

Q4 2025 Sector Snapshot

A Look into Cell and Gene Therapy Sector Trends


ARM's Q4 2025 cell and gene therapy sector data

Review of the clinical pipeline, clinical milestones, and regulatory trends

Financial, investment, and commercial trends

Q4 2025 Cell and Gene Therapy Sector Data

Below is a glance at the latest data for the cell and gene therapy sector.

Data provided by:  **CITELINE**
a nonstella company

	North America	Europe	Asia-Pacific	Total
Clinical Trials	916	337	990	2,130
Developers	703	396	685	1,856
Investment	\$2.5B	\$0.6B	\$0.2B	\$3.2B

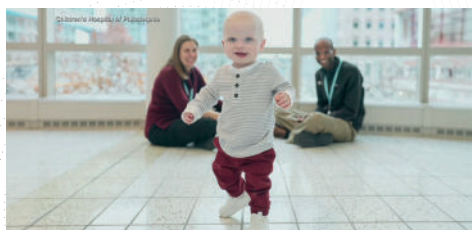
To digest our full data, including different breakdowns such as by geography, phase, or therapeutic approach, visit ARM's website at: www.alliancerm.org

Quarterly Spotlight

First Ex-Vivo Gene Therapy to be Led by a Non-Profit from Laboratory Research to Approval

Fondazione Telethon announced in December that the FDA had approved its gene therapy for Wiskott-Aldrich syndrome, a rare genetic disorder with a high unmet medical need. The milestone makes it the first non-profit organization to have successfully led the whole pathway of an ex-vivo gene therapy from laboratory research to regulatory approval.

The treatment also received approval for market use from the European Commission on January 16, 2026. We congratulate the entire team at Fondazione Telethon on this outstanding achievement.



Baby KJ's First Steps

Earlier this year, 'Baby KJ' became the first patient to receive a customized in-vivo gene editing treatment. The therapy was designed to treat CPS1, a devastating ultra-rare disease. Even more remarkable was that it was developed and administered within six months of his diagnosis. Without the gene therapy, Baby KJ's illness could have been fatal. Instead, 'KJ' took his first steps right before the holidays, marking a significant milestone in his post-treatment development.

Notable Clinical Developments

New Data Signals Growing Promise of CGT for Cardiovascular Diseases

New clinical developments show that cell and gene therapies (CGTs) for cardiovascular diseases have strong potential in the early and pivotal stages of the pipeline.



Capricor Therapeutics announced positive topline results from its pivotal Phase 3 study of its cell therapy for treating Duchenne cardiomyopathy, providing strong evidence of slowing the disease's progression on both skeletal and cardiac functions.



Tenaya Therapeutics announced positive interim Phase 1b/2 data from its gene therapy for PKP2-ACM, showing that the treatment provided clinically meaningful reductions in arrhythmia burden after >6 months of follow-up.



CRISPR Therapeutics released positive Phase 1 data for its first-in-human gene editing treatment targeting mutations in the ANGPTL3 gene. ANGPTL3 produces proteins crucial for reducing triglyceride and LDL cholesterol levels. The study demonstrated that the therapy has the potential to help correct mutations that inhibit the gene's function in producing these proteins, resulting in significantly lower LDL cholesterol and triglyceride levels in patients.

The Cell Therapy Pipeline for Autoimmune Diseases Grows Stronger

CAR-T cell therapies have shown great promise as a new class of medicines for durably treating autoimmune diseases. Since October, more data has emerged to support this promise.

