Alliance for Regenerative Medicine

Q1 2026 Sector Snapshot

APRIL 2026

A Look into Cell and Gene Therapy Sector Trends

Review of the clinical pipeline and clinical milestones

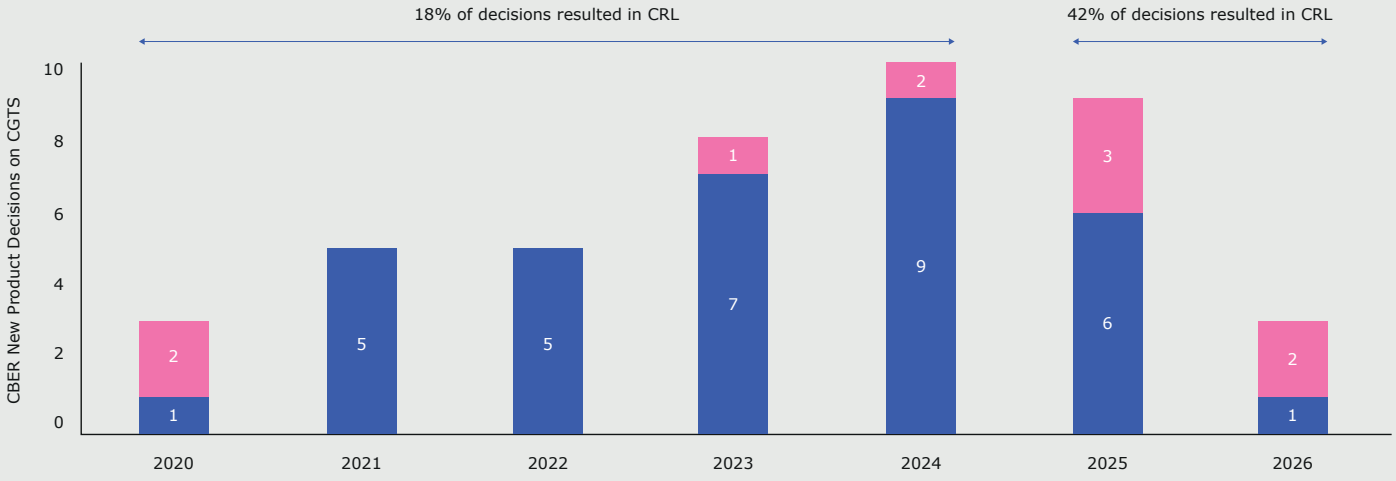
Recent policy developments and regulatory trends

Financial, investment, and commercial trends

Quarterly Spotlight

In Focus: The U.S. FDA

During the first quarter, the FDA issued a string of surprising complete response letters (CRLs) and delays in Biologics License Applications (BLAs) for cell and gene therapy (CGT) products. The regulatory decisions appeared to reverse previous alignments with companies and introduced new evidentiary requirements, confusing patients, companies, and investors.



CRL Source: ARM Data

In response, ARM has been engaging with the FDA, policymakers, and other stakeholders to advocate for four key course corrections that the FDA should take: 1. The FDA should honor its past alignment with companies; 2. If there is a reasonable scientific disagreement, the FDA should call an Advisory Committee meeting to bring in outside experts, hear from the patient community, and let the company present its data; 3. CBER should find problems early in the review cycle and try to resolve them favorably to enable medicines to be approved; 4. The new CBER director needs to rebuild the senior ranks of both CBER and the Office of Therapeutic Products.

First Gene Therapy Approval of 2026

On March 26, the cell and gene therapy community welcomed the FDA's accelerated approval of KRESLADI, Rocket Pharmaceuticals' gene therapy for severe leukocyte adhesion deficiency-I, the first-ever gene therapy approved for this indication.The decision also marked the first U.S. gene therapy approved by the FDA this year.

