Alliance for Regenerative Medicine

Reasons to Believe:

Innovation, Access & Sustainability in CGT

April 2026

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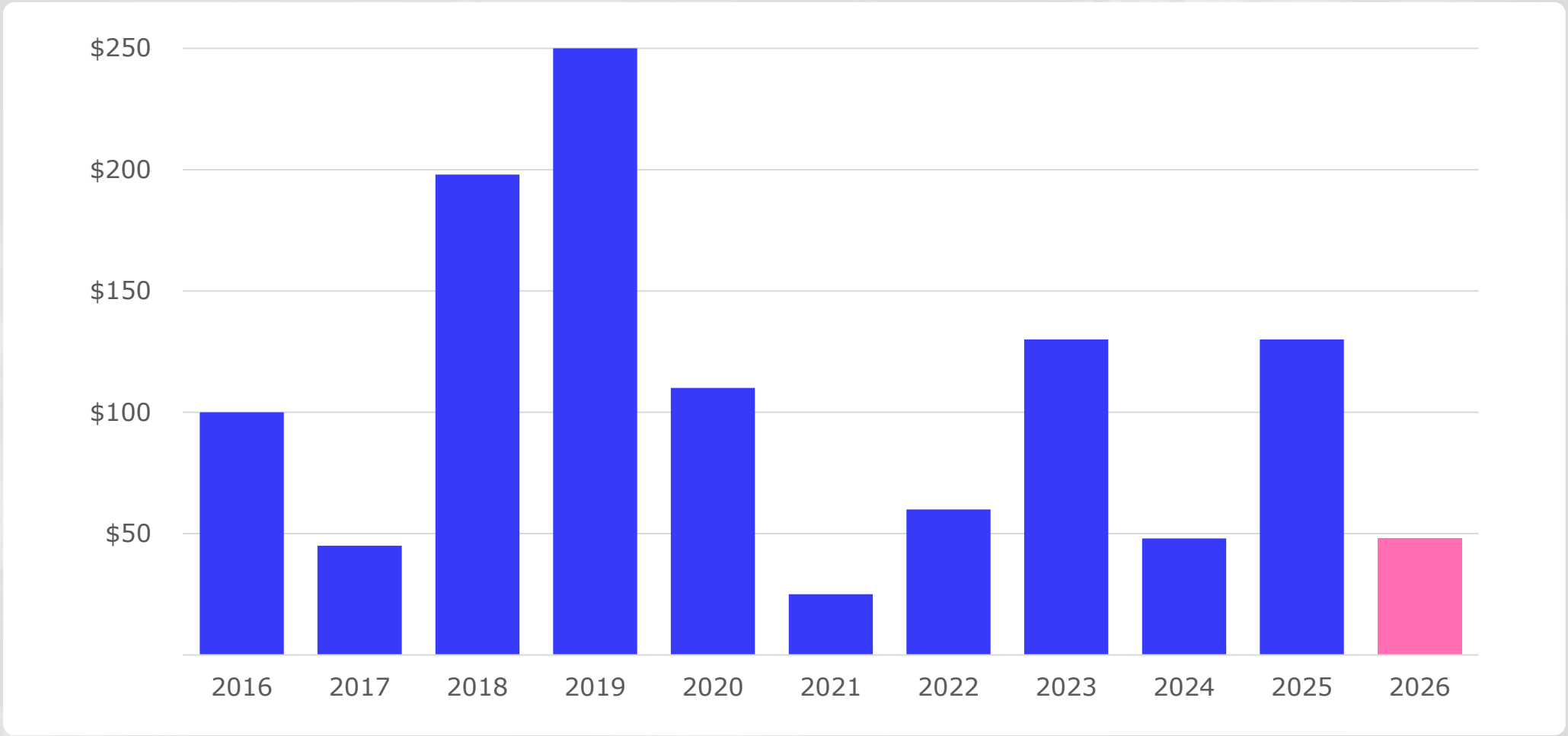
Q1 2026 CGT Capital Markets Update

Biopharma M&A is surging

With $48 billion in deals in Q1, 2026 is on pace to be best year since 2019



Source: Societe Generale; Endpoints News

Cell therapy ($18.3B) is one of the largest beneficiaries of biopharma M&A boom in 2026 YTD

Acquisitions

GILEAD
ARCELLX

$7.8B

Cell Therapy | Oncology & Autoimmune

Among the three largest acquisitions Gilead has ever completed and its largest in nearly a decade

Lilly
kelonia

$7B ($3.25B upfront)

In-Vivo CAR-T Therapy | Oncology & Autoimmune

Lilly's largest acquisition in its growing CGT portfolio

Lilly
ORNA™

$2.4B

In-Vivo CAR-T Therapy | Autoimmune

Largest ever preclinical acquisition

ucb
NEURONA THERAPEUTICS

$1.15B ($650m upfront)

Cell Therapy | Neurological Diseases

Builds on UCB's previous history of acquiring epilepsy-related therapeutics

Strong raises YTD from public and private companies across CGT modalities

2026 Public Offerings

SOLID BIOSCIENCES	Allogene THERAPEUTICS	CENTURY THERAPEUTICS	CANDEL THERAPEUTICS	MEIRAGTx
$240M PIPE	$200M Public Offering	$135M PIPE	$100M Public Offering	$100M Public Offering

2026 Venture Deals

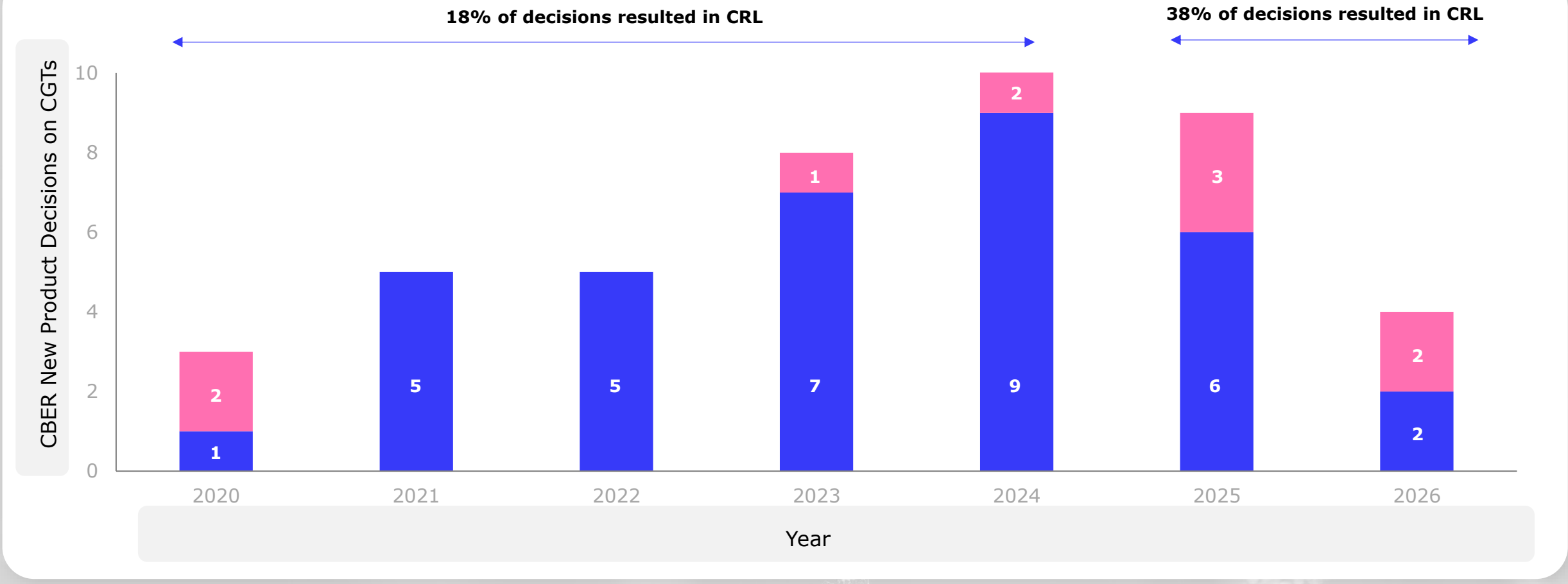
CELLARES	orcabio	MEDIPOST	RAY THERAPEUTICS	beacon therapeutics
$257M Series D	$250M Series F	$140M Private Funding	$125M Series B	$75M Series C

FDA: New Challenges. But Positive Signs Emerging.

FDA has issued an unusual number of surprise CRLs/reversals in 2026

Atara/ Pierre Fabre	**EBV+ PTLD**	CRL – January 2026	Single-arm trial no longer considered adequate for accelerated approval; Type A meeting scheduled
REGENXBIO	**Hunter syndrome**	CRL – February 2026	Uncertainty regarding natural history control and appropriateness of surrogate endpoint; Pursuing Type A meeting
uniQure	**Huntington's Disease**	Type A meeting – January 2026	Phase 1/2 studies compared to external control not sufficient to provide primary evidence of effectiveness; FDA strongly recommends Phase 3 RCT with sham surgery; pursuing Type B meeting

CRLs on CGTs have surged in 2025-2026



= New product approvals

= CRLs

A shift toward ‘methodological purity,’ along with structural issues, has taken hold at CBER

Philosophical shift to methodological purity

- Demands for randomized controlled trials, spurring concerns over ethics and practicality for rare diseases
- Regulatory flexibilities granted by Congress on rare disease programs are not being fully leveraged
- Skepticism around using natural history studies as evidence in clinical trials

Structural issues

- OTP is not adequately staffed; few senior leaders to guide reviews
- Communication with sponsors is often poor and sporadic

ARM & CGT community shined spotlight on the FDA

NEW YORK POST

Feb. 23, 2026

OPINION

How Trump's FDA is breaking his promise to America's patients

Authored by ARM CEO Tim Hunt

We urgently need a common-sense course-correction at FDA that puts patients first.

First, the agency should honor the commitments it originally made to companies and patients about what evidence would be acceptable for approval.

Second, it could further verify the strong signals that these medicines work by gathering additional data from patients *after* approving the therapies.

Finally, it could assemble a group of outside experts, known as an FDA Advisory Committee, to transparently review these medicines and listen to testimony from patients.

