Alliance for Regenerative Medicine

CELL GENE

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

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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 immuno-modulatory 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

916
North America

990
Asia-Pacific

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

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	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** — FDA
- **Broader acceptance of real-world evidence** — FDA
- **Plausible Mechanism Pathway/Gene editing as a platform** — FDA
- **Updates of CAR-T label and REMS removal** — FDA
- **Emphasis on CMMI CGT Access Model** — CMS

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.

"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

FDA U.S. FOOD & DRUG ADMINISTRATION

EMA EUROPEAN MEDICINES AGENCY

MHRA Regulating Medicines and Medical Devices

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

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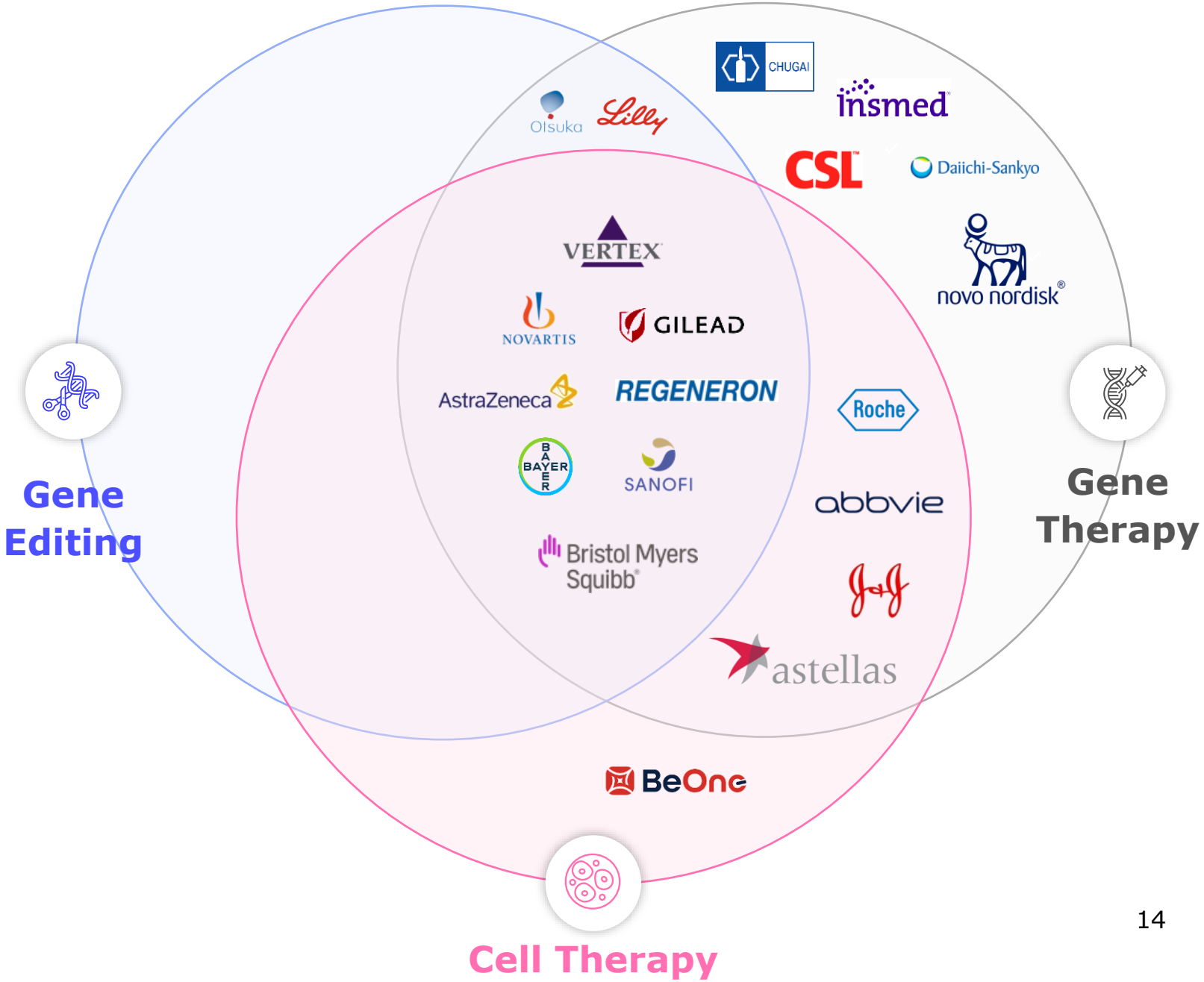
Real commercial opportunities are here … and emerging

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

zolgensma

YESCARTA

Two more blockbusters in 2025

CARVYKTI® (ciltacabtagene autoleucel)