- ▶ Kyverna Therapeutics announced positive topline data from a Phase 2 CAR-T trial showing a significant clinical benefit in treating Stiff Person Syndrome. A regulatory submission to the FDA is expected in the first half of 2026.
- ▶ Cabaletta Bio presented positive Phase 1/2 data for its CAR-T cell therapy, demonstrating transformative clinical responses in patients with myositis, as well as promising preliminary data for patients with systemic sclerosis (SSc) and systemic lupus erythematosus (SLE).
- ▶ Fate Therapeutics released Phase 1 data showing that patients with SLE achieved durable responses with its off-the-shelf CAR-T cell therapy, which uses less intensive or no conditioning regimens, depending on the patient's circumstances.
- ▶ Bristol Myers Squibb presented encouraging Phase 1 data showing that 94% of patients with SSc, SLE, or idiopathic inflammatory myopathies remained off immunosuppressive therapy at the time of publication after treatment with their CAR-T cell therapy.
- ▶ Cartesian Therapeutics announced data showing a strong efficacy signal in the Phase 2 trial of its CAR-T cell therapy for SLE. The study showed that 100% of patients achieved a Lupus Low Disease Activity State during a 3-month follow-up.

Reaching Larger Patient Populations

The fourth quarter of 2025 delivered two promising developments for patient access.



Gene therapy for spinal muscular atrophy (SMA) is now approved for patients two years and older

Novartis announced that the FDA has approved Itivisma, a new form of Zolgensma with a different route of administration, as the first gene therapy to treat patients two years and older with SMA who have an SMN1 gene mutation. Zolgensma is approved only for children with SMA under age 2, and the new approval helps expand this transformative treatment to more patients with few other treatment options.



CRISPR gene editing for treating sickle cell disease and transfusion-dependent beta thalassemia (TDT) holds transformative potential for younger patients

Vertex Pharmaceuticals presented new pivotal-stage clinical studies in December that showed Casgevy, its CRISPR-based ex-vivo gene editing treatment for severe sickle cell disease and TDT, has transformative potential as a therapy for children aged 5-11 years. Casgevy was initially approved for patients 12 and older. The data paves the way for a regulatory decision that could expand the treatable patient population.

Policy and Regulatory Trends

Passage of the Mikaela Naylon Give Kids a Chance Act and the Accelerating Kids Access to Care Act

ARM applauds the bipartisan passage of the Mikaela Naylon Give Kids a Chance Act and the Accelerating Kids' Access to Care Act as part of the FY26 Appropriations Package. These policies, which became law with President Trump's signature, represent hope for the rare disease community and were key priorities in ARM's 2023-2025 3-Year Strategic Plan.

The Mikaela Naylon Give Kids a Chance Act reauthorizes the Rare Pediatric Disease Priority Review Voucher (RPD PRV) Program, which Congress let expire last year. The RPD PRV encourages the development of treatments for the estimated 15 million children in the U.S. living with a rare disease. The Accelerating Kids' Access to Care Act will help address unnecessary treatment delays when pediatric patients need to travel across state lines to receive care.



Release of the EU Biotech Act

The European Commission released the EU Biotech Act on December 16, marking a potential turning point for Europe's advanced therapies agenda. The act takes necessary steps toward advancing Europe's CGT landscape and properly recognizes that the success of the sector depends on how regulation, investment, and delivery work together. It offers a welcome mix of incentives and regulatory improvements for CGT, including streamlining GMO rules for clinical trials, providing investment funds, introducing a 12-month extension to supplementary patent rights, and setting up centers of excellence. As negotiations progress, ARM stresses that the Biotech Act must remain ambitious, delivering tangible benefits for patients while enhancing Europe's leadership in CGT.

FDA Unveils 'Plausible Mechanism Pathway' to Accelerate Personalized Gene Editing Treatments

FDA Commissioner Marty Makary and CBER Director Vinay Prasad outlined the agency's "plausible mechanism pathway" in Q4 2025 to help patients more quickly access personalized gene editing therapies and other therapies targeting diseases with high unmet need. ARM has encouraged the FDA to drive the execution of its CGT vision through formal guidance that enables its implementation with consistency, clarity, and appropriate oversight, so that other patients can access transformative therapies.