Notable Clinical Developments

With a maturing CGT clinical pipeline, 2026 will be a year rich in data readouts and potential catalysts. Below is a non-exhaustive list of notable readouts in the first quarter.

January 12, 2026: Lexeo Therapeutics announced phase 1/2 data for its AAV gene therapy candidate, LX2020, for the treatment of PKP2-associated arrhythmogenic cardiomyopathy.

- The majority of patients showed increased protein expression and meaningful improvements in stabilization, as measured by arrhythmia burden.

March 11, 2026: REGENXBIO announced phase 1/2 interim data for its AAV gene therapy, RGX-202, to treat Duchenne muscular dystrophy.

- Participants outperformed comparable untreated patients on physical function tests after one year.

March 11, 2026: Solid Biosciences released an interim clinical update on its phase 1/2 gene therapy program, SGT-003, to treat Duchenne muscular dystrophy.

- Patients demonstrated robust microdystrophin expression, with evidence of restoration of the dystrophin-associated protein complex and improvements in muscle integrity.

March 12, 2026: Ultragenyx released phase 3 data for its AAV gene therapy, DTX301, for the treatment of Ornithine transcarboxylase deficiency.

- The double-blind, placebo-controlled study showed that patients achieved a meaningful reduction in 24-hour plasma ammonia compared with a placebo.

March 18, 2026: Aspen Neuroscience published 12-month positive data from its ASPIRO clinical trial program, in which patients received its autologous stem cell therapy, Sasineprocel, to treat Alzheimer's and Parkinson's disease.

- Two patient cohorts showed numerical improvements in function, outcomes, and quality of life.

March 25, 2026: Beam Therapeutics announced phase 1/2 data from its base-editing program to treat alpha-1 antitrypsin (AAT) deficiency.

- Treated patients saw rapid and durable increases in AAT, decreases in mutant AAT that cause liver toxicity, and the production of corrected, therapeutic levels of protein.

March 24, 2026: Ocugen announced positive 12-month data from its Phase 2 clinical trial evaluating OCU410, its novel modifier gene therapy for geographic atrophy secondary to dry AMD.

- The study showed patients had meaningful reduction in geographic atrophy growth.

2026 Clinical Watchlist

Below is a non-exhaustive list of notable programs expected to deliver readouts in 2026.

1H 2026	2H 2026	
Taysha Gene Therapies (Rett syndrome)	**Neurogene** ** (Rett syndrome)	**REGENXBIO** (Wet AMD)
Cabaletta Bio (Myasthenia gravis, systemic sclerosis, and systemic lupus erythematosus)		
REGENXBIO (Duchenne muscular dystrophy)	**Beacon Therapeutics** (X-linked retinitis pigmentosa)	**Intellia Therapeutics** (Hereditary Angioedema)
Kyverna Therapeutics* (Stiff person syndrome)		

*Data released in Q2 2026 and will be highlighted in ARM's Q2 2026 Sector Snapshot.
**Company expects a mid-year readout.

Policy and Regulatory Updates

The Mikaela Naylon Give Kids a Chance Act and the Accelerating Kids Access to Care Act Became Law | The FDA's PRV Program is Reauthorized

A big win for patients and the cell and gene therapy community! The Mikaela Naylon Give Kids a Chance Act and the Accelerating Kids Access to Care Act have been passed into law. These policies represent hope for the rare disease community and will meaningfully improve access to life-changing and life-saving treatments.

The **Mikaela Naylon Give Kids a Chance Act reauthorizes** the Rare Pediatric Disease Priority Review Voucher (RPD PRV) Program, which Congress let expire in 2025. The RPD PRV encourages the development of treatments for the estimated 15 million children in the U.S. living with a rare disease, and a voucher can be used to secure a speedier FDA review of a future therapy or be sold to another company. 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. The legislation streamlines processes for specialty providers caring for children with complex medical needs, including children with rare diseases who must often travel to other states to receive cell and gene therapy treatments.