AXIOS

Mar. 3, 2026

FDA slammed over rare disease drug decisions

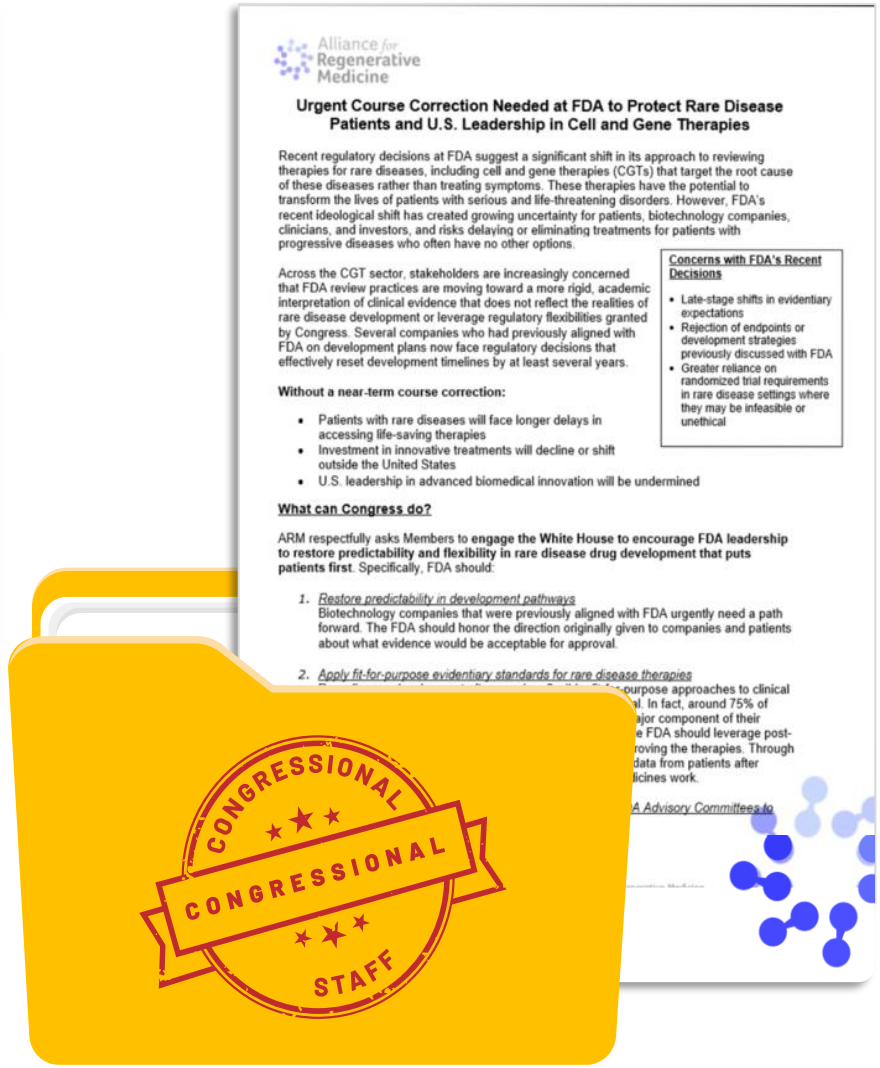
- Critics say a series of recent rejections fly in the face of the Trump administration's stated commitment to fighting rare diseases and finding innovative new approaches.
- "Something has changed in the last few months. I think of it as a new, toxic ideology that has crept into the FDA, and it's having tragic consequences," said Tim Hunt, CEO of the Alliance for Regenerative Medicine, which advocates for cell and gene therapies.
- Hunt described the agency as having adopted a mindset of "methodological purity."

The New York Times

Mar. 5, 2026

F.D.A. Faces Upset Over Denials of New Drugs

"The F.D.A. actions are not backing up the positive rhetoric about accelerating development of cell and gene therapies," said Stephen Majors, vice president of communications for the Alliance for Regenerative Medicine, a trade group. "And over the past few months, surprising regulatory U-turns on promising medicines for rare diseases are frustrating patients and confusing the companies trying to bring them to the market."



Members of Congress grew frustrated with FDA in Q1

Advocates here today will describe inconsistent review practices, shifting standards and redundant, often late appearing data requests that in many cases may not be driven by safety concerns, but by an overly cautious and rigid approach.

Sen. Rick Scott (R-Fla.), U.S. Senate Committee on Ageing, Feb. 26, 2026

We've seen a pattern of hesitation to use authorized flexibilities, limited communication with drug sponsors, failure to incorporate patient experience and real-world evidence reviews and FDA shifting its regulatory position on trial design at the last minute, rejecting drug applications and requiring new clinical trials the sponsor may be unable to perform.

Sen. Kirsten Gillibrand (D-NY), U.S. Senate Committee on Ageing, Feb. 26, 2026

The stories are so outrageous… It just appears that they're looking for excuses to say no.

You're expecting people to go through sham surgeries where they get holes drilled in their heads? That's just unbelievable.

Sen. Ron Johnson (R-WI), *Key US Senator Investigates FDA Over Rare-Disease Drug Denials*, Bloomberg, March 9, 2026

These actions are undermining patient confidence in the therapies that come to market, and that is undermining the ability of innovators to bring medicines to market with confidence.

Rep. Jake Auchincloss (D-MA), *CNBC Cures Event*, CNBC, March 3, 2026

Reasons for optimism despite setbacks

Mar. 6, 2026

FDA vaccine chief to leave the agency again

1mo • 2 min read

Mar. 25, 2026

DIVE BRIEF

FDA clears Denali drug in 'clear step' for rare disease biotechs

The approval of Denali's Hunter syndrome treatment, Avlayah, comes after a series of drug rejections and delays that had led to criticism of the FDA's stance on rare disease therapies.

Mar. 28, 2026

BIOPHARMADIVE

Rocket gene therapy cleared by FDA for rare immune disorder

April 23, 2026

REUTERS

Regeneron wins FDA approval for first gene therapy for genetic hearing loss

Programs that have reached recent alignment with FDA

Beam THERAPEUTICS · LEXEO therapeutics · Taysha GENE THERAPIES · NEUROGENE

CGT products still getting approved in 2025-2026

Product name	Approval date	Modality	Indication
ENCELTO	March 2025	Cell therapy	Macular telangiectasia type2
ZEVASKYN	April 2025	Cell therapy	Recessive dystrophic EB
PAPZIMEOS	August 2025	Gene therapy	Recurrent respiratory papillomatosis
ITVISMA	November 2025	Gene therapy	Spinal muscular atrophy
WASKYRA	December 2025	Gene therapy	Wiskott-Aldrich Syndrome
AVANCE	December 2025	Tissue therapy	Damaged peripheral nerves
KRESLADI	March 2026	Gene therapy	Severe leukocyte adhesion deficiency type I
OTARMENI	April 2026	Gene therapy	Genetic hearing loss

Promising programs announcing recent regulatory alignment with FDA

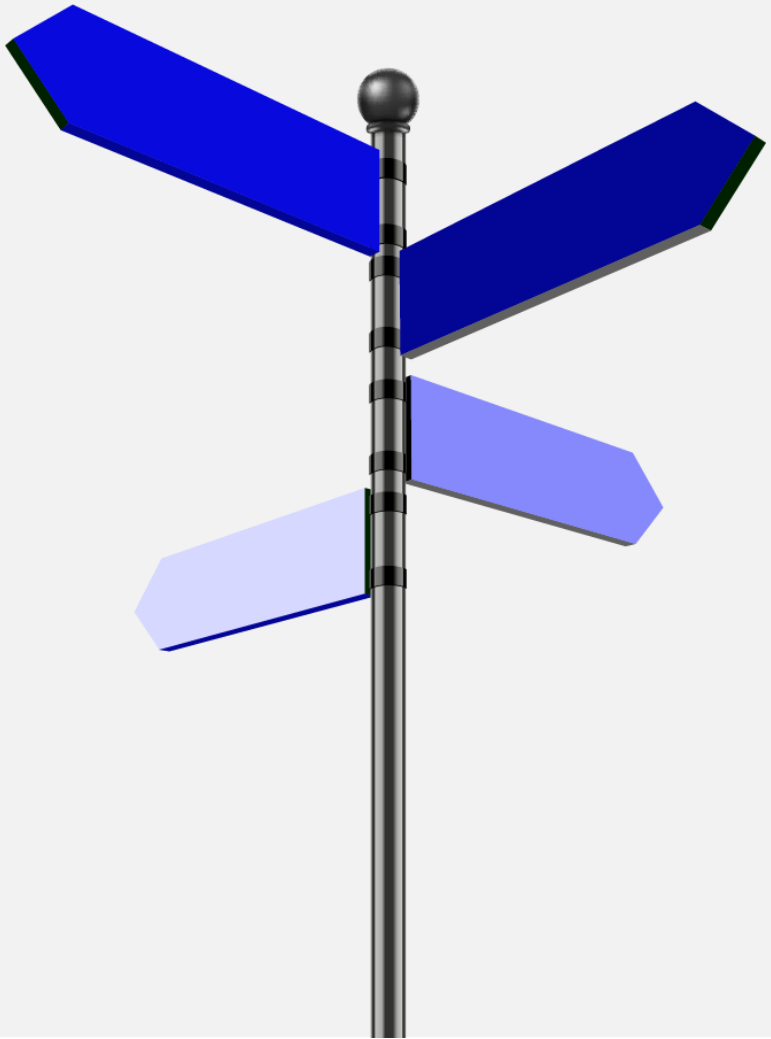
NEUROGENE	Selected to participate in FDA START Pilot Program and received FDA RMAT and Breakthrough Therapy designations for **NGN-401**, gene therapy for Rett syndrome	• 100% of participants enrolled in Embolden™ registrational clinical trial • EMBOLDEN is a single-arm trial designed to support a potential broad-label across a wide age range • Confirmed with FDA that commercial manufacturing scale is the same as current clinical manufacturing scale; no comparability studies needed
Taysha GENE THERAPIES	Received FDA Breakthrough and RMAT designations for **TSHA-102,** gene therapy for Rett syndrome	• Dosed multiple patients in REVEAL single-arm clinical trial • Received FDA clearance to initiate ASPIRE trial and written alignment on data for inclusion in BLA submission to enable broad label • Reached written FDA alignment on CMC requirements for BLA submission
LEXEO therapeutics	Received FDA Breakthrough Designation for **LX2006,** gene therapy for FA cardiomyopathy	• FDA open to a BLA submission for accelerated approval that includes data from Phase 1/2 trial pooled with new data from pivotal study • Lexeo plans to initiate a registrational study in the first half of 2026, pending FDA feedback on trial protocol in 2Q 2026.
Beam THERAPEUTICS	Received RMAT Designation for **BEAM-302**, a base-editing therapy to treat AATD; also accepted into FDA's CMC Development and Readiness Pilot	• Based on feedback from FDA, Beam intends to pursue accelerated approval based on primary endpoint of AAT biomarkers evaluated over 12 months • To support BLA submission, the company anticipates enrolling ~50 additional patients in expansion of Phase 1/2 trial; expects to initiate pivotal cohort in H1 2026

Source: Company press releases

ARM's recommendations to re-establish confidence and predictability in CBER

1. Honoring Previous Alignments	The FDA should honor its past alignment with companies. If they previously told companies that data would be acceptable for review, they should review that data.
2. Transparency & FDA Advisory Committees	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 their data.
3. Philosophy of Regulatory Flexibility	CBER should find problems early in the review cycle and try to resolve them favorably to enable medicines to be approved, embracing Congressionally authorized flexibility tools, including accelerated approval.
4. Bolstering Scientific Leadership	The new CBER director needs to rebuild the senior ranks of both CBER and the Office of Therapeutic Products with strong scientific leadership and management experience.