Soon after the release of the plan, the Children's Hospital of Philadelphia announced intentions for an 'umbrella trial' structure to utilize the gene editing therapy that saved Baby KJ's life to develop bespoke therapies for treating variants of different rare diseases.



The Near-Term Cell and Gene Therapy Clinical Pipeline

The following two pages contain a non-exhaustive list of cell and gene therapies that have been or may be submitted for a regulatory decision in the United States and the European Union.



2026 United States Clinical Pipeline

Therapy Name (Developer)	Therapy Type (Indication)	Regulatory Status
RGX-121 (REGENXBIO)	Gene Therapy (Hunter syndrome)	Decision date: February 8, 2026
Kresladi (Rocket Pharmaceuticals)	Gene Therapy (Severe leukocyte adhesion deficiency type 1)	Decision date: March 28, 2026
Orca-T (Orca Bio)	Cell Therapy (Graft versus host disease)	Decision date: April 6, 2026
DTX401 (Ultragenyx)	Gene Therapy (Glycogen storage disease type 1a)	FDA BLA submitted
Isaralgagene Civaparvovec (Sangamo)	Gene Therapy (Fabry disease)	
UX111 (Ultragenyx)	Gene Therapy (MPS IIIA)	
Deramiocel (Capricor Therapeutics)	Cell Therapy (Duchenne muscular dystrophy cardiomyopathy)	CRL response (Under existing BLA)
NXC-201 (Immix Biopharma)	CAR-T Cell Therapy (Relapsed/refractory AL amyloidosis)	FDA BLA submission possible in H1 2026
Revascor (Mesoblast)	Cell Therapy (End-stage ischemic heart failure)	
Anito-cel (Arcellx and Kite Pharma)	CAR-T Cell Therapy (Multiple myeloma)	
AMT-130 (UniQure)	Gene Therapy (Huntington's disease)	
Miv-cel (Kyverna Therapeutics)	CAR-T Cell Therapy (Stiff person syndrome)	
MCO-010 (Nanoscope Therapeutics)	Gene Therapy (Retinitis pigmentosa)	
RGX-202 (REGENXBIO)	Gene Therapy (Duchenne muscular dystrophy)	
CardiAMP (Biocardia)	Cell Therapy (Microvascular dysfunction)	
Detalimogene Voraplasmid (EnGene)	Gene Therapy (Bacillus Calmette-Guérin (BCTG)-unresponsive disease)	FDA BLA submission possible in H2 2026
CAN-2409 (Candel Therapeutics)	Gene Therapy (Localized prostate cancer)	
NTLA-2002 (Intellia Therapeutics)	Gene Therapy (Hereditary angioedema)	



2026 European Union Clinical Pipeline

Therapy Name (Developer)	Therapy Type (Indication)	Regulatory Status
Etuvetidigene autotemcel (Fondazione Telethon)	Gene Therapy (Wiskott-Aldrich syndrome)	Approved by the European Commission on Jan. 16, 2026
Nadofaragene firadenovec (Ferring Pharmaceuticals)	Gene Therapy (Treatment of adult patients with high-grade, Bacillus Calmette-Guérin-unresponsive non-muscle invasive bladder cancer)	EMA MAA submitted
Papzimeos (Precigen)	Gene Therapy (Recurrent respiratory papillomatosis)	EMA MAA submitted
Lifileucel (Iovance)	Cell Therapy (Metastatic melanoma)	EMA MAA submission possible in 2026
OCU400 (Ocugen)	Gene Therapy (Retinitis pigmentosa)	EMA MAA submission possible in 2026

Current Pipeline Trends



While the U.S. saw six new drug approvals in 2025, a decrease from 2024, it still maintains a robust pipeline with three regulatory decisions scheduled in H1 2026 and 10+ regulatory submissions possible throughout the year



The pipeline for gene therapies to treat rare genetic diseases remains strong within the U.S., with three decisions likely in 2026 and potentially 10+ more regulatory submissions possible



