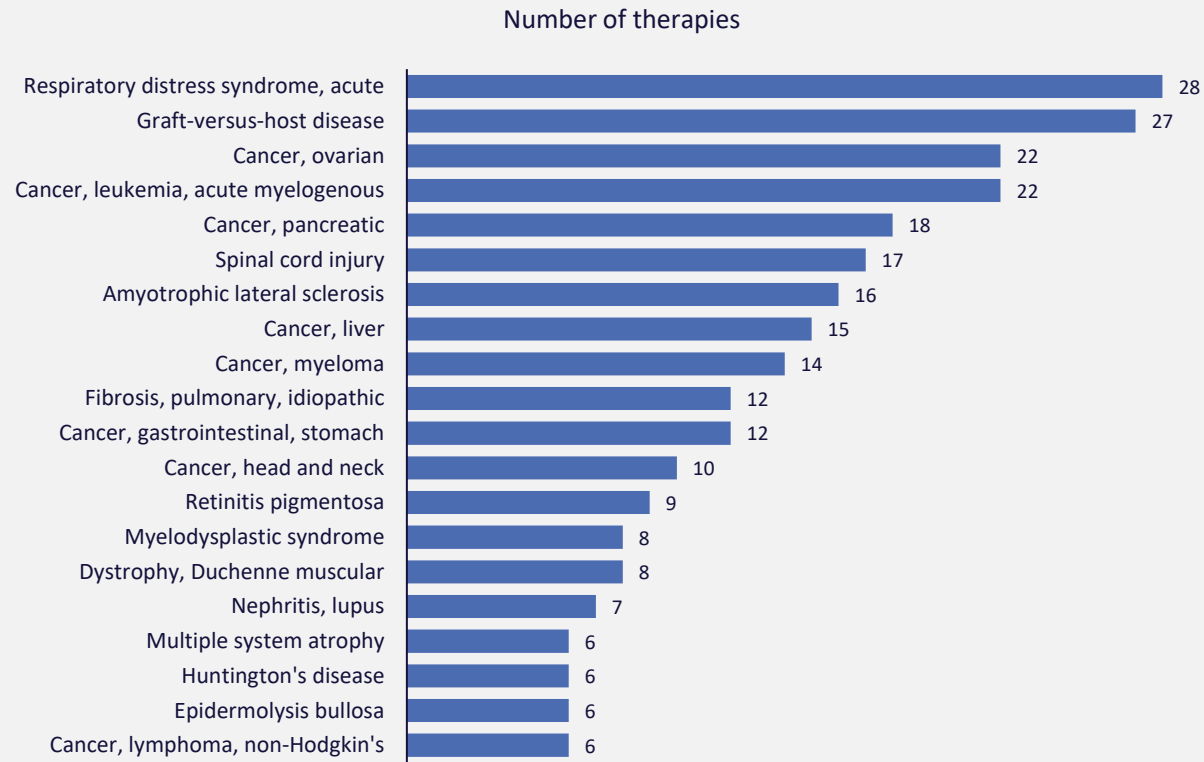
Of the non-genetically modified cell therapies for which indications or disease areas are specified, the most targeted non-rare indications in Q3 2026 were:



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

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

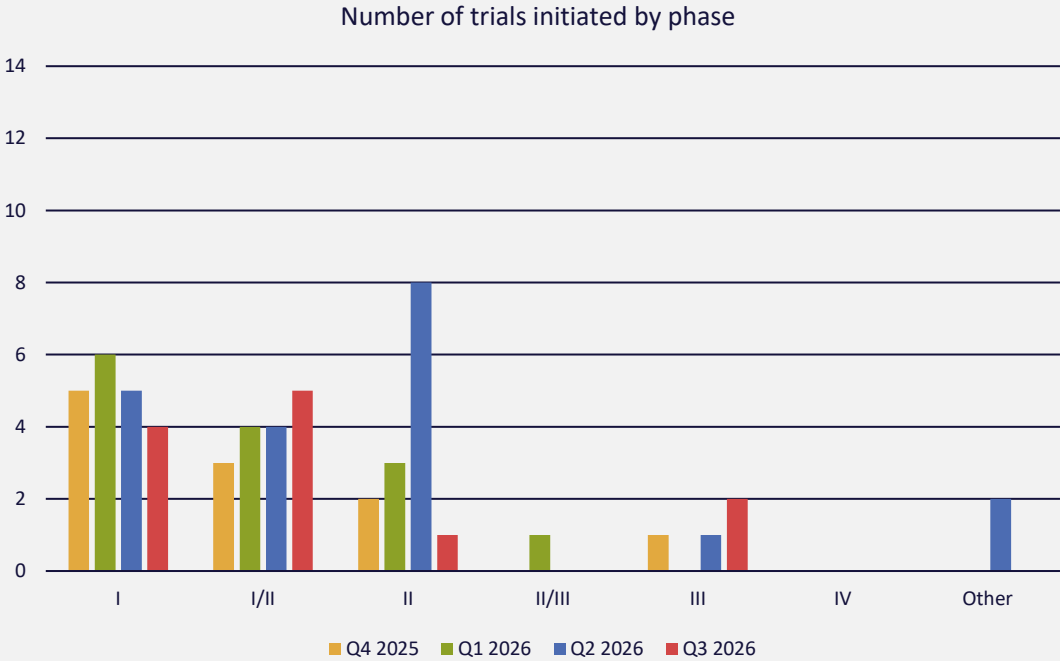
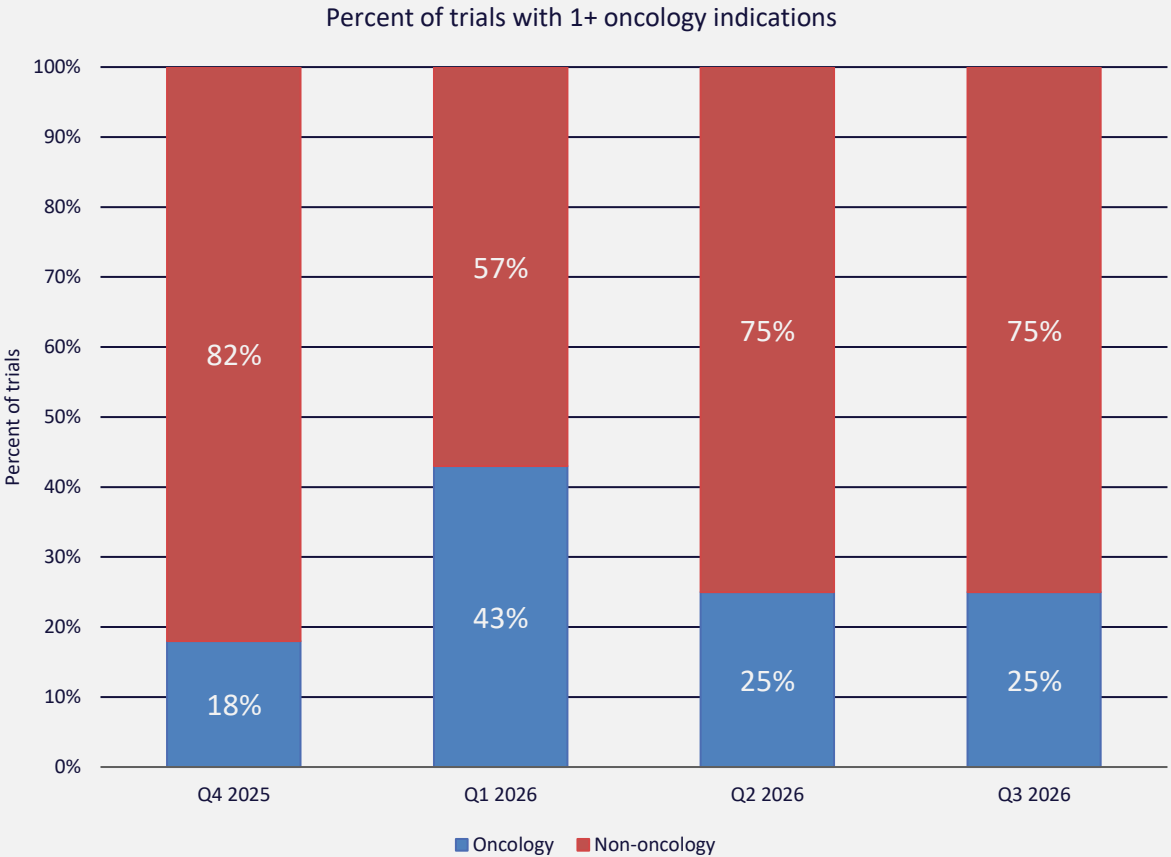
Of the non-genetically modified cell therapies for which indications are specified, the most targeted rare indications in Q3 2026 were:



Source: Pharmaprojects | Citeline, October 2026

Non-genetically modified cell therapy trial activity

- 12 non-genetically modified cell therapy trials were initiated in Q3 2026, eight fewer than in Q2 2026
- Of these 12, 75% were for non-oncology indications, the same as last quarter
- There are currently 646 open non-genetically modified cell therapy clinical trials

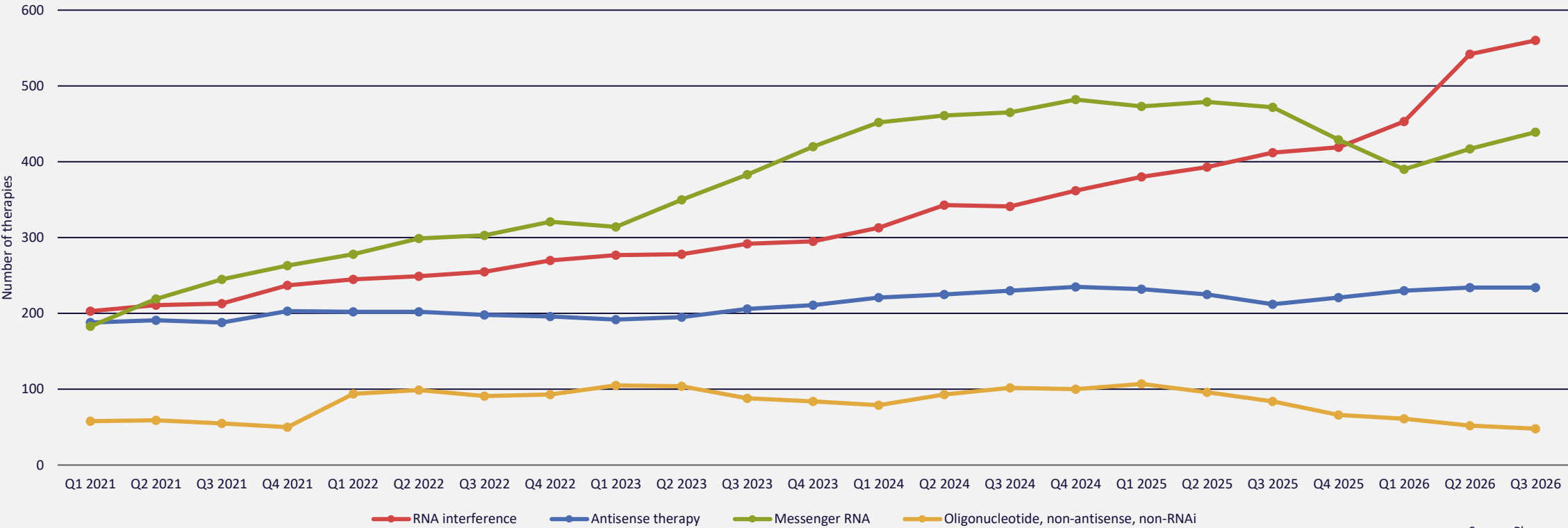


Source: Trialtrove | Citeline, October 2026

RNA therapy pipeline

RNA therapy pipeline: most common modalities

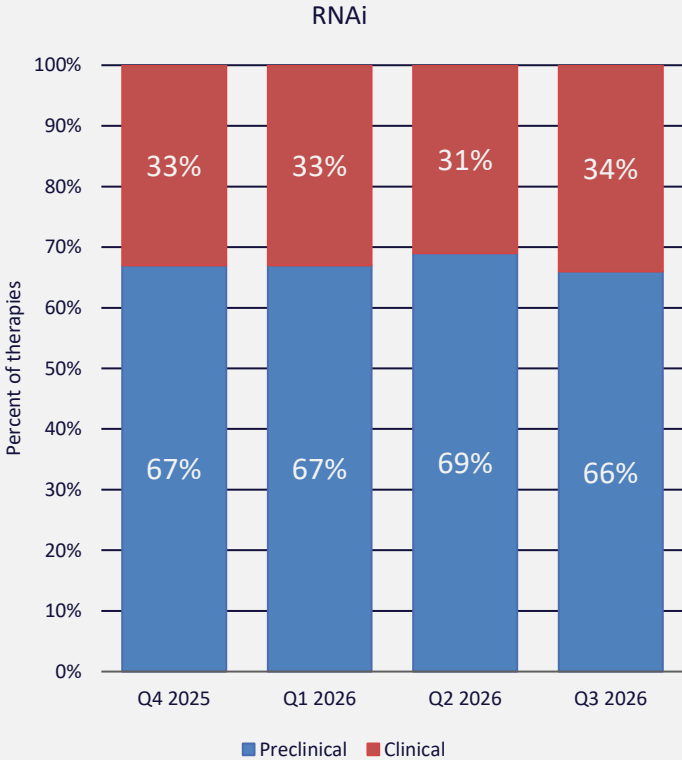
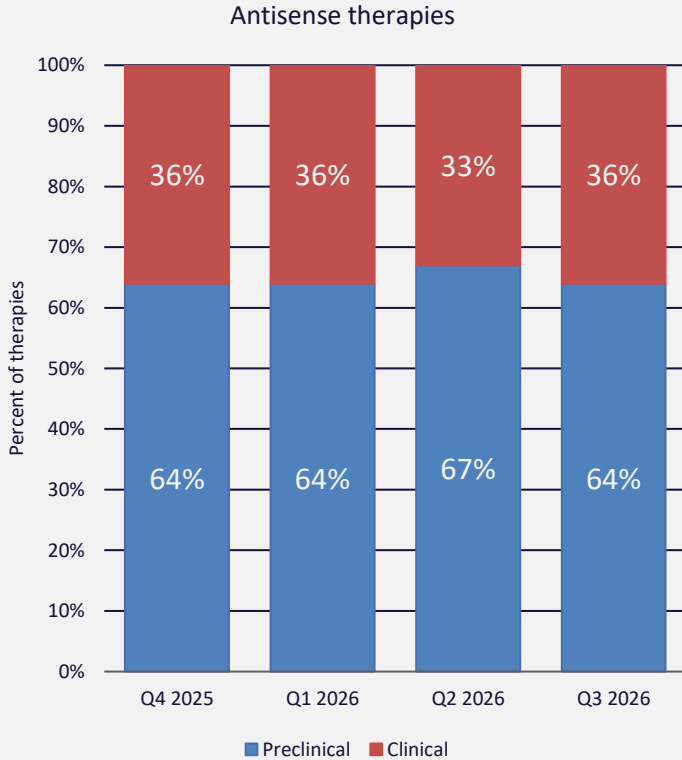
- Of RNA therapies in the pipeline, RNA interference (RNAi) and messenger RNA (mRNA) continued to be the preferred RNA modalities for research



Source: Pharmaprojects | Citeline, October 2026

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

- The majority of RNAi, mRNA, and antisense therapies in development were in the preclinical stage, representing 66%, 60%, and 64% of their respective pipelines