Signposts for CBER's future direction



1

Who will be the new head of CBER?

Status: Unknown

2

Is there a pathway to approval for stalled programs?

- Atara/Pierre Fabre, UniQure, REGENXBIO

Status: Unknown

3

Will level of regulatory flexibility be used?

- Using single arm trials? KRESLADI (March 26) | OTARMENI (April 23)
- Accelerated approval? KRESLADI (March 26) | OTARMENI (April 23)

4

What will happen with upcoming regulatory decisions and key BLA filings?

- Five PDUFA dates
- Four possible decisions on BLA filings

Status: Unknown

ARM is closely tracking FDA decisions in 2026

Approval Decisions to Date

Atara/Pierre Fabre
EBV+ PTLD
Type A Meeting scheduled
CRL issued
Jan. 9

REGENXBIO
Hunter Syndrome
Pursuing a Type A Meeting
CRL issued
Feb. 7
RMAT

Rocket Pharmaceuticals
Severe leukocyte adhesion deficiency Type 1
Approved
Mar. 28
RMAT

Regeneron
Genetic hearing loss
Approved
Apr. 23
CNPV
RMAT

BLA Delays

UniQure
Huntington's Disease
FDA asks for Ph3 RCT w/sham control
Pursuing a Type B Meeting
RMAT
Mar. 2

Upcoming PDUFA Dates

Orca Bio
Hematologic malignancies
RMAT
PDUFA delay of 3 months
PDUFA: July 6, 2026

Capricor Therapeutics
DMD cardiomyopathy
RMAT
BLA accepted after CRL
PDUFA: Aug. 22, 2026

Ultragenyx
Glycogen Storage Disease Type Ia
RMAT
PDUFA: Aug. 23, 2026

Ultragenyx
MPS IIIA
RMAT
BLA accepted after CRL
PDUFA: Sept. 19, 2026

Arcellx
Multiple myeloma
RMAT
PDUFA: Dec. 23, 2026

Upcoming Decisions on BLA Filings

Sangamo
Fabry Disease
Rolling BLA in progress
RMAT
Mar. 30

Intellia Therapeutics
Hereditary Angioedema
Rolling BLA initiated
RMAT
Apr. 27

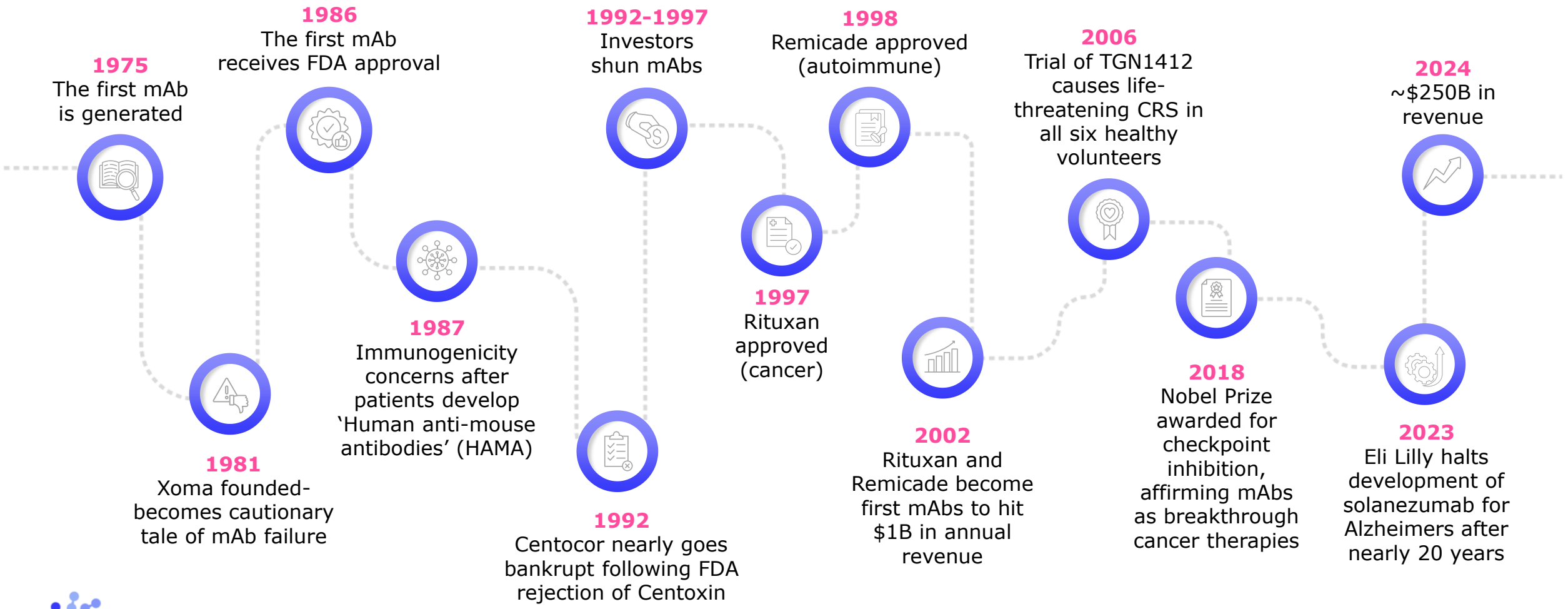
Nanoscope Therapeutics
Retinitis Pigmentosa
BLA submission possible
H1 2026

Kyverna Therapeutics
Stiff person syndrome
BLA submission possible
RMAT
H1 2026

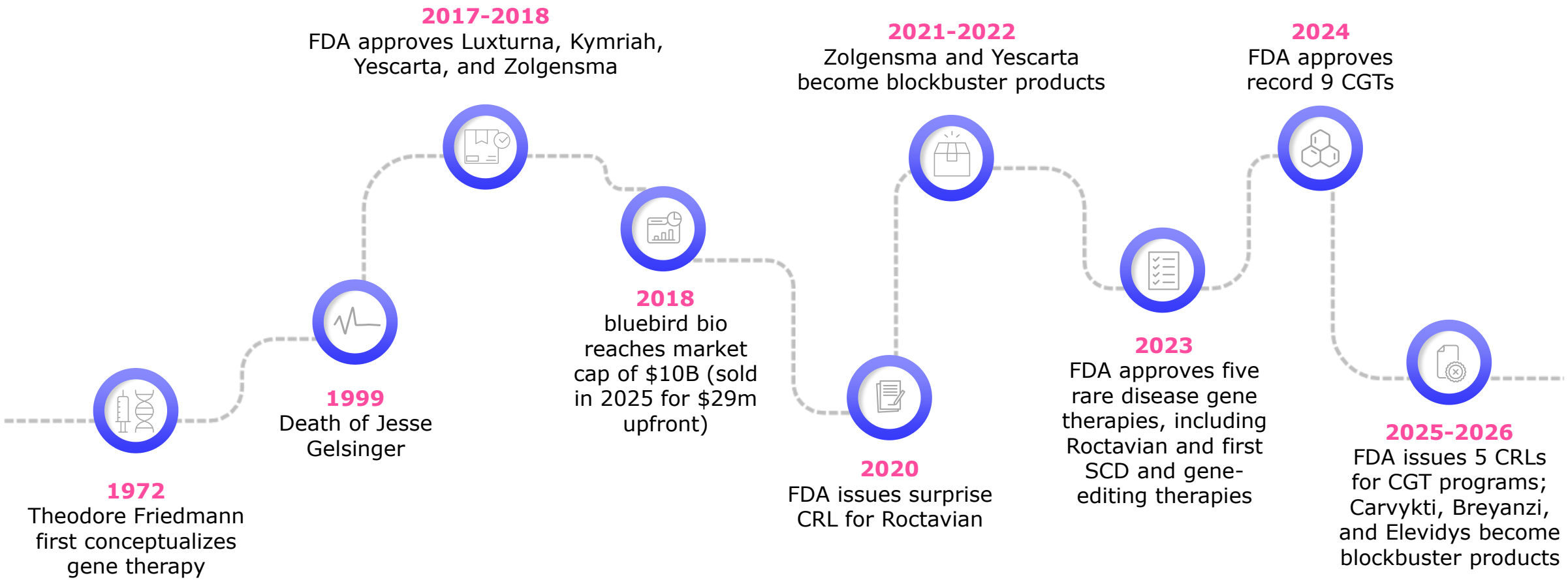
enGene
High-risk, BCG-unresponsive NMIBC
BLA submission possible
RMAT
H2 2026

CGT is Following a Familiar, Non-linear Path to Success

The (long) journey of monoclonal antibodies



The similar (non-linear) journey of CGTs in the US



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-in-class/best-in-class opportunities with larger patient populations

- **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; clinical safety 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

