



Alliance *for*
Regenerative
Medicine



STATE *of the* INDUSTRY BRIEFING

Industry Update

The Sector Enters a Disciplined, Sustainable Growth Cycle

**Tim Hunt, CEO, Alliance for
Regenerative Medicine**

January 12, 2026

Patient stories that illustrate where CGT is going



Baby KJ

A dedicated team of scientists, physicians, biotech leaders, and regulators saved Baby KJ's life with a groundbreaking bespoke base-editing treatment – and this inspiring moment has opened the door to new pathways that will benefit many patients



Marci McCue

In 2025, Marci became the first patient to participate in a CAR-T clinical trial for multiple sclerosis.

"I don't have to worry that I am going to wake up tomorrow and lose function... That fear is not there anymore."





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**Companies are emerging
stronger from hard lessons**

New science inevitably meets early challenges



Early scientific innovation...

Early investment fueled strong science that undoubtedly helped patients and catalyzed the field.



...has met commercial hurdles

However, commercial viability for some products was limited because of one or more factors:

1



Very small patient populations

2



Safety concerns and/or onerous conditioning regimens

3



Entrenched competition

4



Access and reimbursement challenges



But CGT companies are adapting by:

Examples



Focusing on first/best-in-class opportunities with larger patient populations and very high unmet need



Rett Syndrome

Duchenne Muscular Dystrophy

Huntington's Disease

AATD

Parkinson's Disease

Multiple Myeloma



Addressing barriers to patient access



Outpatient:

50% of Carvykti use is in outpatient setting; emerging clinical safety profiles of CAR-T for autoimmune suggests suitability for outpatient use

In-Vivo:

Strong clinical proof-of-concept for gene editing and CAR-T cell therapies



Adjusting approaches to improve safety



Rocket Pharma:

FDA lifted clinical hold after recalibration of dosing and revision of immunomodulatory regimen for Danon Disease

Neurogene:

Discontinued high-dose arm in Phase 1/2 trial for Rett Syndrome

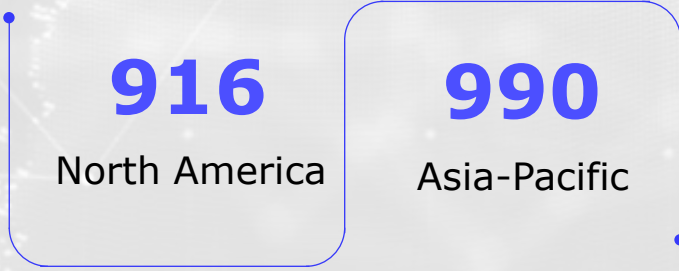
Beam Therapeutics:

Pursuing 'faster' cell collection and 'gentler' editing process for SCD





Global competition in the CGT sector is surging

The Asia-Pacific region surpassed North America in clinical trials for first time in 2025



Trial growth in the Asia-Pacific region has largely been driven by China's push to attract CGT trials



	H1 2025	Growth in 2H 2025	End 2025
 USA	822	8% →	890
 China	596	20% →	716

China



Surge in early-phase CGT trials due to policies that favor rapid initiation of in-human trials

Middle East



Regional competition with countries setting ambitious national plans to attract biotech investment

European Union



The EU Biotech Act takes an ambitious, holistic approach to CGT ecosystem



The EU Biotech Act marks a potential turning point for the CGT sector in Europe

The EU Biotech Act was released in December 2025



The European Commission's proposal properly recognizes that the success of the CGT sector depends on how regulation, investment, and delivery work together – and offers a welcome mix of incentives, funding, and regulatory improvements

Key benefits of the proposal for CGTs:



Accelerates the start of clinical trials



Creates ATMP centers of excellence



Enhances incentives for ATMPs, including a 12-month extension to supplementary patent rights



Provides EUR 10 billion in start-up capital for the field



Competition is a driver of modernization in the US



Geopolitical competition, scientific advancement, and a focus on disrupting the chronic-care model are catalyzing **new CGT regulatory frameworks and access models** in the United States.

