Alliance for Regenerative Medicine

Q1 2025 SECTOR SNAPSHOT

APRIL 2025

A Look into Cell and Gene Therapy Sector Trends

2025 clinical pipeline and milestones

Policy and regulatory trends

Financial, investment, and commercial trends

Notable Clinical Trends and Milestones

IN VIVO'S STEADY PROGRESS

YolTech Therapeutics initiated a clinical trial of an in vivo gene editing therapy using its lipid nanoparticles to treat transfusion-dependent beta-thalassemia.

- This is the first in vivo gene editing therapy to enter a clinical trial for beta-thalassemia

In Europe, the Paul Ehrlich Institute, Germany's regulatory agency, authorized Interius BioTherapeutics' in-vivo CAR gene therapy to treat B-cell malignancies for clinical trials.

- This marks the first in vivo CAR gene therapy trial in Europe

In January, Intellia Therapeutics announced that it enrolled the first patient in its Phase 3 study of a CRISPR-based in vivo gene editing therapy to treat hereditary angioedema.

- The trial is the second-ever in vivo CRISPR gene editing therapy to enter a Phase 3 study

In March, Beam Therapeutics shared positive initial data from its Phase I/II clinical trial of its in vivo base editing therapy for Alpha-1 antitrypsin deficiency.

- This clinical breakthrough demonstrated the potential of base editing in correcting a disease-causing mutation

TRENDING INNOVATIONS

Dual Administration Gene Therapy

The FDA cleared a trial of Solid Bioscience's first-in-industry dual route of administration gene therapy to treat Friedreich's ataxia. (FA)

- This novel approach administers the gene therapy in the heart to treat the cardiac effects caused by FA and in the brain to reduce neurological decline.

Compact CRISPR System

Can the sector engineer CRISPR Cas enzymes to be smaller for more efficient delivery with vectors? A new study from Mammoth Biosciences outlines a proof-of-concept for a novel Cas enzyme that is approximately one-third smaller than Cas9.

- The study showed the system's potential to match the efficiency of other in-vivo gene editing techniques due to its smaller size, allowing room for other editing applications in its vector.

Xenotransplant Organs

In February, United Therapeutics announced that the FDA authorized it to conduct a clinical trial for its xenokidney, a transplantable kidney from a pig that uses 10 gene edits to support immunological acceptance.

- This is the first-ever human clinical trial of kidney xenotransplantation, a big step in potentially transforming the availability of organ transplants.

NOTABLE CLINICAL DEVELOPMENTS

New Horizons for Diabetes Treatment:

Sana Biotechnology announced positive clinical results for its islet cell therapy without transplantation for Type 1 diabetes. The study highlighted a strong safety profile and showed the potential for overcoming allogeneic and autoimmune rejection with pancreatic islet cell transplantation in Type 1 diabetes with no immunosuppression.

Patients with Type 1 diabetes are dependent on insulin injections for treatment. Several cell therapies to reduce insulin dependence are in Phase I and Phase II clinical trials, using approaches like allogeneic islet cells, autologous regulatory T-cells, and autologous dendritic cells.

Encouraging Data for Therapies to Treat Parkinson's Disease:

January was a notable month of progress for cell and gene therapies to treat Parkinson's disease. Bayer AG and BlueRock Therapeutics announced the FDA advanced their cell therapy for Parkinson's disease from Phase I to Phase III clinical trials, showing encouraging trends in improvements with motor impairment.

Aspen Neuroscience reported Phase I/II clinical data for its Parkinson's disease cell therapy, with some seeing improvements in motor symptoms and quality of life. AskBio announced that the first patients were randomized in its Phase II clinical trial for its gene therapy to treat moderate-stage Parkinson's disease, riding momentum from its positive Phase 1b data readout.

There is currently no cure for Parkinson's, and current treatments do not holistically manage the disease's symptoms. These groundbreaking studies range from replacing neuron cells to gene therapy to help cells express proteins to improve brain function.

Continued Momentum in Treating Duchenne Muscular Dystrophy:

Solid Biosciences released groundbreaking data from the Phase I/II trial of its gene therapy, which showed improvements in muscle health and early signals of potential cardiac benefit.

DMD is an aggressive, rare disease with few effective treatment options that is ultimately fatal. One AAV gene therapy has been approved in the US, and several more are in development, providing hope for patients and caregivers.