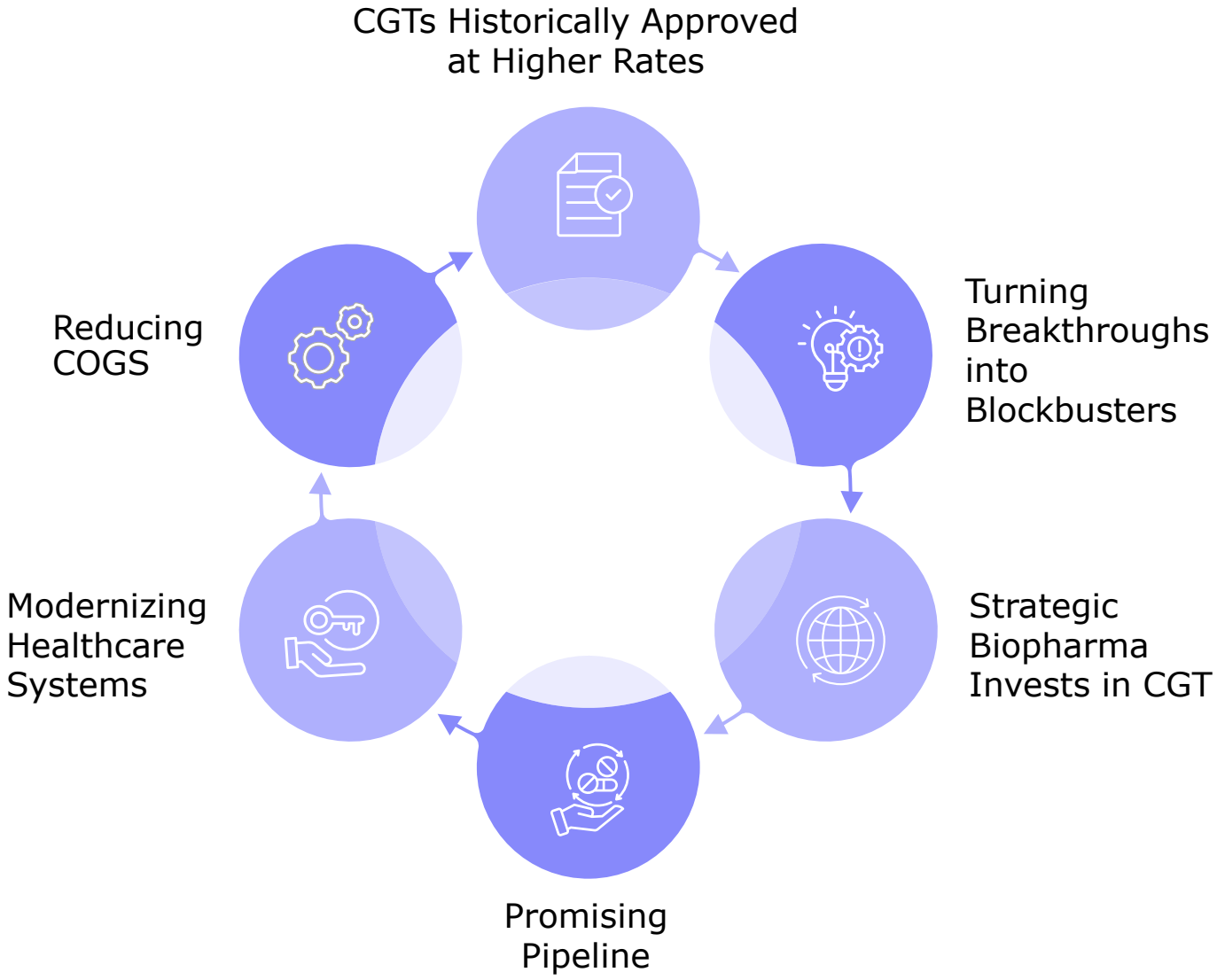
Neurogene:

Discontinued high dose arm in Phase 1/2 trial and implemented monitoring and treatment protocol at lower dose where hyperinflammatory syndrome has not been observed

Beam Therapeutics:

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

These key trends also position CGT for success



CGTs Historically Approved at Higher Rates Than Other Modalities

Historical data show CGTs are more likely to be approved than other modalities

Tufts' NEWDIGS performed a comprehensive peer-reviewed analysis of CGT clinical trial success rates from 1988 through 2023

Durable orphan gene therapies

18.5%
overall likelihood of FDA approval once entering Phase 1 clinical trials

2.5 times
more likely to receive FDA approval than all drugs once entering Phase 1 trials (IQVIA)

Hematological CAR-T/TCRs

40%
more likely to receive FDA approval than other oncology drugs (BIO 2011-2020)

CGT time to approval has in many cases been faster… and pivotal trial enrollment has been smaller

Orphan gene therapies

Zolgensma, Vyjuvek, Casgevy, and Elevidys all took ~5 years from start of clinical trials to FDA approval; Otarmeni took ~3 years

Among all FDA-approved orphan gene therapies, the average time to approval is <9 years – edging out antibodies and small molecules

Average pivotal trial enrollment for approved gene therapies is 42 patients

Hematological CAR-T/TCRs

Among the 7 FDA-approved CAR-Ts, the shortest time to approval is ~5 years (Tecartus, 57 months) while the average is ~7 years (86 months).

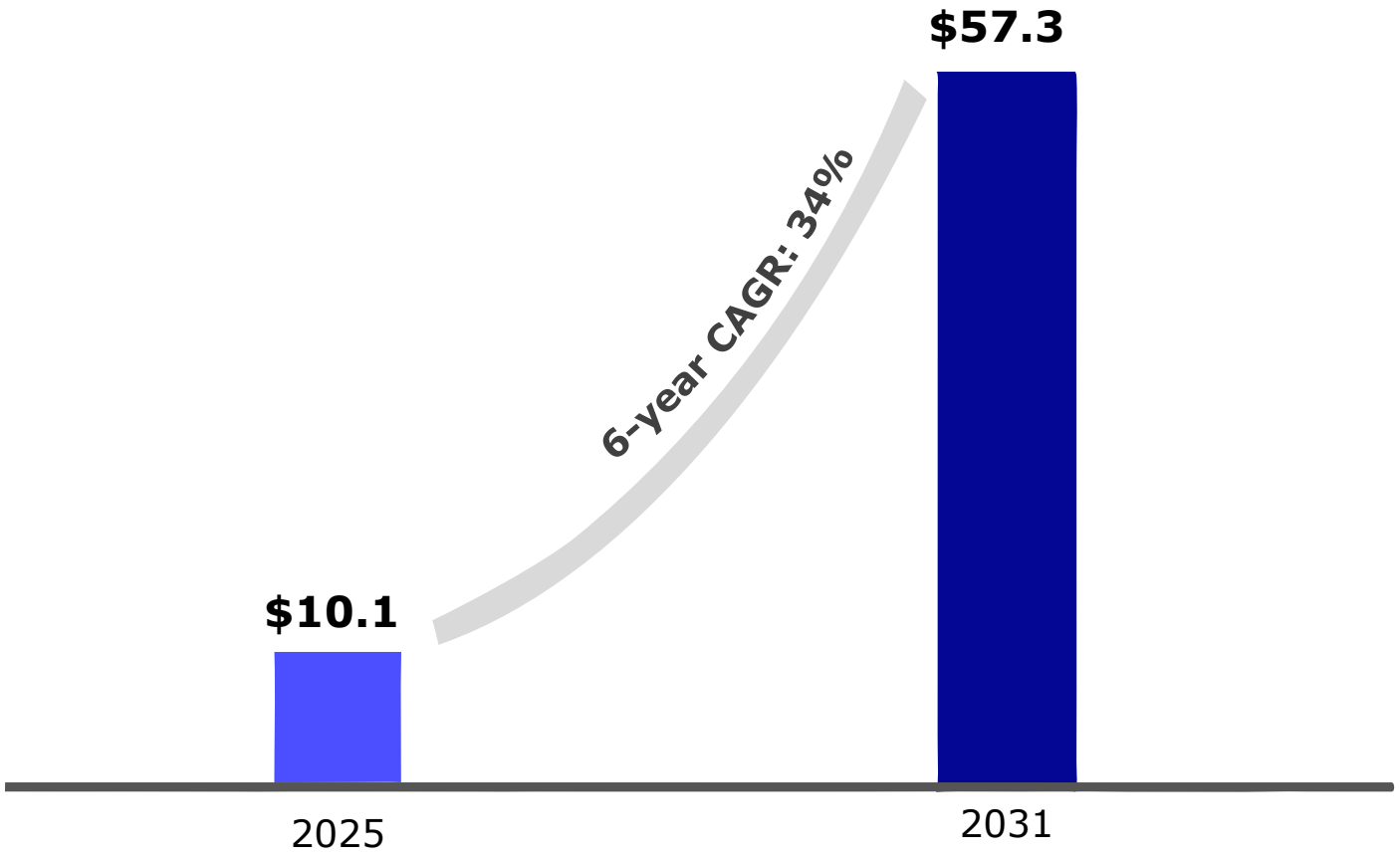
Average pivotal trial enrollment for approved CAR-T therapies is 141 patients

Sources: Citeline Biomedtracker; **https://www.nature.com/articles/d41573-021-00190-9**

Turning Breakthroughs into Blockbusters

Total size of CGT market expected to grow exponentially

CGT Market Size (In $Billions)



Source: GlobalData Analyst Consensus Estimates (April 2026), Guidehouse Analysis

Scientific breakthroughs become commercial successes

Two CGT blockbusters from 2021-2024

zolgensma | YESCARTA

Three more blockbusters in 2025

CARVYKTI (ciltacabtagene autoleucel) | Elevidys (delandistrogene moxeparvovec-rokl) suspension for intravenous infusion | Breyanzi (lisocabtagene maraleucel)

This year's newcomers are fueled by explosive YoY growth

CARVYKTI	Elevidys	Breyanzi
97% YoY	26%* YoY	82% YoY
(12 months ended December 2025)	(12 months ended December 2025)	(12 months ended December 2025)

*Based on reported US sales (Sarepta) & global sales (Roche)

Current blockbusters poised to become multi-billion dollar products

zolgensma
itvisma (onasemnogene abeparvovec-brve) suspension for intrathecal injection
CARVYKTI (ciltacabtagene autoleucel)
Breyanzi (lisocabtagene maraleucel)