We applaud Congress for passing ARM's top two legislative priorities and are grateful to our partner organizations whose collaboration and advocacy were crucial in securing the necessary support.

FDA Plausible Pathway Mechanism Shows Promise, but also Presents CMC Challenges

At the tail end of the first quarter, the Children's Hospital of Philadelphia and Penn Medicine released a proof-of-concept demonstrating a customizable in vivo prime-editing platform. Their research showed that a two-part prime editing system, a lipid nanoparticle that delivers mRNA encoding the editor, plus a customized adeno-associated virus that supplies the short guide RNAs, could potentially function as a therapeutic by correcting mutations in the liver cells of infants with Urea Cycle Disorders. This approach can address the patient's specific genetic variant.

The study shows that the FDA's plausible mechanism framework can help streamline non-clinical and clinical requirements for personalized gene therapies. However, the study sponsors noted that CMC requirements in the framework remained challenging to meet, suggesting further potential to streamline the framework to benefit patients with ultra-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**
Kresladi (Rocket Pharmaceuticals)	**Gene Therapy** (Severe leukocyte adhesion deficiency type 1)	Approved
DB-OTO (Regeneron)	**Gene Therapy** (Genetic hearing loss)	Approved
Orca-T (Orca Bio)	**Cell Therapy** (Graft versus host disease)	Decision date: July 6, 2026
Deramiocel (Capricor Therapeutics)	**Cell Therapy** (Duchenne muscular dystrophy cardiomyopathy)	Decision date: August 22, 2026
DTX401 (Ultragenyx)	**Gene Therapy** (Glycogen storage disease type 1a)	Decision date: August 23, 2026
UX111 (Ultragenyx)	**Gene Therapy** (Mucopolysaccharidosis Type IIA)	Decision date: September 19, 2026
Anito-cel (Arcellx and Kite Pharma)	**CAR-T Cell Therapy** (Multiple myeloma)	Decision date: December 23, 2026
Isaralgagene Civaparvovec (Sangamo)	**Gene Therapy** (Fabry disease)	Rolling BLA in progress
Miv-cel (Kyverna Therapeutics)	**CAR-T Cell Therapy** (Stiff person syndrome)	FDA BLA submission possible in H1 2026
Revascor (Mesoblast)	**Cell Therapy** (End-stage ischemic heart failure)	
MCO-010 (Nanoscope Therapeutics)	**Gene Therapy** (Retinitis pigmentosa)	
Detalimogene Voraplasmid (EnGene Therapeutics)	**Gene Therapy** (Bacillus Calmette-Guérin (BCG)-unresponsive disease)	FDA BLA submission possible in H2 2026
NXC-201 (Immix Biopharma)	**CAR-T Cell Therapy** (Relapsed/refractory AL amyloidosis)	
CAN-2409 (Candel Therapeutics)	**Gene Therapy** (Localized prostate cancer)	
RGX-202 (REGENXBIO)	**Gene Therapy** (Duchenne muscular dystrophy)	
CardiAMP (Biocardia)	**Cell Therapy** (Microvascular dysfunction)	
PM359 (Prime Medicine)	**Gene Therapy** (Chronic Granulomatous Disease)	
NTLA-2002 (Intellia Therapeutics)	**Gene Therapy** (Hereditary Angiodema)	

2026 European Union Clinical Pipeline

Therapy Name (Developer)	**Therapy Type** (Indication)	**Regulatory Status**
etuvetidigene autotemcel (Fondazione Telethon)	**Gene Therapy** (Wiskott-Aldrich syndrome)	Approved
Nadofaragene firadenovec (Ferring Pharmaceuticals)	**Gene Therapy** (Treatment of adult patients with high-grade, Bacillus Calmette-Guérin-unresponsive non-muscle invasive bladder cancer)	Received positive opinion from CHMP, full EU Commission approval pending
Itvisma (Novartis)	**Gene Therapy** (Spinal muscular atrophy for children 2+, teens, and adults)	Received positive opinion from CHMP, full EU Commission approval pending
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

Commercialization and Market Trends

In 2025, the cell and gene therapy sector achieved three new blockbuster products, Breyanzi, Carvykti, and Elevidys, that achieved more than $1 billion in annual sales. This brings the total number of blockbuster products to five, with the recent newcomers accompanying the established blockbusters Zolgensma and Yescarta.