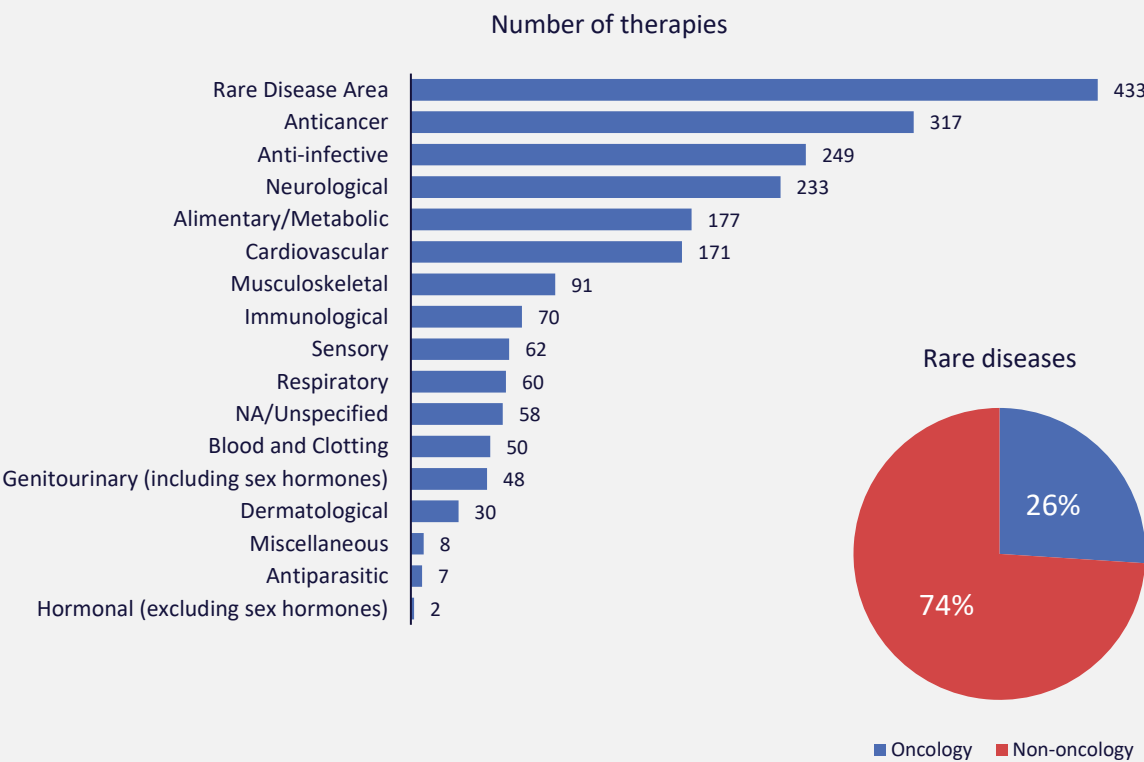


Source: Pharmaprojects | Citeline, October 2026

RNA therapies: most commonly targeted therapeutic areas

Of the 1,436 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 74%

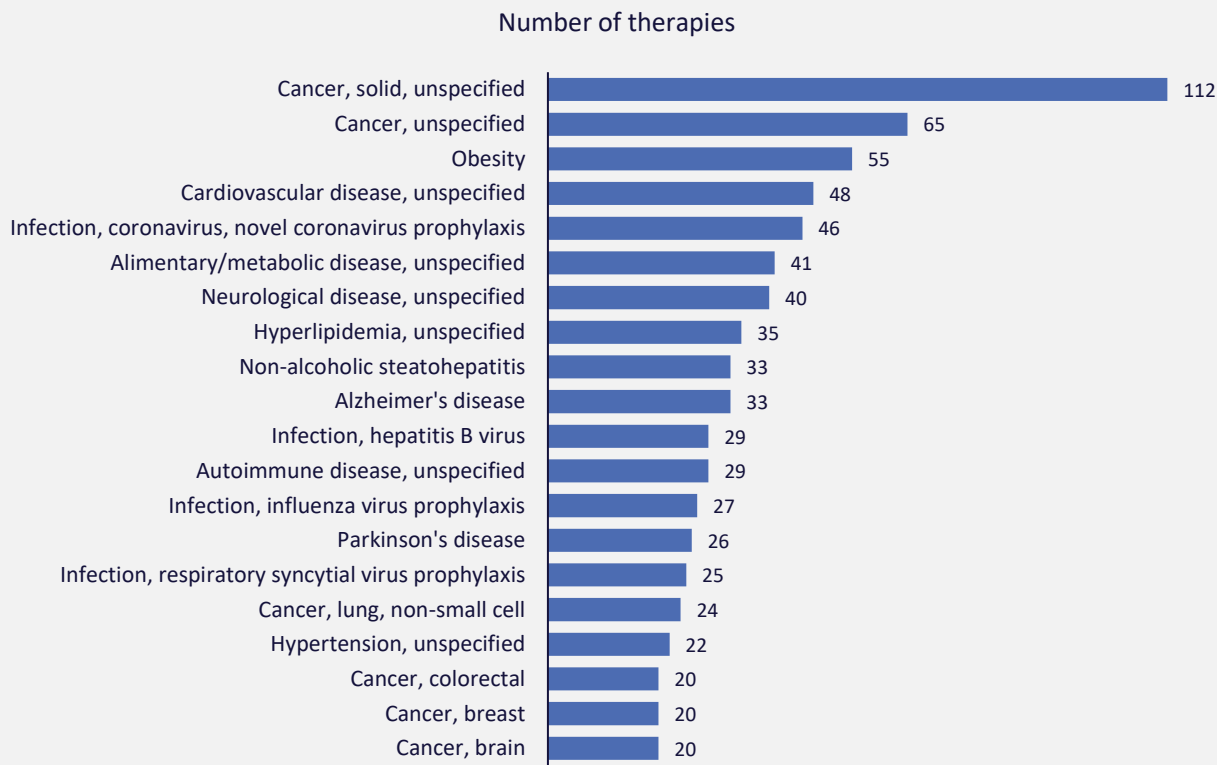


Note: Figures based on indications in pipeline development only for each therapy; Each