“

We are absolutely committed to making sure the U.S. remains the center for cell and gene therapy research around the globe. We understand that this kind of research is absolutely consistent with the MAHA agenda. These are technologies that cure disease. We need to move away from the sick care model where we are treating chronic disease over the lifetime of the patient and actually cure the disease. ”

Robert F. Kennedy, Jr., HHS Secretary

Regulatory flexibility on CMC requirements



Broader acceptance of real-world evidence



Plausible Mechanism Pathway/ Gene editing as a platform



Updates of CAR-T label and REMS removal



Emphasis on CMMI CGT Access Model



FDA: More regulatory flexibility on CMC requirements

Announced January 11, 2026

Intended to help accommodate the unique characteristics of innovative CGTs while maintaining rigorous quality standards through appropriate control measures.

The logo for the U.S. Food and Drug Administration (FDA), consisting of the letters "FDA" in white, bold, sans-serif font, centered on a blue square background.

“Regulatory flexibility must be tailored for cell and gene therapies. These are common-sense reforms that will address the unique characteristics of cell and gene therapies and foster more innovation.”

– **Marty Makary, FDA Commissioner**

“CBER is proactively communicating about regulatory flexibilities that were previously applied case-by-case to select CGT therapies. By communicating these approaches broadly, we aim to expedite product development across the CGT field.”

– **Vijay Kumar, M.D., Acting Director, CBER’s Office of Therapeutic Products**

Unlocking new pathways and reducing development costs for ultra-rare and broader patient populations



New FDA 'plausible mechanism' pathway



The introduction of umbrella trials for gene editing treatments

A Global Effort

Agencies pursuing gene editing platform technology frameworks



But potential barriers to the FDA's positive vision began to emerge in H2 2025

Outstanding questions we hear as we enter 2026



Is OTP adequately staffed, including senior positions?







How to improve communication and minimize unexpected curveballs?



Is there an unhealthy focus on methodological purity over regulatory flexibility for serious diseases?



OTP Scorecard: We are already learning more in early 2026... with more data points coming

Company 	Program 	Milestone 	Timing 
Atara Biotherapeutics/Pierre Fabre	Epstein-Barr virus-associated post-transplant lymphoproliferative disorder	CRL	January 12, 2026
REGENXBIO	Hunter Syndrome	PDUFA	February 8, 2026
UniQure	Huntington's Disease	Type A meeting scheduled re: BLA	Next 30 days
Rocket Pharma	Severe leukocyte adhesion deficiency Type 1	PDUFA	March 28, 2026
Arcellx/Kite	Multiple Myeloma	BLA submission	Q1 2026
Orca Bio	Hematologic malignancies	PDUFA	April 6, 2026
Ultragenyx Pharmaceutical	MPS IIIA	BLA resubmission (2025 CRL)	Early 2026
Capricor Therapeutics	Duchenne Muscular Dystrophy Cardiomyopathy	CRL response (under existing BLA)	Early 2026