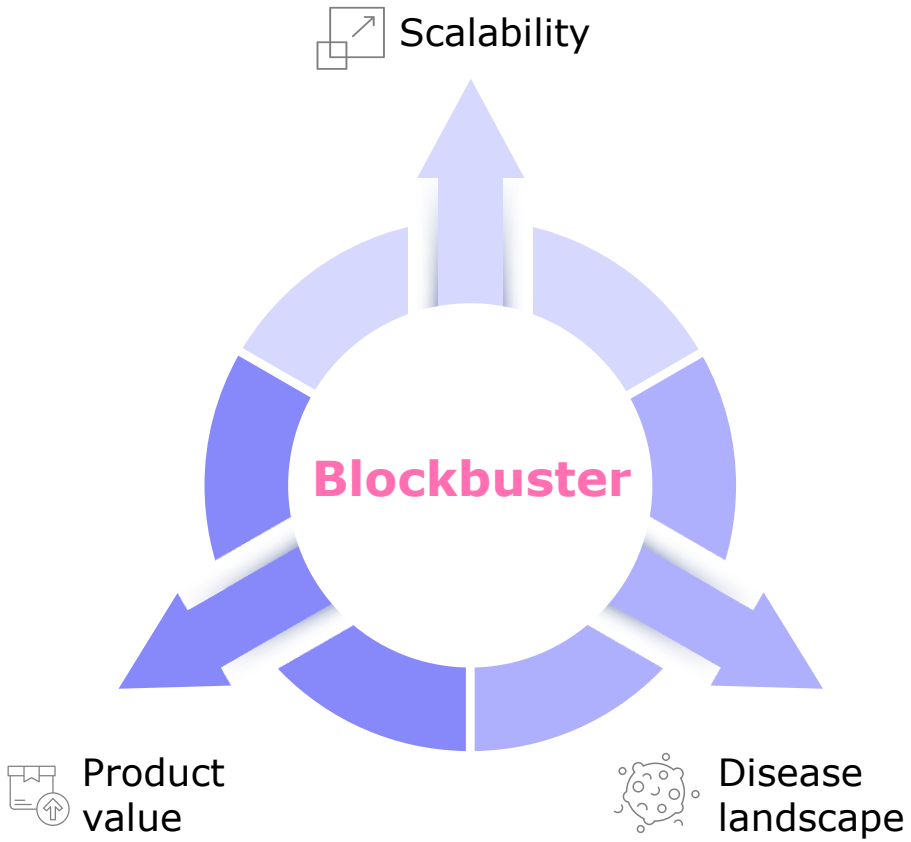
Consensus revenue forecasts show these blockbusters are poised to reach $2B in revenue between 2026 and 2031

~5 more blockbusters are expected by 2031

~10 total blockbusters expected by 2031 (and 20+ other products with $500m+ in sales), according to analyst consensus data

Analyst consensus data from GlobalData, compiled by Guidehouse

Successful CGTs share common characteristics



What are the ingredients of a CGT blockbuster?

1

Severe disease with high unmet need

2

Significant durable treatment effect

3

Global commercial and regulatory capability

4

Scalability in manufacturing

5

Clear value to justify premium pricing

6

Diagnostic availability

The ZOLGENSMA™ success story

NOVARTIS

- Approved in 62 countries
- Access established in over 45 countries (66% of revenue comes from ex-US in 2025)
- Over 5,000 young children treated worldwide; Itvisma recently FDA approved for children and adults above the age of two, with submissions completed in EU and Japan
- Blockbuster status since 2021 and projected to grow to $2 billion by 2028
- Establishing access capabilities early is critical for commercialization

The YESCARTA™ success story

GILEAD

- Approved in ~40 countries
- Access established in over 21 countries (60% of revenue came from ex-US 2025)
- Over 25,000 patients treated worldwide
- Blockbuster status since 2023 and projected to grow to over $2 billion in global sales by 2030
- Captured 92% of US academic ATCs, covering over 97% of the serviceable population. Expanding into the second-line relapsed or refractory DLBCL setting in Europe.

The CARVYKTI™ success story

- Commercially available in 14 countries across 294 sites worldwide with a 97% overall manufacturing success rate
- Significant use in outpatient settings (50%), with 145 authorized treatment centers (33% are community hospitals)
- Over 10,000 patients treated worldwide; 33% of patients treated in CARTITUDE-1 trial had ≥ five-year progression-free survival
- Net trade sales of $1.9 billion in 2025; projected to surpass $5 billion in peak annual sales
- 60% of usage comes from 2nd to 4th line setting

The VYJUVEK™ success story

Krystal®

- US approval in 2023 (first redosable gene therapy)
- Robust nationwide coverage in US and achieved strong access: over 660 reimbursement approvals
- Generated $730.3 million in net product revenue since U.S. launch; gross margin for 4Q and FY 2025 of 94%
- Launched in Germany, France and Japan in 2025; additional global expansion expected in 2026
- September 2025 FDA label expansion extended treatment to children at birth and allows self-managed dosing

The BREYANZI™ success story

Bristol Myers Squibb®

- Approved in over 35 countries
- Robust coverage by commercial and government insurance programs as well as community physicians receiving reimbursements
- Over 30,000 patients treated worldwide with BMY's CAR T cell therapies *(BREYANZI and ABECMA)*
- Achieved blockbuster status in 2025 with over $1.3 billion in worldwide sales
- First CAR-T cell therapy approved for MZL, as well as the first to reach the market for five different types of blood cancer (LBCL, CLL/SLL, FL and MCL) while achieving >90% manufacturing success rates

The ELEVIDYS™ success story

- Approved in nine countries
- Over 1,100 patients treated worldwide
- $1.3B in global sales in 2025 (~$899M US; ~400m ex-US)
- 850+ ambulatory individuals treated; In March 2026, announced enrollment underway in ENDEAVOR Cohort 8 to evaluate enhanced immunosuppression regimen in non-ambulant individuals with Duchenne
- Roche to conduct global, phase 3 placebo-controlled study of ~100 ambulatory patients to seek approval from the EMA and in other regions

Commercial strength of pipeline is emerging

Potential blockbuster markets with product(s) in or close to pivotal stage

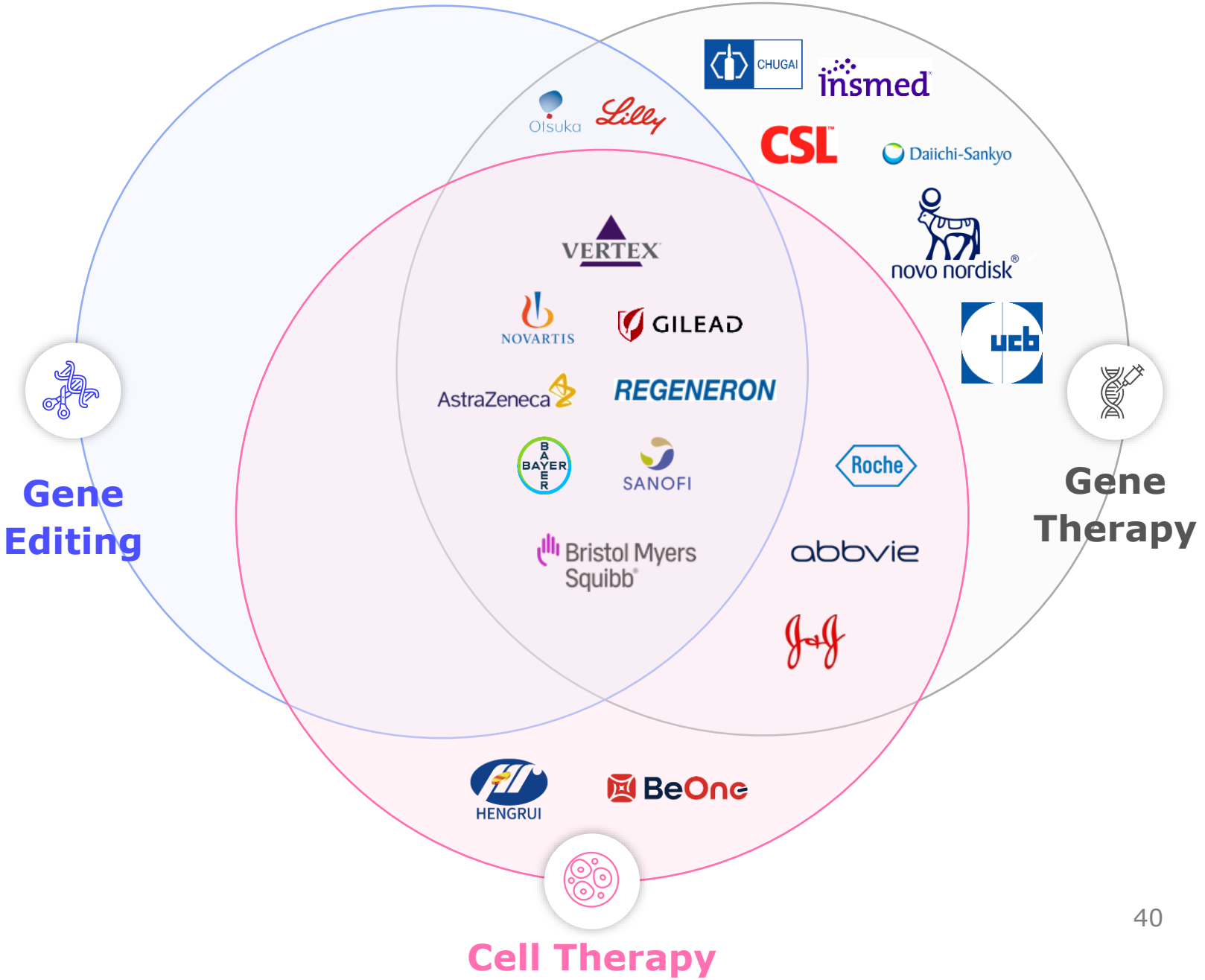
AATD	Beam Therapeutics, Krystal
PKP2	Tenaya Therapeutics, Rocket Pharma, Lexeo Therapeutics
Wet AMD	REGENXBIO, AbbVie, 4DMT
Huntington's Disease	uniQure
Rett Syndrome	Neurogene, Taysha Gene Therapies
Hereditary Angioedema	Intellia Therapeutics
Type 1 Diabetes	Vertex, Poltreg
Danon Disease	Rocket Pharma
DMD	REGENXBIO, Sarepta Therapeutics, Solid Biosciences
Myositis & SLE	Cabaletta Bio, Cartesian Therapeutics
Multiple Myeloma	Arcellx, Kite, A Gilead Company
Retinitis Pigmentosa	Ocugen, beacon therapeutics, Nanoscope Therapeutics, RAY Therapeutics
Parkinson's Disease	BlueRock Therapeutics, MeiraGTx, AskBio
Myasthenia Gravis	kyverna, Cartesian Therapeutics
Localized Prostate Cancer	Candel Therapeutics
ATTR	Intellia Therapeutics

Sources: GlobalData analyst consensus forecasts, compiled by Guidehouse; FactSet

Strategic Biopharma Invests in CGT

Strategic biopharma sees commercial promise across modalities

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



*Market cap as of April 24, 2026

Significant increase in strategic pharma acquisitions of emerging CGT companies in 2025 and 2026

Since 2017, the ~30 largest biopharma companies* have acquired 35+ CGT-focused biotechs, surpassing $200 billion in aggregate deal value