Breyanzi® (lisocabtagene maraleucel) SUSPENSION FOR IV INFUSION

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

zolgensma

itvisma® (onasemnogene abeparvovec-brve) suspension for intrathecal injection

CARVYKTI® (ciltacabtagene autoleucel)

Breyanzi® (lisocabtagene maraleucel) SUSPENSION FOR IV INFUSION

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

*Analyst consensus data from GlobalData, compiled by Guidehouse

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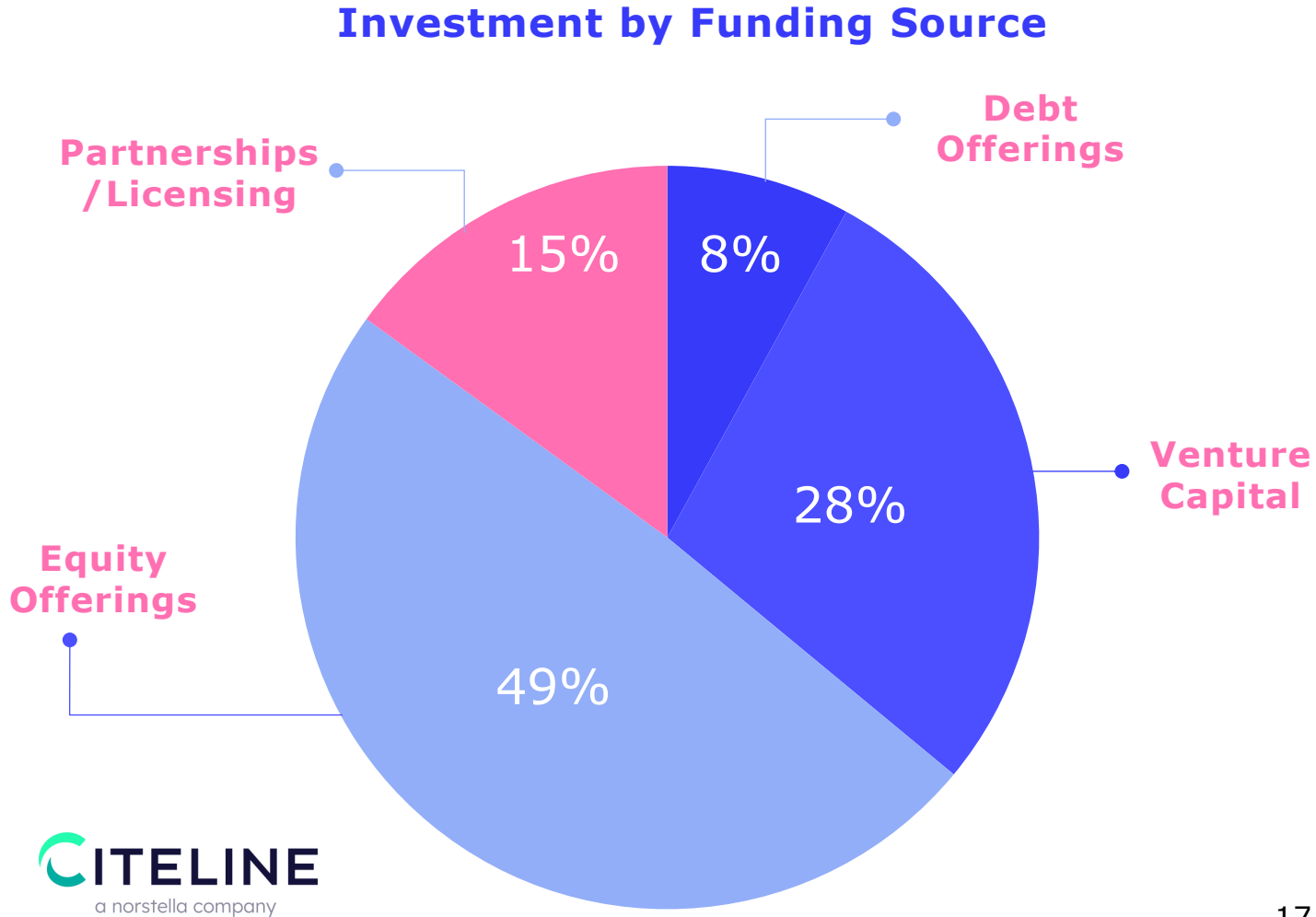
Investors and strategic biopharma drive a disciplined growth cycle

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

2025 Sector Funding: $11.1B from 216 financings



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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+)

abbvie
capstan therapeutics

$2.1B

Bristol Myers Squibb®
ORBITAL THERAPEUTICS

$1.5B

Lilly
verve THERAPEUTICS

$1B Upfront

Partnerships ($100M+)