Policy and Regulatory Trends

ACCELERATION OF ACCELERATED APPROVAL:

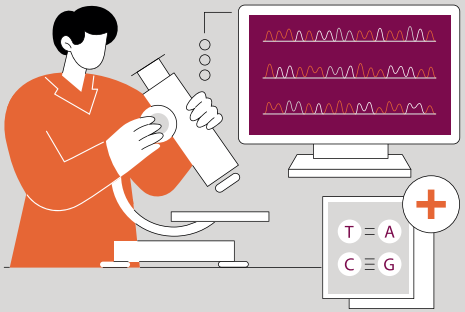
The FDA's Accelerated Approval Program is a pathway created in 1992 for the earlier approval of drugs to treat serious conditions and fill an unmet medical need. Through 2024, only three gene therapies for rare diseases have ever been approved through this designation.

In 2023 and 2024, at least six companies aligned with the FDA on the use of accelerated approval. Those regulatory decisions could occur in 2025 or 2026. Several other companies have also announced their intention to pursue accelerated approval.

1992	2024	3 gene therapies approved through accelerated approval
2025	2026	6 cell and gene therapies could be approved via accelerated approval

Companies Seeking Accelerated Approval for Gene Therapies in 2025-2026

REGENXBIO	Hunter syndrome
ULTRAGENYX	MPS IIIA
UNIQURE	Huntington disease
ROCKET PHARMACEUTICALS	Danon disease
SANGAMO	Fabry disease
PRECIGEN	Recurrent respiratory papillomatosis



THE 2025 CELL AND GENE THERAPY CLINICAL PIPELINE

2025 UNITED STATES CLINICAL PIPELINE

Therapy Name (Developer)	Therapy Type (Indication)	Regulatory Status
NT-501 (Neurotech Pharmaceuticals)	**Cell Therapy** (Macular Telangie)	Approved
Pz-cel (Abeona Therapeutics)	**Gene Therapy** (Recessive dystrophic epidermolysis bullosa)	Decision date: April 29, 2025
UX111 (Ultragenyx)	**Gene Therapy** (Mucopolysaccharidosis Type IIIA (MPS IIIA)	Decision date: August 18, 2025
PRGN-2012 (Precigen)	**Gene Therapy** (Recurrent respiratory papillomatosis)	Decision date: August 27, 2025
Deramiocel (Capricor Therapeutics)	**Cell Therapy** (Duchenne muscular dystrophy cardiomyopathy)	Decision date: August 31, 2025
Avance Nerve Graft (Axogen)	**Tissue Engineered Therapy** (Peripheral nerve discontinuities)	Decision date: September 5, 2025
RGX-121 (REGENXBIO)	**Gene Therapy** (Hunter syndrome)	FDA BLA submitted
etuvetidigene autotemcel (Fondazione Telethon)	**Gene Therapy** (Wiskott-Aldrich syndrome)	
rexlemestrocel-L (Mesoblast)	**Cell Therapy** (End-stage ischemic heart failure)	FDA BLA submission possible
MCO-010 (Nanoscope Therapeutics)	**Gene Therapy** (Retinitis pigmentosa)	
Orca-T (Orca Bio)	**Cell Therapy** (Graft versus host disease)	
Rilparencel (Prokidney)	**Cell Therapy** (Chronic kidney disease)	
RPL102 (Rocket Pharmaceuticals)	**Gene Therapy** (Fanconi anemia)	
Kresladi (Rocket Pharmaceuticals)	**Gene Therapy** (Severe leukocyte adhesion deficiency type 1)	
Isaralgagene civaparvovec (Sangamo Therapeutics)	**Gene Therapy** (Fabry disease)	
AMT-130 (uniQure)	**Gene Therapy** (Huntington's disease)	
DTX401 (Ultragenyx)	**Gene Therapy** (Glycogen story disease type 1a)	