The Current Roster of CGT Blockbusters

Therapy	2025 Revenue	YoY Increase	Blockbuster Status
Carvykti	$1.9 billion	97%	Achieved in 2025
Yescarta	$1.5 billion	-5%	Achieved in 2022
Breyanzi	$1.3 billion	82%	Achieved in 2025
Zolgensma	$1.2 billion	1%	Achieved in 2021
Elevidys	$898.7 million (US) | CHF 341 million (Global)*	26%	Achieved in 2025

Products with Blockbuster Potential

Therapy	2025 Revenue	YoY Increase	Blockbuster Status
Vyjuvek	$389 million	34%	Possible by 2031
Amtagvi	$220 million	112%	Possible by 2031

*Figures represent U.S. commercial sales (Sarepta Therapeutics) and global commercial sales. (Roche)

Notable Financing Deals for Q1 2026

Mergers & Acquisitions

Gilead and Arcellx

Gilead to acquire Arcellx and its CAR-T program in a deal worth up to $7.8 billion

Eli Lilly and Orna Therapeutics

Eli Lilly to acquire Orna Therapeutics and its in vivo CAR-T program in a deal worth $2.4 billion

Capital Raises

Cellares

Series D financing worth $257 million to support cell therapy manufacturing and automation programs

Lead investors: BlackRock and Eclipse

Orca Bio

Series F financing worth $250 million to support the potential commercialization of its cell therapy programs

Lead investors: Lightspeed Venture Partners

Solid Biosciences

Oversubscribed private placement worth $240 million to support potential clinical gene editing programs

Lead investors: Leerink Partners and Citigroup

MEDIPOST

Closed $140 million in funding to support its cord blood-derived stem cell therapy programs

Lead investors: Skylake Equity Partners and Crescendo Equity Partners

Century Therapeutics' $135M PIPE

Oversubscribed private placement worth $135 million to support lead cell therapy program

Lead investors: TCGX

Candel Therapeutics' $100M public offering

Closed $100 million public offering to support viral cell immunotherapies

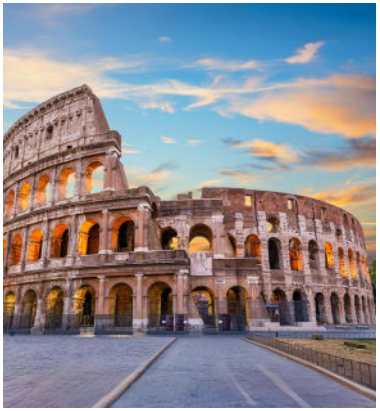
Enjoy our Sector Snapshots? Here are a few ways to stay engaged with our network.

Upcoming Events

Cell and Gene Meeting on the Med

Rome, Italy

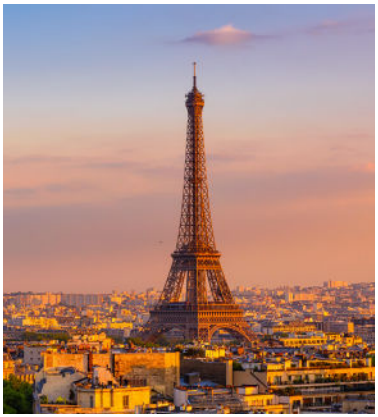
April 28-30, 2026
meetingonthemed.com



Workshop | Defining Market Readiness for Genetic Medicines

Paris, France

June 19, 2026
https://alliancerm.org/events



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

Stay Engaged

Become a Member
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