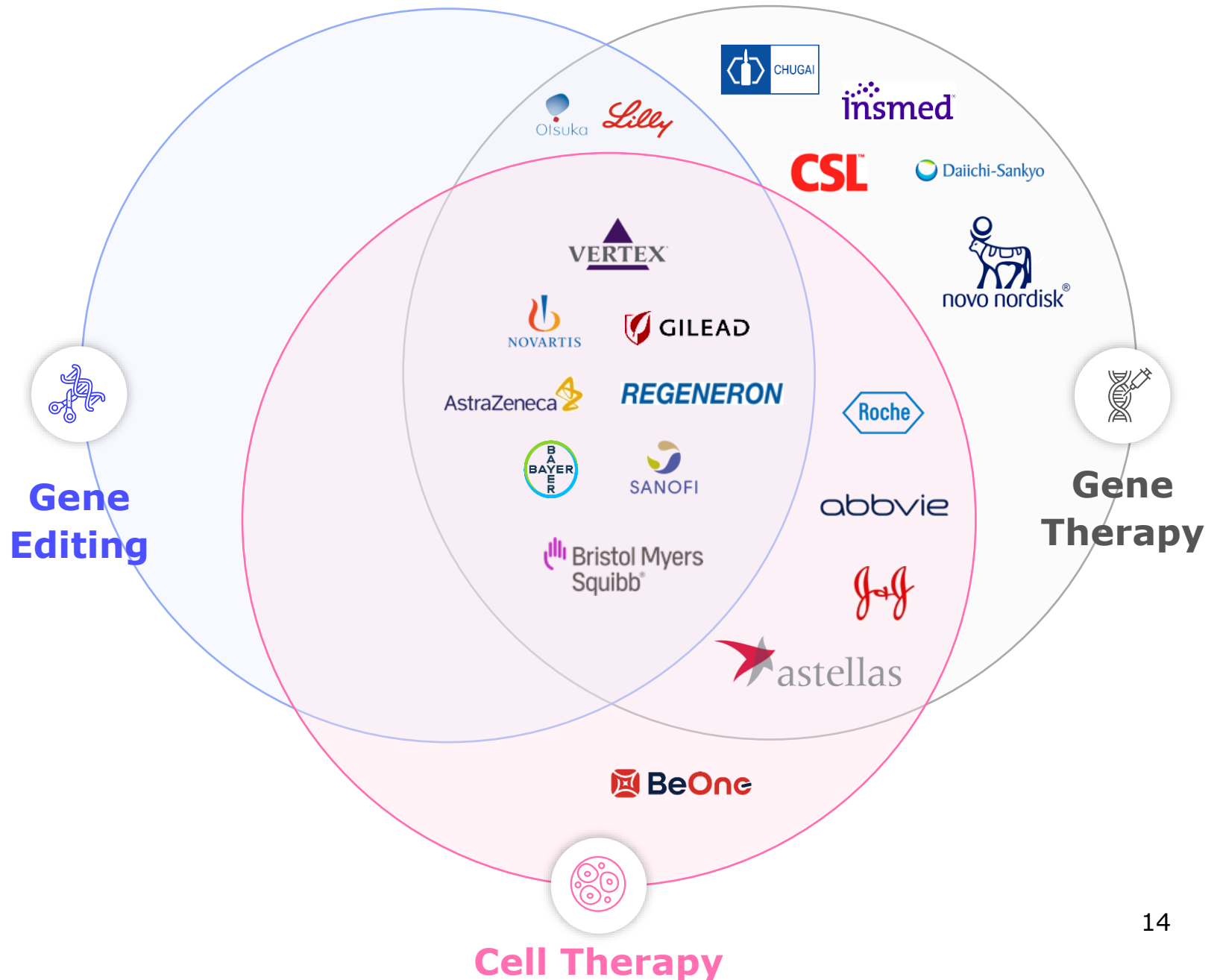




**Real commercial
opportunities are here ... and
emerging**

Strategic biopharma sees commercial promise across modalities

20 of 30 largest biopharma companies by market cap are investing in the development and/or commercialization of CGT



Scientific breakthroughs become commercial successes

Two CGT blockbusters from 2021-2024



Two more blockbusters in 2025



This year's newcomers are fueled by explosive YoY growth



111% YoY
(9 months ended September 2025)

101% YoY
(9 months ended September 2025)

Current blockbusters poised to become multi-billion dollar products



Consensus revenue forecasts show these blockbusters are poised to reach \$2B in revenue between 2026 and 2031