Data per BioCentury database;
Slide shows deals valued at >$1B; *includes companies that have recently fluctuated in and out of top 30 by market capitalization

Commercial results from strategic biopharma partnerships and acquisitions

Kite Pharma
YESCARTA (axicabtagene ciloleucel)
GILEAD
TECARTUS (brexucabtagene autoleucel)

CAR-T therapy for Blood Cancers & MCL
Yescarta: $1.5B sales in 2025
Tecartus: $344M sales in 2025

LEGEND BIOTECH
Johnson & Johnson
CARVYKTI (ciltacabtagene autoleucel)

CAR-T therapy for RRMM
$1.9B net trade sales in2025

Penn
NOVARTIS
KYMRIAH (tisagenlecleucel)

CAR-T therapy for ALL and LBCL
$381M sales in 2025

uniQure
CSL Behring
HEMGENIX etranacogene dezaparvovec-drlb

Gene therapy for hemophilia B
$57M sales in 1H2026 (July '25 – Dec. 25')

avexis
NOVARTIS
zolgensma (onasemnogene abeparvovec-xioi)

Gene Therapy for SMA
$1.2B sales in 2025

Roche
SAREPTA THERAPEUTICS
Elevidys delandistrogene moxeparvovec-rokl suspension for intravenous infusion

Gene Therapy for DMD
~1.3B sales in 2025

JUNO THERAPEUTICS
Bristol Myers Squibb
Breyanzi (lisocabtagene maraleucel)

CAR-T therapy for LBCL
$1.4B sales in 2025

Roche
Spark THERAPEUTICS
LUXTURNA voretigene neparvovec-rzyl for subretinal injection

Gene therapy for IRDs
$52M sales in 2025

Data per the Companies' financial statements (SEC 10-Q)

Promising Pipeline

Near-term regulatory outlook

Despite recent regulatory challenges, the near-term approval pipeline remains strong

2026 approvals

2 | 1

Current 2026 decisions pending

5 | 2

Other planned submissions

10+

2

Select indications:

- Mucopolysaccharidosis type IIIA (Ultragenyx)
- DMD cardiomyopathy (Capricor Therapeutics)
- Stiff person syndrome (Kyverna Therapeutics)*
- Retinitis pigmentosa (Nanoscope Therapeutics)
- Duchenne muscular dystrophy (REGENXBIO)
- Multiple myeloma (Arcellx/Kite)
- Inter/HR localized prostate cancer (Candel Therapeutics)
- End-stage ischemic heart failure (Mesoblast)
- Hereditary Angioedema (Intellia Therapeutics)**

As of April 23, 2026

*Would be first ever CAR-T therapy approved for autoimmune disease

**Would be first ever in-vivo gene editing therapy approved

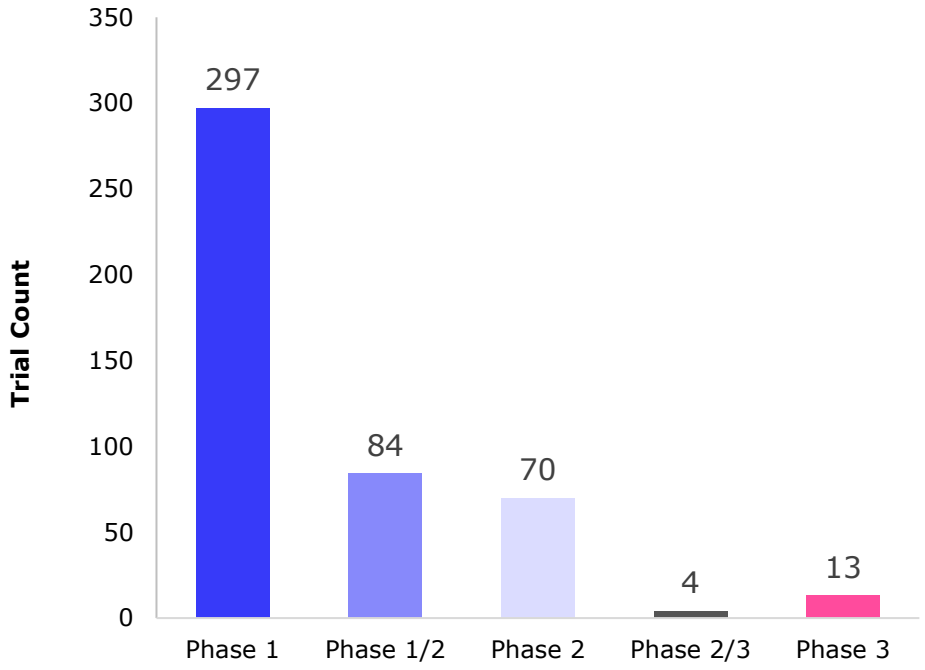
2026 is a catalyst-rich year for the CGT sector

LEXEO therapeutics	Phase 1/2 data readout for PKP2-ACM **1Q 2026**	✓
ultragenyx	Phase 3 data readout for OTC Deficiency **1Q 2026**	✓
Beam THERAPEUTICS	Phase 1/2 data readout for AATD **Q1 2026**	✓
Allogene THERAPEUTICS	Phase 2 data readout for first-line LBCL **Q2 2026**	✓
SOLID BIOSCIENCES	Phase 1/2 data readout for Duchenne muscular dystrophy **Q2 2026**	✓
ocugen	Phase 2 data readout for Dry AMD **Q2 2026**	✓
kyverna	Updated Phase 2 data readout for gMG and registrational trial primary analysis results for SPS - **Q2 2026**	✓
Intellia THERAPEUTICS	Phase 3 data readout on in vivo gene editing program for HAE **Q2 2026**	✓
Taysha GENE THERAPIES	Phase 1/2 data readout for Rett Syndrome **1H 2026**	
Cabaletta Bio®	Phase 1/2 data readout for gMG, SSc, and SLE **1H 2026**	
REGENXBIO™	Phase 3 data readout for Duchenne muscular dystrophy **2Q 2026**	
NEUROGENE	Phase 1/2 data readout for Rett Syndrome **Mid-2026**	
beacon therapeutics	Phase 3 data readout for X-linked Retinitis pigmentosa **2H 2026**	

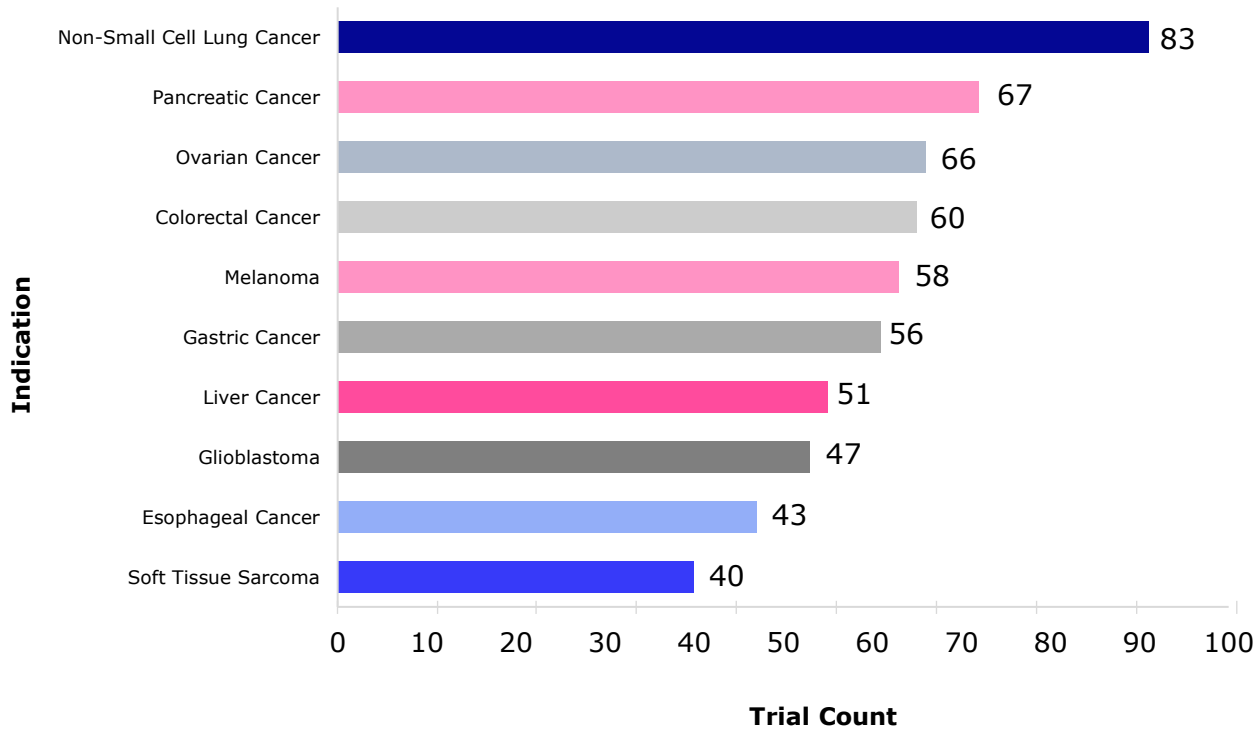
~400 ongoing CGT trials focused on solid tumors