HOLOGEN AI
MEIRAGTx

$200M Upfront

REGENERON
TESSERA

$150M Upfront

Kite A GILEAD Company
PREGENE

$120M Upfront

Chiesi
arbor biotech

$115M Upfront

Investors made significant investments in private companies

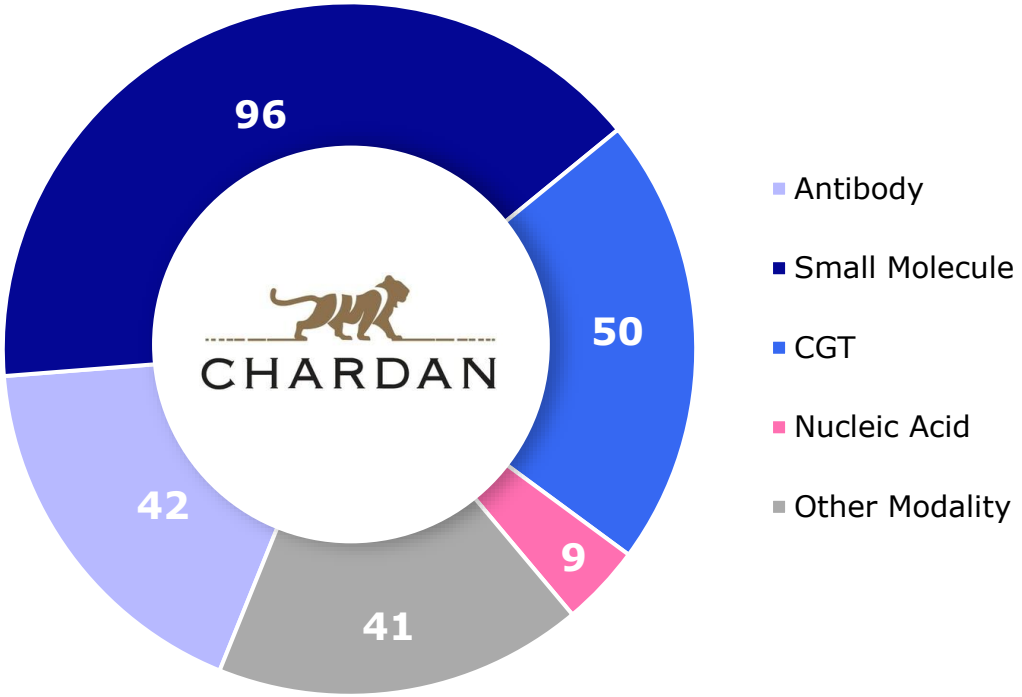
Venture Financings ($70M+)

Kriya	TUNE THERAPEUTICS	ATSENA THERAPEUTICS	AAVANTGARDE	SPLICEBIO	XyloCor Therapeutics
$320M	$175M	$150M	$141M	$135M	$135M
Aspect biosystems	Aspen NEUROSCIENCE	Profluent	NEURONA THERAPEUTICS	Umoja BIOPHARMA	Addition Therapeutics
$115M	$115M	$106M	$102M	$100M	$100M
trōgenix	RHYGAZE	STYLUS	azalea therapeutics	A2 BIO	arbor biotech
$95M	$86M	$85M	$82M	$80M	$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 categories[1]



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

Source: Chardan and publicly available filings, press releases. PitchBook. [1]Counts involve overlaps, thus total counts do not reflect total deals. Data as of 31 December 2025.

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

Public Offerings ($100M+)

Beam Therapeutics	uniQure	SOLID Biosciences	Taysha Gene Therapies	Capricor Therapeutics
$500M	$345M	$200M	$200M	$150M
LEXEO therapeutics	prime medicine	enGene	immatics	TENAYA Therapeutics
$144M	$144M	$130M	$125M	$110M
4DMT	BENITEC Biopharma silencing genes for life	Cabaletta Bio	IMMIX Biopharma	kyverna
$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

4

2

Other planned submissions

10+

3

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)

As of January 12, 2026

Notable potential clinical milestones in 2026 that can drive sector forward

Beam Therapeutics	Phase 1/2 data readout for AATD **Early 2026**
Taysha Gene Therapies	Phase 1/2 data readout for Rett syndrome **1H 2026**
Lexeo Therapeutics	Phase 1/2 data readout for PKP2-ACM **1H 2026**
Cabaletta Bio®	Phase 1/2 data readout for MG, SSc, and SLE **1H 2026**
REGENXBIO™	Phase 3 data readout for Duchenne muscular dystrophy **1H 2026**
Neurogene	Phase 1/2 data readout for Rett syndrome **Mid-2026**
kyverna®	Phase 2 data readout for gMG **2H 2026**
ocugen	Phase 3 data readout for retinitis pigmentosa **2H 2026**
Candel Therapeutics	Phase 3 data readout for metastatic NSCLC and progressive disease despite ICI treatment **2H 2026**
REGENXBIO™	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