~6 more blockbusters are expected by 2031

~10 total blockbusters expected by 2031, according to analyst consensus data





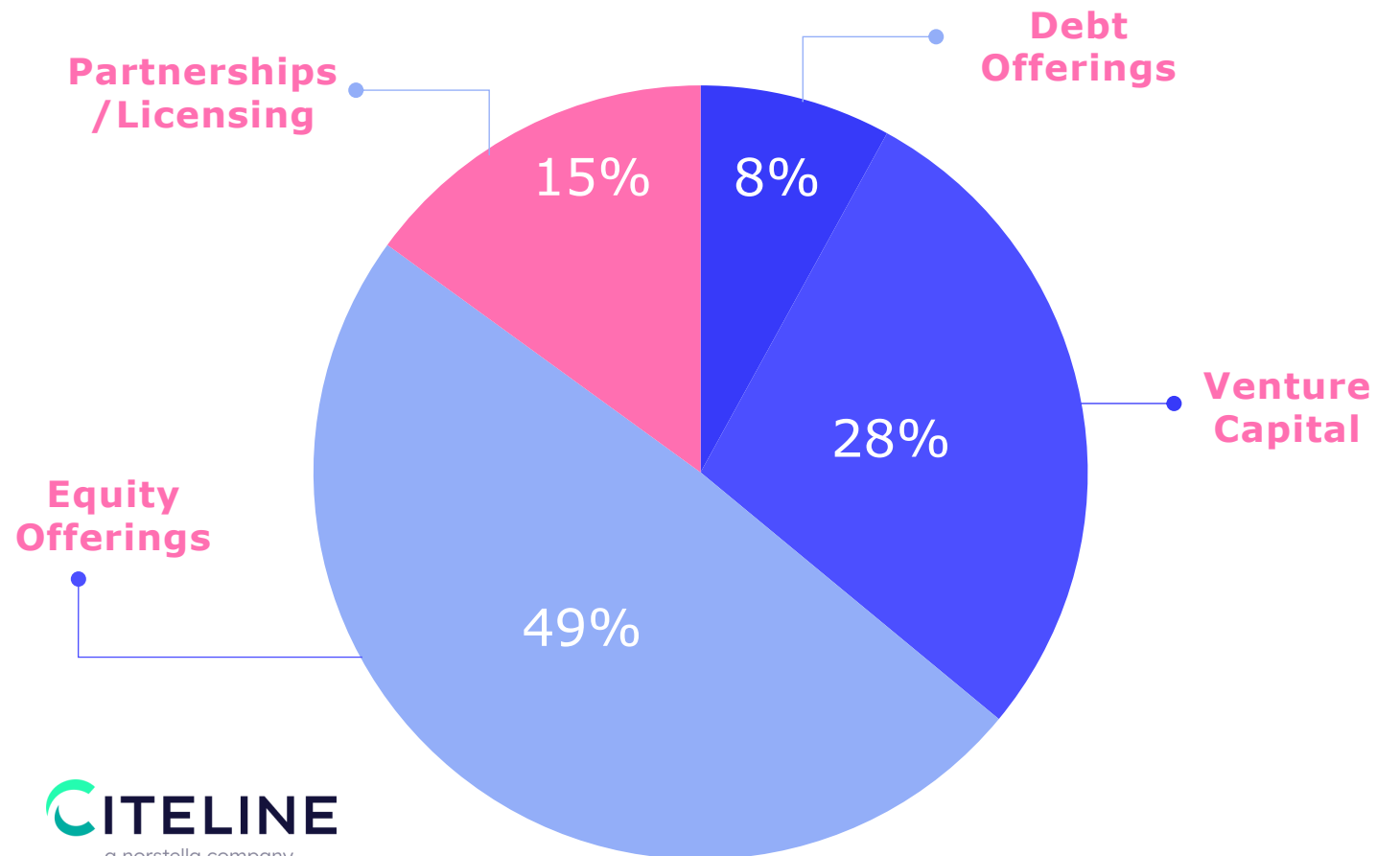
Investors and strategic biopharma drive a disciplined growth cycle

Despite some headwinds, the sector saw significant capital flow from multiple funding sources



2025 Sector Funding: \$11.1B from 216 financings

Investment by Funding Source



Source: Citeline and ARM analysis; upfront payments only; Equity offerings include IPOs, FOPOs, and PIPEs.



Strategic biopharma acquisitions also picked up in 2025; partnership activity was robust

Mergers & Acquisitions (\$1B+)



\$2.1B



\$1.5B



\$1B Upfront

Partnerships (\$100M+)

HOLOGEN AI



\$200M Upfront

REGENERON



\$150M Upfront



PREGENE

\$120M Upfront



\$115M Upfront



Investors made significant investments in private companies

Venture Financings (\$70M+)