therapy can be in development across multiple disease areas

Source: Pharamaprojects | Citeline, October 2026

RNA therapies: most common non-rare diseases targeted

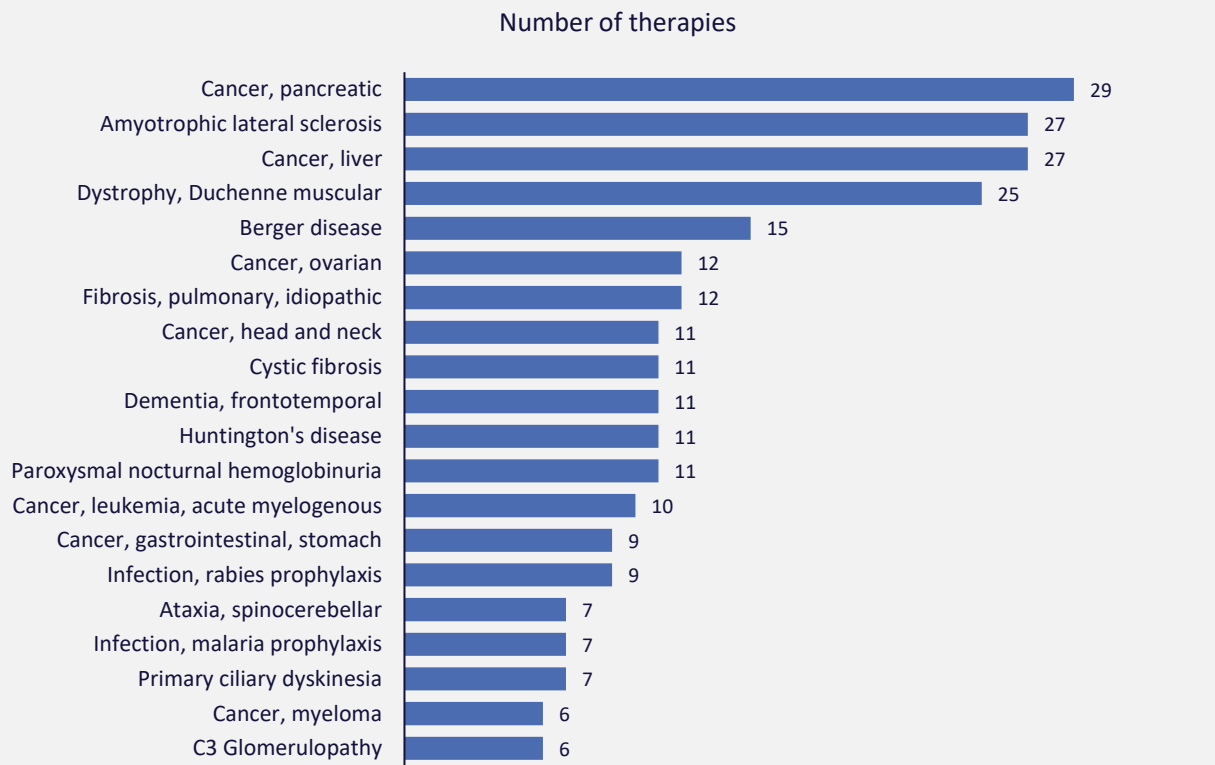
Of the RNA therapies for which indications or disease areas are specified, the most targeted non-rare indications in Q3 2026 were:



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

RNA therapies: most common rare diseases targeted

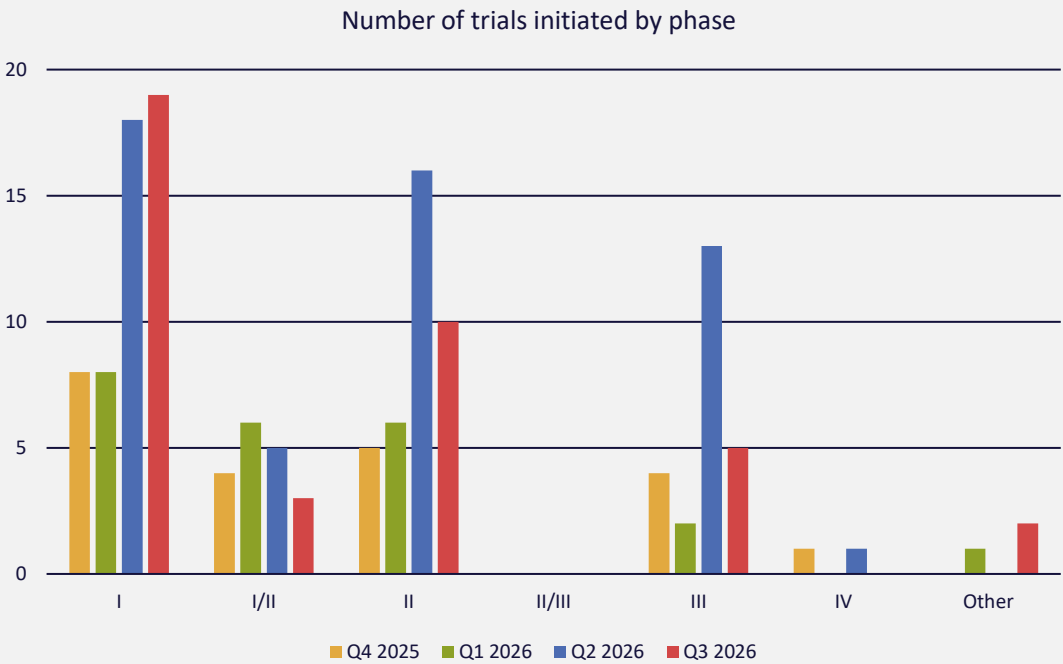
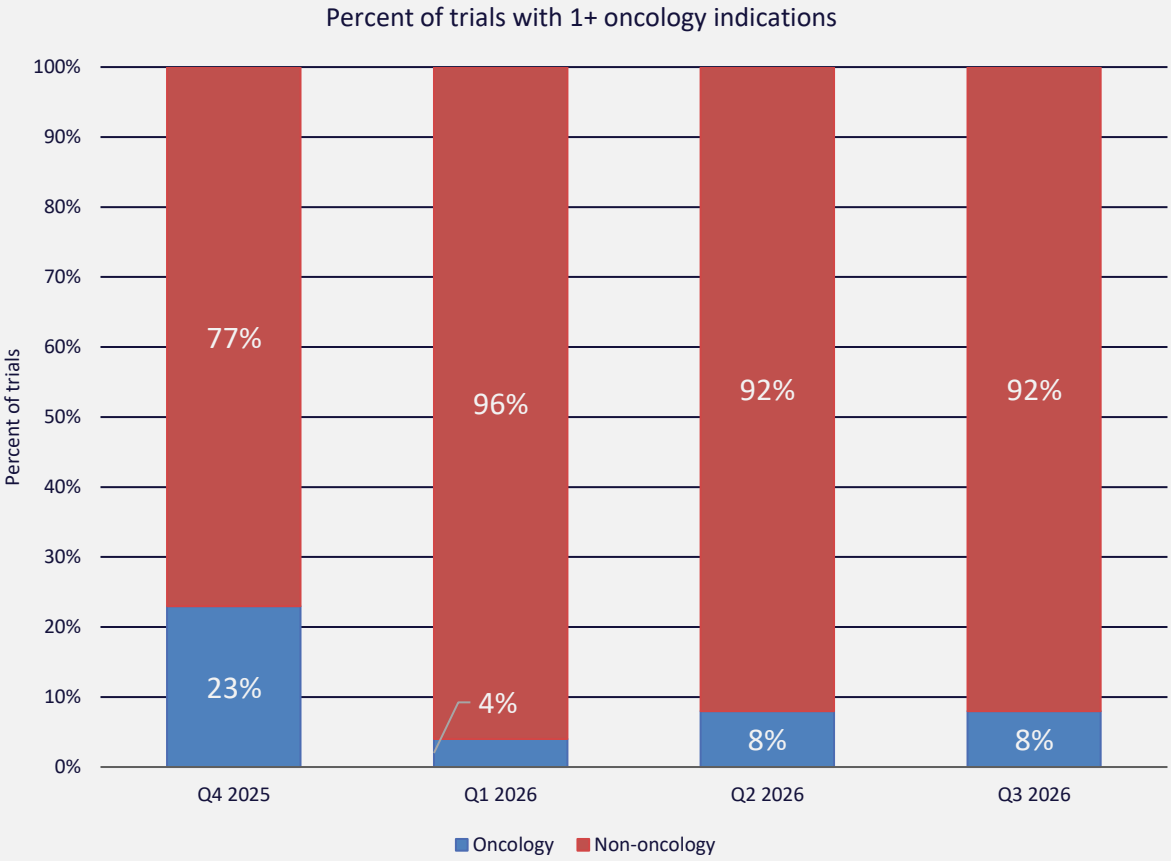
Of the RNA therapies for which indications are specified, the most targeted rare indications in Q3 2026 were:



Source: Pharmaprojects | Citeline, October 2026

RNA therapy pipeline: clinical trial activity

- 39 RNA trials were initiated in Q3 2026, 14 fewer than in Q2 2026
- Of these 39, 92% were for non-oncology indications, the same as the previous quarter
- There are currently 538 open RNA therapy clinical trials



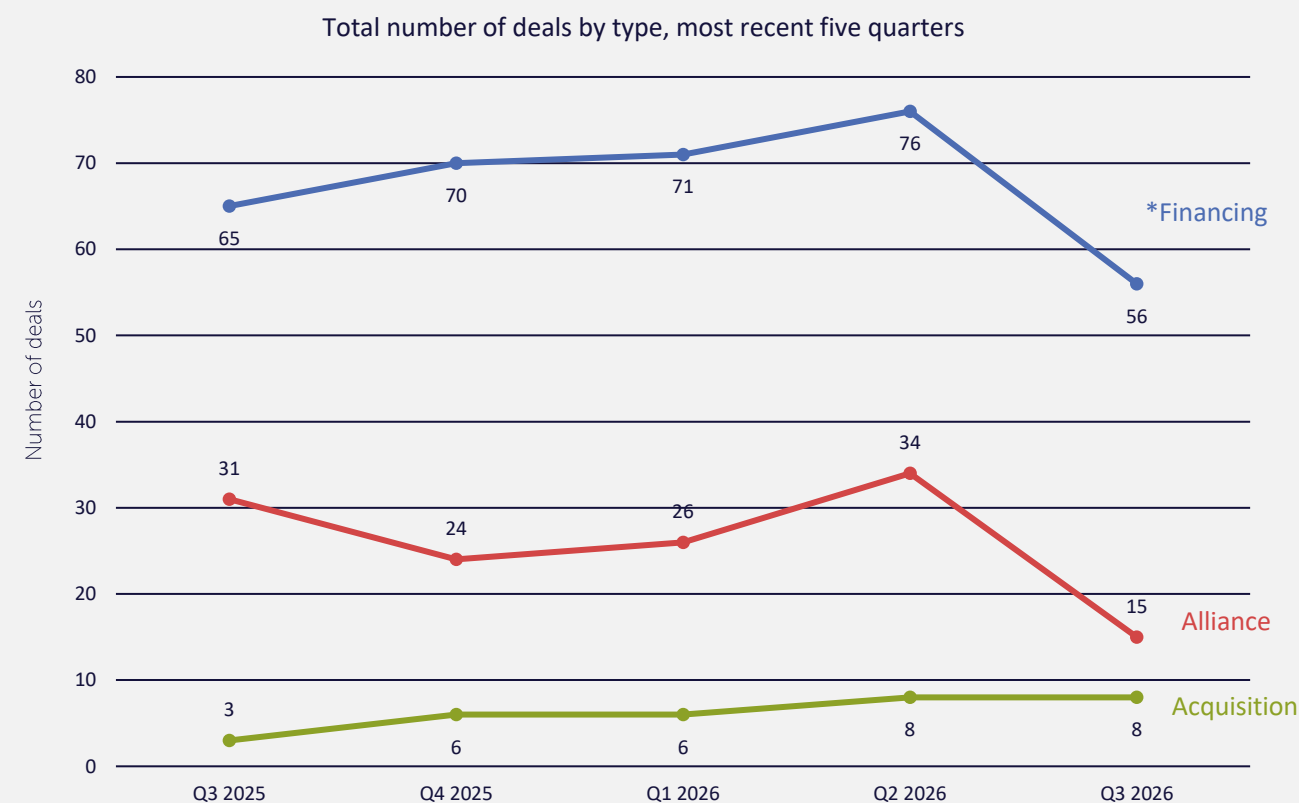
Source: Trialtrove | Citeline, October 2026

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

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

- Advanced molecular therapy dealmaking was down 33% in Q3 2026, decreasing to a total of 79 transactions from 118 in the previous quarter
- Declines were seen most impactfully in financings and alliances
- The Q3 2026 aggregate was the lowest seen in the last year, falling 20% below Q3 2025's 99 transactions