Of the top 10 indications targeted, only one – melanoma – has an approved CGT

Solid Tumor Trials by Phase



Top 10 Most Explored Solid Tumor Indications

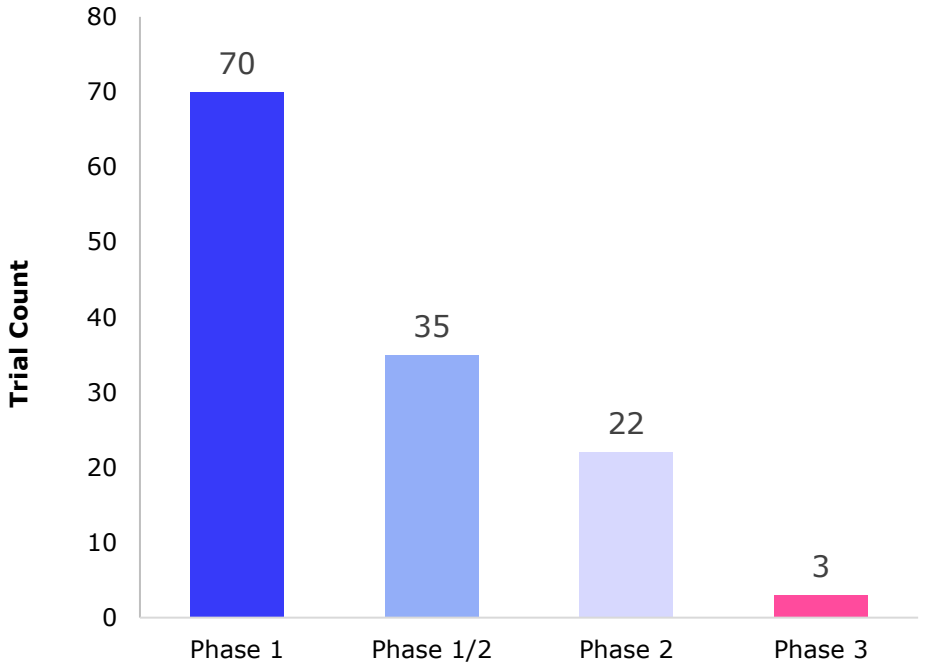


1. Visualization is limited to trials with clearly defined solid tumor targets

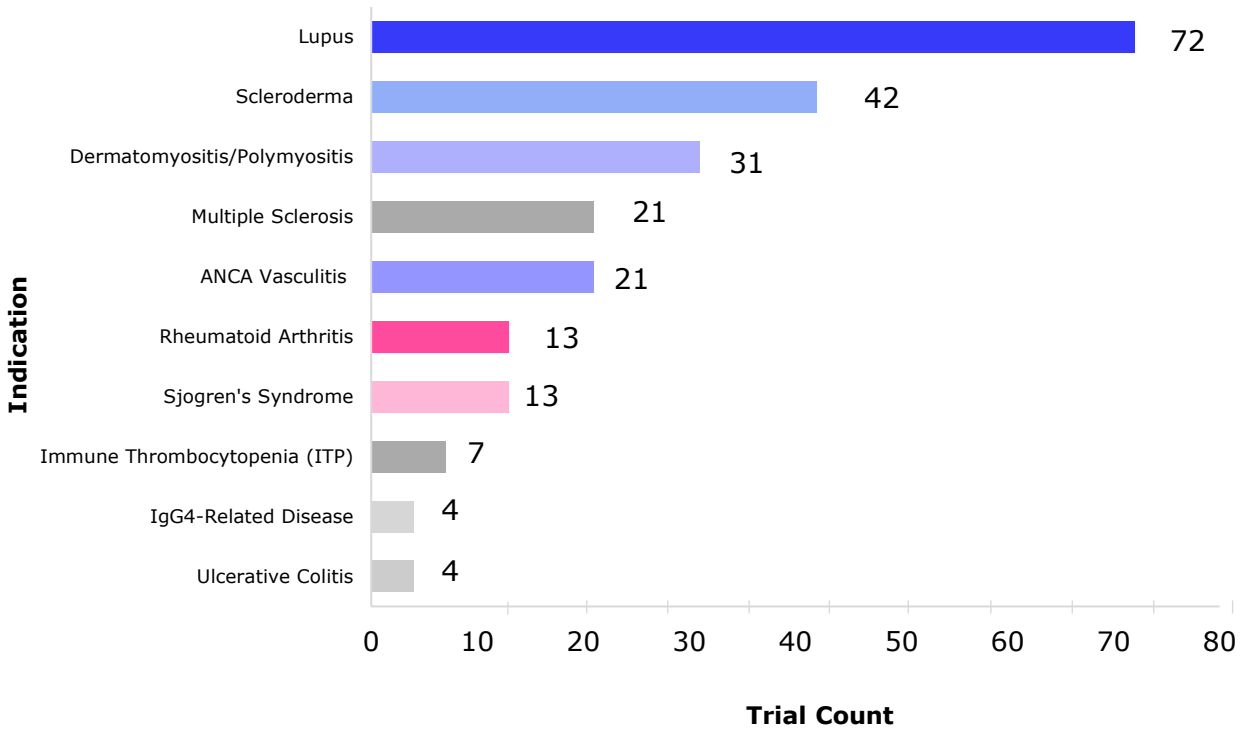
Promise in autoimmune diseases: ~100 ongoing trials

Significant activity in Phase 2 and 3

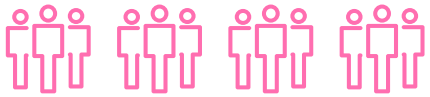
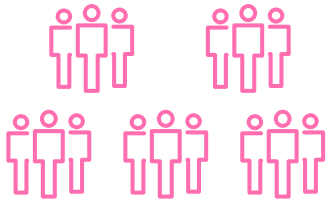
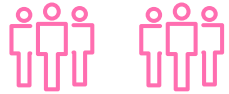
Autoimmune Trials by Phase



Top 10 Most Explored Autoimmune Indications



Prevalent disease breakthroughs are coming

Wet AMD	Parkinson's	Multiple Sclerosis	Type 1 Diabetes	HeFH / Premature CAD
				
5.7 million patients in US, EU and Japan	**10 million** patients worldwide	**1.5 million** patients in US, EU and Japan	**3.8 million** patients in US, EU and select geographies	**3 million*** patients in US and EU
Phase 3	**Phase 3**	**Phase 1/2**	**Phase 1/2/3**	**Phase 1b**
REGENXBIO, abbvie, 4DMT	BlueRock Therapeutics, Bayer, MeiraGTx	Bristol Myers Squibb, kyverna	Vertex, Sana Biotechnology	verve Therapeutics, Lilly

Source: Company estimates from Vertex Pharmaceuticals, Kyverna Therapeutics, REGENXBIO, BlueRock Therapeutics and Verve Therapeutics

* Patient figure is Heterozygous Familial Hypercholesterolemia (HeFH) only

Modernizing Healthcare Systems

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

CITELINE
a norstella company

	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

Draft guidance supporting 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

2025 FDA updates to CAR-T monitoring will significantly boost access

~~4 weeks away from home~~ → **2 weeks**

~~8 weeks no driving~~ → **2 weeks**

~~Risk Evaluation and Mitigation Strategy (REMS) requirements~~

Lowers cost and logistical barriers for patients

01
Makes it easier for physicians to administer CAR-Ts

02
Promotes CAR-T administration outside specialized treatment centers

LEERINK PARTNERS Research

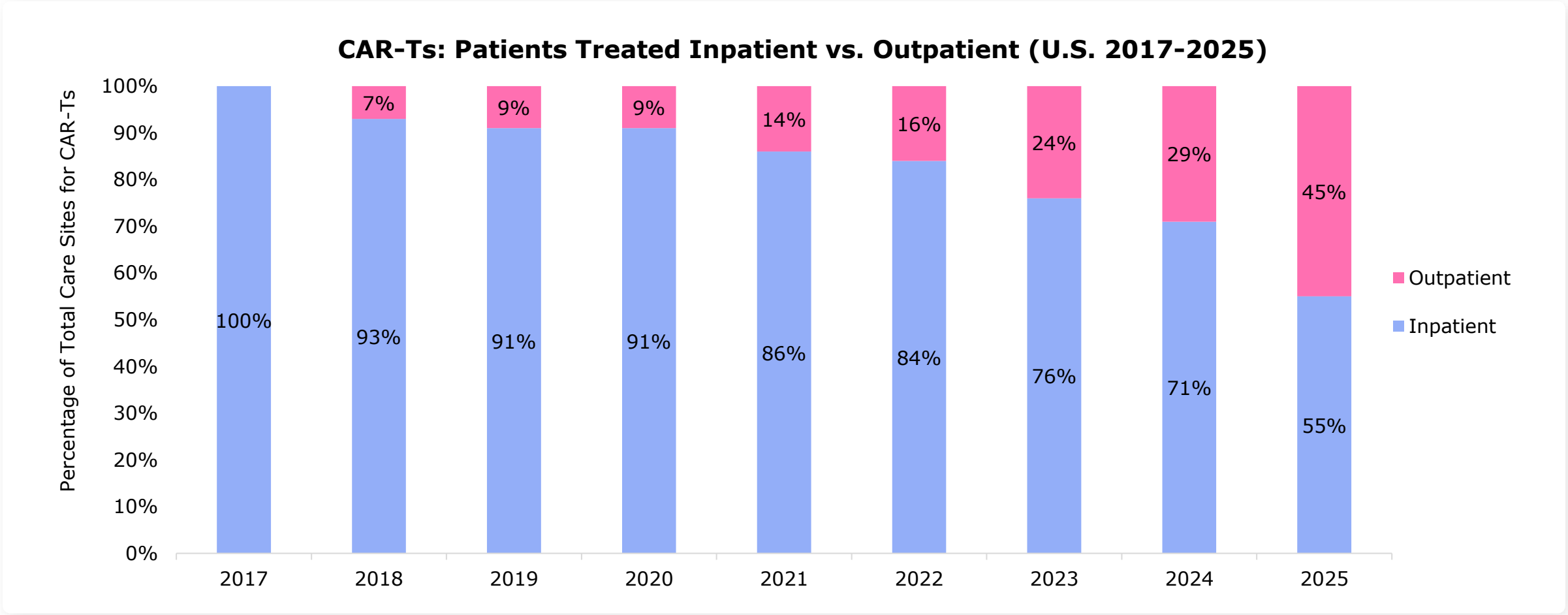
FDA Reduces Burden to Access CD19 & BCMA CAR-T, Likely Expanding Markets

> One KOL told us that if FDA reduced monitoring requirements and driving restrictions to 14 days, he would expect rates of CAR-T infusion to double

William Blair

> Overall, we view the removal of the REMS program for CAR-T cell therapies as a positive development for the space, as it supports the notion that the FDA is working to minimize red tape and increase access to potentially curative therapies for patients.

CAR-T label updates accelerating outpatient trend



Source: Guidehouse Analysis of McKesson Compile Patient Ready Data (U.S.). Data cut-off date, January 1, 2026.

Guidehouse

Center for Medicare and Medicaid Innovation's (CMMI) Cell and Gene Therapy Access Model

What is the model?

The model establishes a voluntary partnership among CMS, state Medicaid agencies, and Vertex/CRISPR & bluebird bio to set up and administer outcomes-based agreements (OBAs) for approved sickle cell gene therapies

What is the purpose?

The purpose is to expand access to SCD gene therapies for Medicaid patients; it should be particularly helpful for smaller states without the resources to administer OBAs

How many states are participating?