2025 EUROPEAN UNION CLINICAL PIPELINE

Therapy Name (Developer)	Therapy Type (Indication)	Regulatory Status
Vyjuvek (Krystal Biotech)	**Gene Therapy** (Dystrophic epidermolysis bullosa)	EMA CHMP recommended approval; pending EU Commission decision
RPL102 (Rocket Pharmaceuticals)	**Gene Therapy** (Fanconi anemia)	EMA MAA accepted
Obe-cel (Autolus Therapeutics)	**CAR-T** (B-cell acute lymphoblastic leukemia)	
UM171 (ExCellThera)	**Cell Therapy** (Hematological malignancies in patients who lack a readily available suitable donor)	
Lifileucel (Iovance Biotherapeutics)	**Cell Therapy** (Metastatic melanoma)	
etuvetidigene autotemcel (Fondazione Telethon)	**Gene Therapy** (Wiskott-Aldrich syndrome)	EMA MAA submitted
Elevidys (Sarepta Therapeutics and Roche)	**Gene Therapy** (Duchenne muscular dystrophy)	
Nadofaragene firadenovec (Ferring Pharmaceuticals)	**Gene Therapy** (High-grade, Bacillus Calmette-Guérin-unresponsive non-muscle invasive bladder cancer)	
OCU400 (Ocugen)	**Gene Therapy** (Retinitis pigmentosa)	EMA MAA submission possible
Ixo-vec (Adverum)	**Gene Therapy** (Wet AMD)	

NOTABLE PIPELINE TRENDS

The regulatory momentum for gene therapies to treat rare genetic diseases continues in the U.S., with 5+ approvals possible in 2025 after seeing eight approvals between 2023 and 2024.

After only having three product approvals over the past two years, the European Union may see a rebound, with five or more potential approvals.

This year could mark the first approval of a cell therapy to treat chronic kidney disease.

Commercialization and Market Trends

BLOCKBUSTER WATCH

Only two cell and gene therapies have achieved revenue of $1 billion+ a year. As more cell and gene therapies accelerate toward global markets, we'll monitor which ones are close to achieving blockbuster status.

BLOCKBUSTER STATUS ACHIEVED

Zolgensma ▸ **2021** ✓ | **Yescarta** ▸ **2022** ✓

THERAPIES NEAR BLOCKBUSTER STATUS

THERAPY	TRACKING TO 2024 SALES MILESTONE	BLOCKBUSTER STATUS
Carvykti	→	$963 million
Elevidys	→	$821 million
Breyanzi	→	$747 million

Notable Q1 2025 Financing Deals

AstraZeneca

Acquisition of EsoBiotec

$425 million initial payment
Additional $575 million contingent on milestones

Focus: In vivo cell therapies for oncology and immune-mediated diseases

Beam Therapeutics

Underwritten Offering

$500 million

Focus: In vivo base editing programs for various disorders

Solid Biosciences

Underwritten Offering

$200 million

Focus: Gene therapies to treat rare neuromuscular and cardiac diseases

Tune Therapeutics

Series B Financing

$175 million

Focus: Epigenetic editing therapy to treat Hepatitis B

Lead investors: New Enterprise Associates, Yosemite, Regeneron Ventures, and Hevolution Foundation

Umoja Biopharma

Series C Financing

$100 million

Focus: In vivo CAR-T therapies for oncology and autoimmune disorders

Lead Investors: Double Point Ventures and DCVC Bio

Be Biopharma

Series C Financing

$92 million

Focus: B-cell therapies to treat hemophilia B hypophosphatasia

Lead Investors: ARCH Venture Partners, Atlas Venture, RA Capital Management, Alta Partners, Longwood Fund, Bristol Myers Squibb, and Takeda Ventures

Rhygaze

Series A Financing

$86 million

Focus: Gene therapy for optogenetic vision restoration in diseases causing blindness

Lead investors: Google Ventures, Arch Venture Partners, F-Prime Capital, BioGeneration Ventures, and Novartis Venture Fund

A2 Biotherapeutics

Series C Financing

$80 million

Focus: CAR-T cell therapies using the Tmod platform to treat high unmet need in cancers

Lead Investors: The Columb Group and Samsara BioCapital

Arbor Biotechnologies

Series C Financing

$73.9 million

Focus: Gene editing programs that target diseases in the liver and central nervous system

Lead Investors: ARCH Venture Partners and TCGX

XyloCor Therapeutics

Series B Financing

$67 million

Focus: Gene therapy to treat cardiovascular diseases

Lead Investors: Jeito Capital, EQT, Fountain Healthcare Partners, and Lumira Ventures

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 15-17, 2025
meetingonthemed.com

Cell and Gene Meeting on the Mesa
Phoenix, AZ

October 6-8, 2025
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: Elaine Blauser, eblauser@alliancerm.org

STAY ENGAGED

BECOME A MEMBER
Contact: Robin Muthig, rmuthig@alliancerm.org ↗

MEDIA INQUIRIES
Contact: Stephen Majors, smajors@alliancerm.org ↗

Alliance *for* Regenerative Medicine

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