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



Sources: Biomedtracker, BioSciDB | Evaluate, October 2026

Q3 2026 acquisitions in gene, cell, and RNA therapy

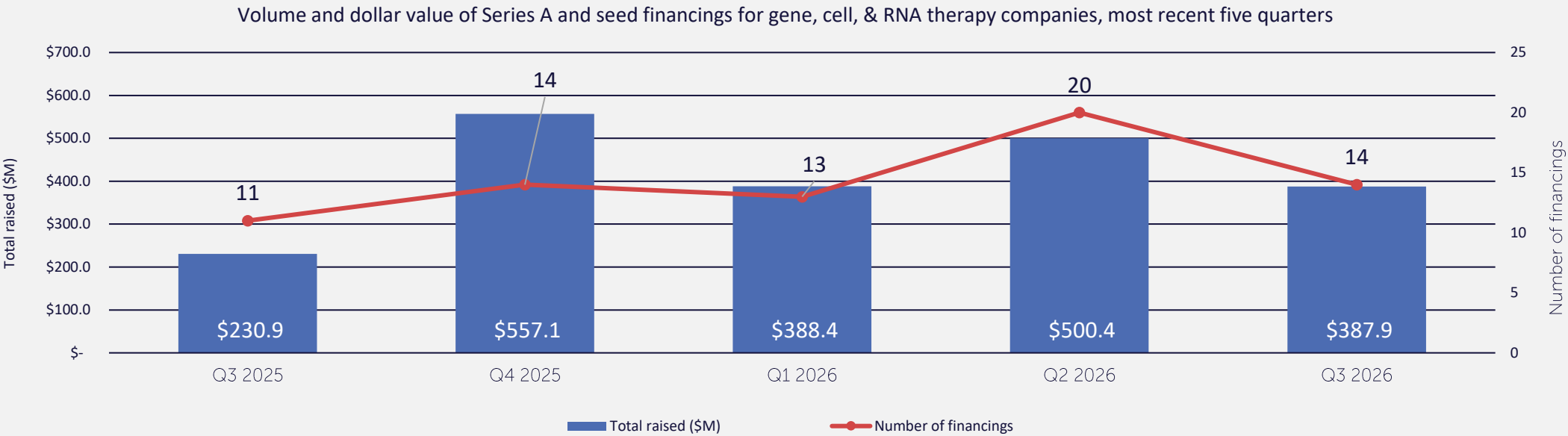
- Acquisition volume of advanced molecular therapy companies remained consistent, with eight deals done in Q3 2026, the same as in the previous quarter, with volume trending upward throughout the year
- In a major takeover in the cell bioprocessing industry, Repligen acquired BioLife Solutions and its lead biopreservation product CryoStor in a transaction worth \$1.5 billion in cash and equity
- Thymic cell engineering was the focus of the second-largest acquisition in Q3 2026, with United Therapeutics spending \$140 million in cash up front for Thymune Therapeutics, which could also receive up to \$160 million in earn-outs

Deal date	Deal title	Potential deal value (US \$)
Jul 1, 2026	Valerio Therapeutics to Acquire Ethernal Immunotherapies	34,200,000
Jul 2, 2026	United Therapeutics Acquires Thymune Therapeutics for \$300M, Including CVR	300,000,000
Jul 15, 2026	PE Firm Celadon Partners to Acquire Senti Biosciences	60,000,000
Jul 22, 2026	Repligen to Acquire BioLife Solutions for Cash And Stock	1,500,000,000
Jul 27, 2026	Oak Bio Reverse Merges with SPAC RACC	175,000,000
Aug 13, 2026	IASO Biotechnology Announces Acquisition of MediSix Therapeutics	Undisclosed
Sep 8, 2026	Sernova Mergers with Seraxis	Undisclosed
Sep 16, 2026	Lexeo Therapeutics to Acquire Mantle Therapeutics to Expand Friedreich Ataxia Platform	21,300,000

Sources: Biomedtracker, BioSciDB | Evaluate, October 2026

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

- In Q3 2026, 14 start-up advanced molecular therapy companies raised a total of \$387.9 million, a 30% and 22% decrease in volume and value, respectively, from Q2 2026
- Despite the decrease in number of Series A and seed rounds from Q2 to Q3 2026, the 14 financings in Q3 remained ahead or in line with most other quarters throughout the year other than the Q2 outlier
- Compared with the same quarter in 2025, Q3 2026's volume was ahead by 27%, with a 68% increase in the total amount raised



Source: Biomedtracker | Evaluate, October 2026

Q3 2026 start-up financing for gene, cell, and RNA therapy companies

Deal date	Deal title	Modality type	Company location	Academic source	Potential deal value (\$M)
Jul 1, 2026	Wondercel Therapeutics Raises \$20M in Series A Round	CAR-T	China / Shenzhen	Founder is Xiaohu Fan/Xi'an Jiaotong University's Institute of Gene and Cell Therapy	20
Jul 1, 2026	ARTAN Bio Closes \$1M Seed Round	Engineered suppressor tRNA system	United States / Florida / St. Petersburg	Founder is Michael Torres	1
Jul 14, 2026	Lyora Therapeutics Launches with Seed Funding	Gene therapy and RNA targeting	United States / Rhode Island / Providence	Co-founders Luk Vandenberghe and Eric Pierce, distinguished researchers from Harvard Medical School and Mass Eye and Ear	2.5
Jul 22, 2026	Tikva Allocell Secures \$8M Series A Financing	Allogeneic cell therapy	Singapore	Baylor College of Medicine	8
Jul 30, 2026	GenAns Biotech Closes Series A Financing	Optogenetics	China / Beijing	Scientific founder Minmin Luo (Chinese Institute for Brain Research)	Undisclosed
Aug 10, 2026	Remedium Bio Gets \$10M in Series A Round	Gene therapy	United States / Massachusetts / Needham	Research operations established at Lilly Gateway Labs (part of Lilly Catalyze360)	10
Sep 2, 2026	Typewriter Therapeutics Raises \$56M in Series A Financing	CAR-T	United States / Massachusetts / Cambridge	University of Tokyo; University of Rochester	56
Sep 8, 2026	Cloverleaf Bio Raises \$33 Million Seed Financing Led by 4BIO Capital	Engineered tRNA	United States / Connecticut / New Haven	Yale University	33
Sep 8, 2026	BrainChild Bio Raises \$116M in Series A Round	CAR-T	United States / Washington / Seattle	Seattle Children's	116
Sep 9, 2026	Allogenetics Gets €3.8M in Pre-Series A Financing	Gene therapy	Germany / Hanover	Hannover Medical School	4.4
Sep 15, 2026	RegCell Raises \$44M Series A Financing	Epigenetic T cell reprogramming	United States / California / Emeryville	Osaka University; Kyoto University	44
Sep 29, 2026	AptaDir Raises €40M Seed Financing	RNA therapy	Italy / Milan	Beth Israel Deaconess Medical Center of the Harvard Medical School; Italian Research National Council; Cancer Science Institute of Singapore	45
Sep 29, 2026	ai3Bio Launches with \$48M Series A Round	mRNA	United States / Massachusetts / Watertown	Spun off Corner Therapeutics, and merged with Novasenta (University of Pittsburgh spin-off)	48
Sep 29, 2026	Nanomerics Raises Undisclosed Series A Financing	Non-viral gene delivery	United Kingdom / London	University of Strathclyde; University of Glasgow; UCL School of Pharmacy	Undisclosed