Kriya

\$320M

TUNE
THERAPEUTICS

\$175M

ATSENA
THERAPEUTICS

\$150M

AAVANTGARDE

\$141M

SPLICEBIO

\$135M

XyloCor
Therapeutics

\$135M

Aspect
biosystems

\$115M

Aspen
NEUROSCIENCE

\$115M

Profluent

\$106M

NEURONA
THERAPEUTICS

\$102M

Umoja
BIOPHARMA

\$100M

Addition
Therapeutics

\$100M

trogenix

\$95M

RHYGAZE

\$86M

STYLUS

\$85M

azalea
therapeutics

\$82M

A2
BIO

\$80M

arbor
biotech

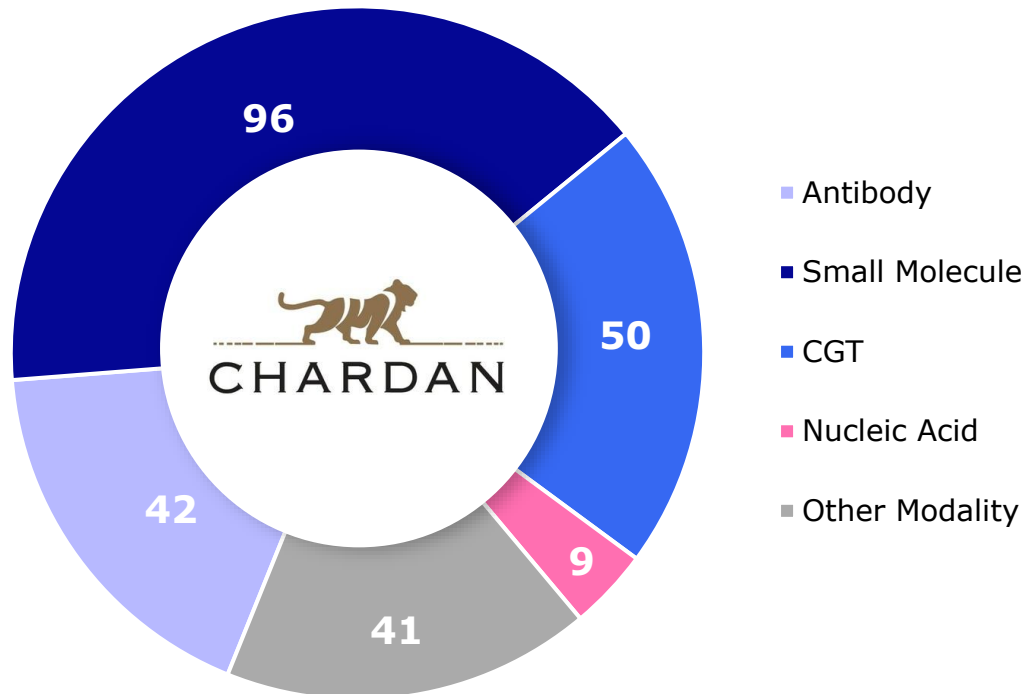
\$74M



Confidence in CGT

The sector comprised ~18% of the \$ value of all biotech venture financing in 2025, compared to ~15% in 2024

Count of private deals that fall within each of the 5 modality categories¹



Accounting for overlap, 50 out of the 222 (~23%) therapeutics deals were in CGT - either the cell therapy, gene therapy, or gene editing categories

This translates to **18%** of the \$ value of all private deals in biotech therapeutics in 2025



Companies tapped a more favorable public market... often on the heels of promising data

Public Offerings (\$100M+)



\$500M

uniQure

\$345M



\$200M



\$200M



\$150M



\$144M



\$144M



\$130M



\$125M



\$110M



\$100M



\$100M



\$100M



\$100M



\$100M





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Sector catalysts in 2026



Near-term regulatory outlook

Despite some speed bumps in 2025, the near-term approval pipeline remains strong



Current 2026
decisions pending



Other planned
submissions



Select indications:

- Stiff person syndrome (Kyverna Therapeutics)
- Retinitis pigmentosa (Nanoscope Therapeutics)
- End-stage ischemic heart failure (Mesoblast)
- Duchenne muscular dystrophy (REGENXBIO)
- Glycogen storage disease type Ia (Ultragenyx)
- AL amyloidosis (Immix Biopharma)
- Multiple myeloma (Arcellx/Kite)
- Hereditary Angioedema (Intellia Therapeutics)
- Intermediate/high-risk localized prostate cancer (Candel Therapeutics)



Notable potential clinical milestones in 2026 that can drive sector forward



Phase 1/2 data readout for AATD

Early 2026



Phase 1/2 data readout for Rett syndrome

1H 2026



Phase 1/2 data readout for PKP2-ACM

1H 2026



Phase 1/2 data readout for MG, SSc, and SLE

1H 2026



Phase 3 data readout for Duchenne muscular dystrophy

1H 2026



Phase 1/2 data readout for Rett syndrome

Mid-2026



Phase 2 data readout for gMG

2H 2026



Phase 3 data readout for retinitis pigmentosa

2H 2026



Phase 3 data readout for metastatic NSCLC and progressive disease despite ICI treatment

2H 2026



Phase 3 data readout for wet AMD

4Q 2026

This is why we say that CGT is entering a sustainable, disciplined growth cycle



The patient impact is irrefutable



Companies are learning and adapting to sector challenges



Science and global competition are accelerating system modernization



Real commercial opportunities are here... and emerging



Investors and strategic biopharma are enforcing and rewarding discipline... and driving more growth