The model launched in 2025; 33 states, DC, and PR participating

Value of gene therapy increasingly recognized

01

Target devastating, often deadly diseases

The average life expectancy for rare diseases targeted by approved gene therapies is **<40 years** – HALF the normal lifespan

02

Target incredibly expensive diseases

The lifetime cost to manage most rare diseases targeted by approved gene therapies is several million $

03

Provide strong value for healthcare systems (a)

The Institute for Clinical and Economic Review (ICER) confirmed the high value of one-time durable gene therapies for SMA, SCD, MLD, hemophilia and more

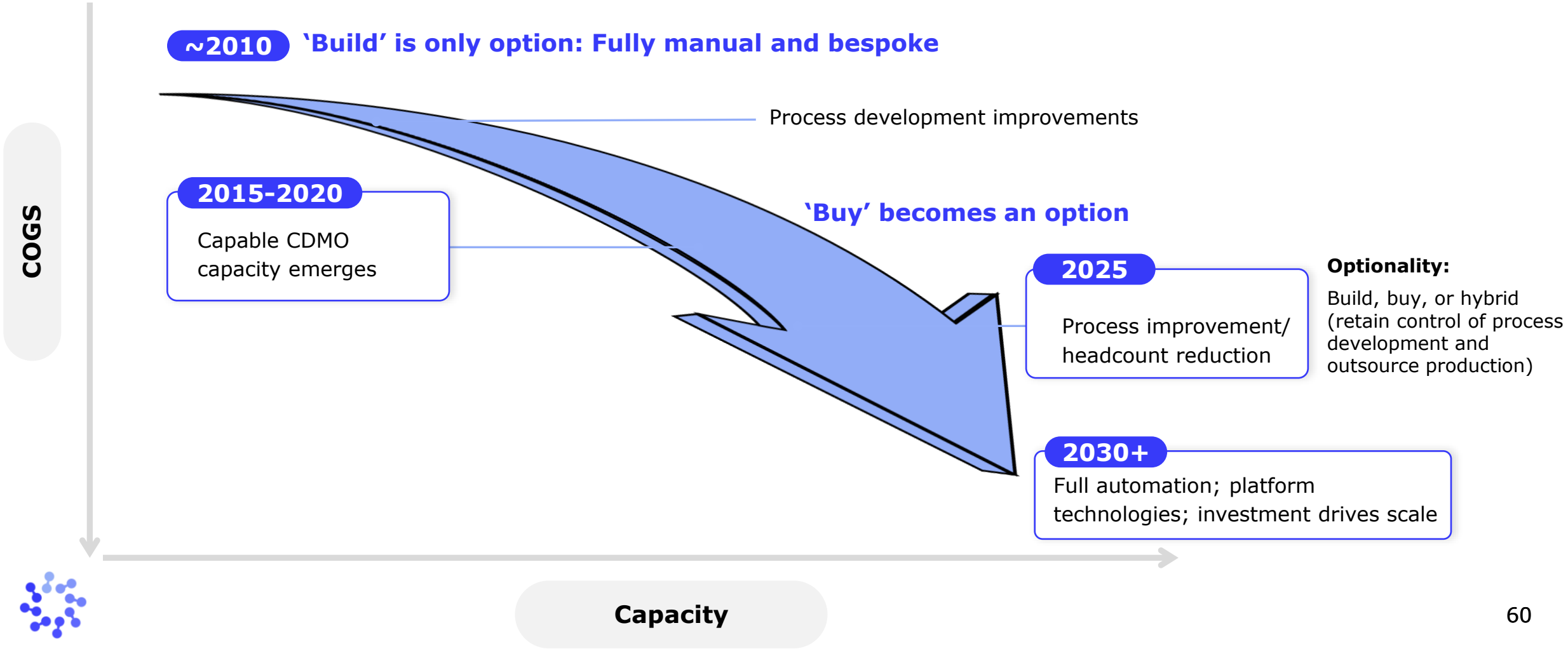
04

Provide strong value for healthcare systems (b)

An illustrative case: FDA-approved gene therapies for hemophilia A (1) and hemophilia B (2) give significant savings back to the system, according to ICER analysis

Reducing COGS

Manufacturing advances boost efficiency and optionality



Cost of goods for therapeutics, such as autologous cell therapies, poised to decline

Leerink estimates gross margins improving to ~80% by 2030, driven by:

Product Volume

CAR-T market (NHL+MM) growth

2023: $2.4Bn

↓

2030: $6.8Bn

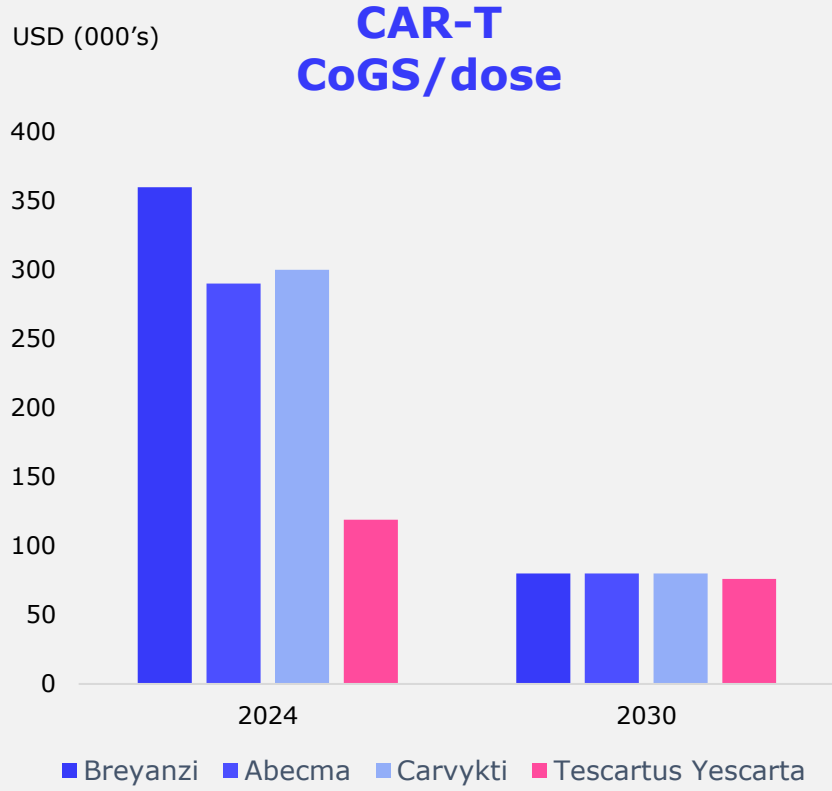
Process Improvement

	2023	2029
OOS Rate:	13%	→ 3%
MFG Time:	30d	→ 12d

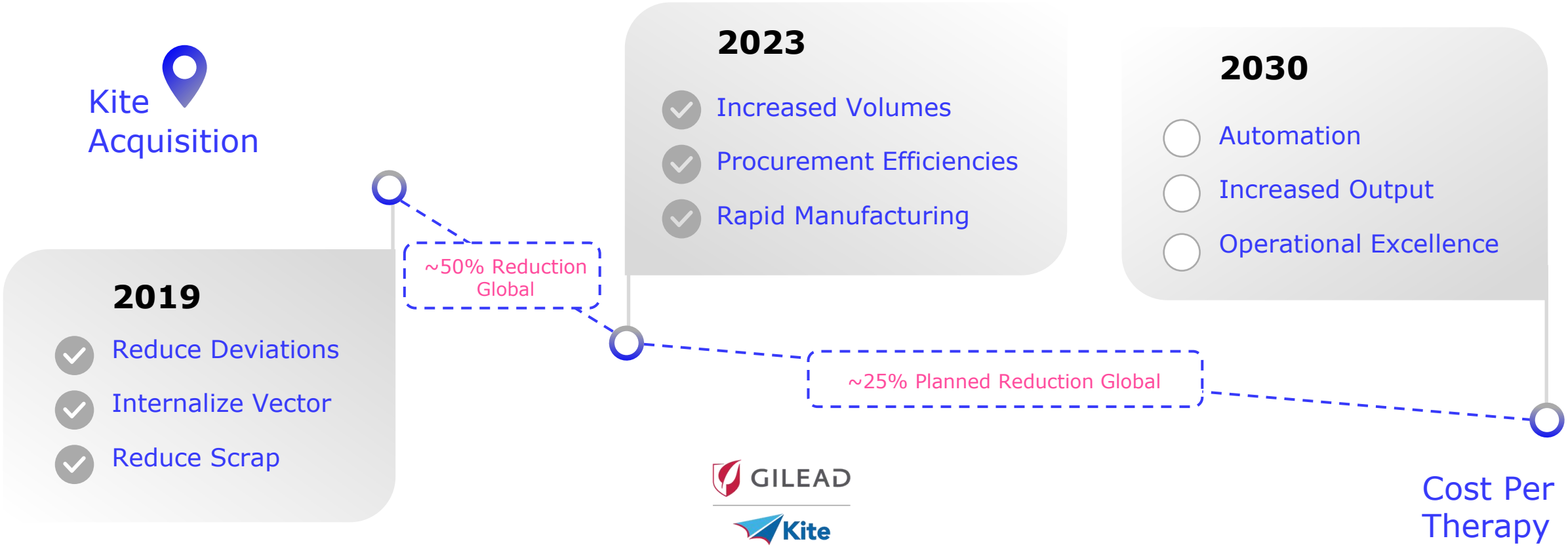
Innovation

Automation & Tools

LVV Cost Reduction



Delivering consistent cost improvements in CAR-T



1. As compared to 2023; projected estimates and subject to change. 2. Excluding accounting treatment of Arcellx profit share. Target subject to revision as portfolio expands.

Targeting biologics product gross margin of ~80% in the U. S. by 2030[2]

Transforming – and often saving – lives…

Emily Whitehead

Diagnosed with acute lymphoblastic leukemia at age 5, Emily was the first pediatric patient globally to receive a CAR-T cell therapy. Over 13 years later, she is still cancer-free.

Jimi Olaghere

After receiving a gene therapy treatment for his sickle cell disease, Jimi reports that he no longer requires regular care for his condition other than clinical follow-up.

Weston Cook

Weston was diagnosed with Spinal Muscular Atrophy type 1, which kills many patients by age 2. Weston received gene therapy, which gave him a shot at a normal life.

'KJ'

KJ was born with a rare disease that put his life at risk. Thanks to a collaborative effort between CHOP, Danaher, and many others, he became the first patient to receive a custom gene editing therapy. He recently took his first steps.

… With more breakthroughs on the way

“I don’t have to worry that I am going to wake up tomorrow and lose function… The fear is not there anymore.”

Marci McCue became the first patient to participate in a CAR-T trial clinical trial for multiple sclerosis

Thank you to our data partners