Source: Biomedtracker | Evaluate, October 2026

Notable Q3 2026 start-up gene, cell, and RNA therapy companies

	Company details	Academic source	Financing type/amount raised	Lead investor(s)	Therapy areas of interest
	CAR-T therapy focused on avoiding the blood-brain barrier by repetitive dosing directly to cerebroventricular space	Seattle Children's	Series A/\$116M	Undisclosed private family fund and foundation	Pediatric and adult oncology (CNS tumors), including diffuse intrinsic pontine glioma (DIPG)
	Non-viral and completely RNA-based gene insertion, founded on R2 retrotransposon research; initial focus on in vivo CAR-T therapy	University of Tokyo; University of Rochester	Series A/\$56M	AN Venture Partners; RA Capital Management	Oncology, autoimmune, and hepatic
	Removal of pathogenic Th17 cells in vivo while preserving function of remaining immune system; STARx platform enables mRNA delivery via LNPs to target disease-causing Th17 cells; T Deplete platform recruits body’s immune cells to clear Th17 cells	Spun off from Corner Therapeutics, and merged with Novasenta (University of Pittsburgh spin-off)	Series A/\$48M	UPMC Enterprises; Ziff Capital Partners	Autoimmune

Source: Biomedtracker | Evaluate, October 2026

Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q4 2026

Therapy	Generic name	Disease	Catalyst	Catalyst date
Hibsago	bepirovirsen	Hepatitis B (HBV) Treatment	PDUFA for BLA - First Review	Oct 26, 2026
INO-3107	human papillomavirus (HPV) vaccine	Respiratory Papillomatosis (RP)	PDUFA for BLA - First Review	Oct 30, 2026
BBM-H901	dalnacogene ponparovec	Hemophilia B	CHMP Opinion	Jul 30, 2026 - Oct 31, 2026
BBM-H901	dalnacogene ponparovec	Hemophilia B	PDUFA for BLA - First Review	Jul 30, 2026 - Oct 31, 2026
deramiocel	deramiocel	Duchenne Muscular Dystrophy (DMD)	PDUFA for BLA - Second Review	Nov 20, 2026
cemdisiran	cemdisiran	Myasthenia Gravis (MG)	PDUFA for NDA - First Review	Nov 1, 2026 - Nov 30, 2026
Anito-cel	anitocabtagene autoleucel	Multiple Myeloma (MM)	PDUFA for BLA - First Review	Dec 23, 2026
detalimogene voraplasmid	detalimogene voraplasmid	Bladder Cancer	Pre-BLA Meeting with FDA	Oct 1, 2026 - Dec 31, 2026
Ebstrocel	pobistrocel	Epidermolysis Bullosa	Approval Decision (UK)	Oct 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
SGT-003		Duchenne Muscular Dystrophy (DMD)	Meeting with FDA	Oct 1, 2026 - Dec 31, 2026
Tryngolza	olezarsen	Dyslipidemia / Hypercholesterolemia	Supplemental Approval Decision	Sep 24, 2026 - Dec 31, 2026
Tryngolza	olezarsen	Dyslipidemia / Hypercholesterolemia	Supplemental CHMP Opinion	Jul 1, 2026 - Dec 31, 2026
Otarmeni	lunsotogene parvec	Otoferlin Gene-Mediated Hearing Loss	CHMP Opinion	Nov 1, 2026 - Jan 31, 2027
BBM-H901	dalnacogene ponparovec	Hemophilia B	Approval Decision (Europe)	Sep 25, 2026 - Mar 31, 2027

Source: Biomedtracker | Evaluate, October 2026

Upcoming catalysts

Below are noteworthy catalysts (forward-looking events) expected in Q4 2026 *continued*

Therapy	Generic name	Disease	Catalyst	Catalyst date
JointStem	mesenchymal stem cells	Osteoarthritis	Pre-BLA Meeting with FDA	Sep 28, 2026 - Mar 31, 2027
Zemcelpro	dorocubicel	Myelodysplastic Syndrome (MDS)	European Approval Decision	Nov 25, 2025 - Mar 31, 2027
Allo-JaCE03	cultured epidermal autograft	Burn Injury	Approval Decision (Japan)	Dec 1, 2026 - Jun 30, 2027
Hibsago	bepirovirsen	Hepatitis B (HBV) Treatment	CHMP (European Panel) Results	Dec 1, 2026 - Jun 30, 2027
Mogenry	sonpiretigene isteparvovec	Retinitis Pigmentosa (RP)	PDUFA for BLA - First Review	Sep 27, 2026 - Jun 30, 2027
SA-030		Obesity	Pre-IND meeting with FDA	Mar 2, 2026 - Sep 30, 2027
prulacabtagene leucel	prulacabtagene leucel	Lupus Nephritis	Meeting with FDA	Jun 30, 2026 - Dec 31, 2027
Neuronata-R	lenzumestrocel	Amyotrophic Lateral Sclerosis (ALS)	PDUFA for BLA - First Review	Jul 21, 2026 - Jan 21, 2028

Source: Biomedtracker | Evaluate, October 2026

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

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 is 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 <i>ex vivo</i> , 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 from 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 <i>in</i> or <i>ex vivo</i> . Used to replace defective or missing genes (as in cystic fibrosis) as well as to introduce broadly acting genetic sequences for the treatment of multifactorial diseases (e.g., cancer). Direct administration of oligonucleotides without using vectors is covered separately in the antisense therapy class; RNA interference class; or oligonucleotide, non-antisense, non-RNAi class. Platform technologies for gene delivery are covered separately in the gene delivery vector class
Lytic virus (falls under gene therapy in this report)	Therapies that have a replication-competent virus, that lyse pathogenic cells directly. These are normally genetically modified to render them harmless to normal tissues. Examples include oncolytic viruses that specifically attack cancer cells

RNA therapy includes the following therapeutic classes:	
Antisense therapy	Antisense compounds under development as potential therapeutics. These may be synthetic oligonucleotides, or antisense RNA may be expressed from a vector as a form of gene therapy. They may prevent the expression of a specific protein <i>in vivo</i> 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). <i>In vivo</i> , 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, assessing 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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