The first-ever regulatory submission of a CAR-T cell therapy to treat an autoimmune disease (Stiff person syndrome) is possible in 1H 2026



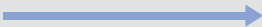
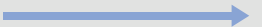
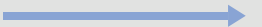
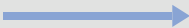
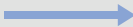

Europe saw three regulatory approvals in 2025, with at least three more possible in 2026



Commercialization and Market Trends

Following the emergence of two blockbuster products from 2021 through 2024, **the CGT sector has achieved two more in 2025**, Carvykti and Breyanzi, bringing its total to four. This year's newcomers are driven by explosive growth and are already poised to become multibillion-dollar products in the next few years.

Notable Revenue Trends for Approved CGTs

Therapy	2025 Revenue	YoY Increase	Blockbuster Status
Carvykti	 \$1.9 billion	96%	✓ Achieved in 2025
Breyanzi	 \$1.3 billion	82%	✓ Achieved in 2025
Yescarta	 \$1.1 billion*	-5%	✓ Achieved in 2022
Zolgensma	 \$1.2 billion	1%	✓ Achieved in 2021
Elevidys	 \$899 million	9%	Possible by 2031
Vyjuvek	 \$388 million**	33.6%	Possible by 2030
Amtagvi	 \$155 million*	182%	Possible by 2031

*Figures are based on Q1-Q3 2025 financial figures. Q4 2025 figures were not available at the time of publication. **Figures are 2025 revenue projections to be finalized.

Notable Financing Deals

Mergers & Acquisitions

Bristol Myers Squibb and Orbital Therapeutics

BMS acquired Orbital for \$1.5 billion

Partnerships

Regeneron and Tessera Therapeutics

Regeneron to provide \$150 million upfront

Focus: In vivo gene writing program for AATD

Chiesi and Arbor Biotechnologies

Chiesi to provide \$115 million in near-term payments

Focus: Gene editing to treat primary hyperoxaluria type 1

Kite Pharma and Pregene

Kite to provide \$120 million upfront

Focus: In vivo CAR-T therapies

Public Offerings

Lexeo Therapeutics

\$154 million

Focus: Gene therapy for liver and heart diseases

Capricor Therapeutics

\$150 million

Focus: Cell therapy for DMD cardiomyopathy

EnGene

\$130 million

Focus: Gene therapy for BCG unresponsive disease

Immatics

\$125 million

Focus: Cell therapy for oncology

Kyverna Therapeutics

\$100 million

Focus: CAR-T cell therapy for autoimmune diseases

4DMT

\$100 million

Focus: Gene therapy for ocular diseases

Immix Biopharma

\$100 million

Focus: CAR-T cell therapy for r/r AL Amyloidosis

Precision Biosciences

\$75 million

Focus: Gene editing treatments for rare diseases

Private Capital Raises

AAVantage

\$141 million (Series B)

Focus: Gene therapy for rare ocular diseases

Lead investors:

Schroders Capital, Atlas Venture, and Forbion

Aspen Neuroscience

\$115 million (Series C)

Focus: Gene therapy for neurological diseases

Lead investors:

OrbiMed, ARCH Venture Partners, Frazier Life Sciences, and Revelation Partners

Profluent

\$106 million (Financing)

Focus: Gene editing platforms

Lead investors:

Altimeter Capital and Bezos Expeditions

Azalea Therapeutics

\$82 million (Series A)

Focus: Engineered cell therapies for oncology

Lead investors:

Third Rock Ventures

Stay Engaged with ARM's Network

Cell and Gene Meeting on the Med

Rome, Italy

April 28-30, 2026
meetingonthemed.com



Cell and Gene Meeting on the Mesa

Phoenix, AZ

October 5-7, 2026
meetingonthemesa.com



Get Involved

From Advisory Groups to Forums, there are many ways to get involved with ARM. This is your chance to influence the direction of the sector. Get in touch with our team today.

Contact: member@alliancerm.org

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