Welcome & State of Play

Advanced Therapies in 2026

Ilya Yasny, PhD

Partner, LanceBio Ventures | Head of Science, Bioptic

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Fifteen years ago there were none

Gene therapies authorised, 2011 → 2026

EUROPEAN UNION

United States 0 → **27**

China 2 → **15**

In 2011 the only approved gene therapies in the world were two in China.

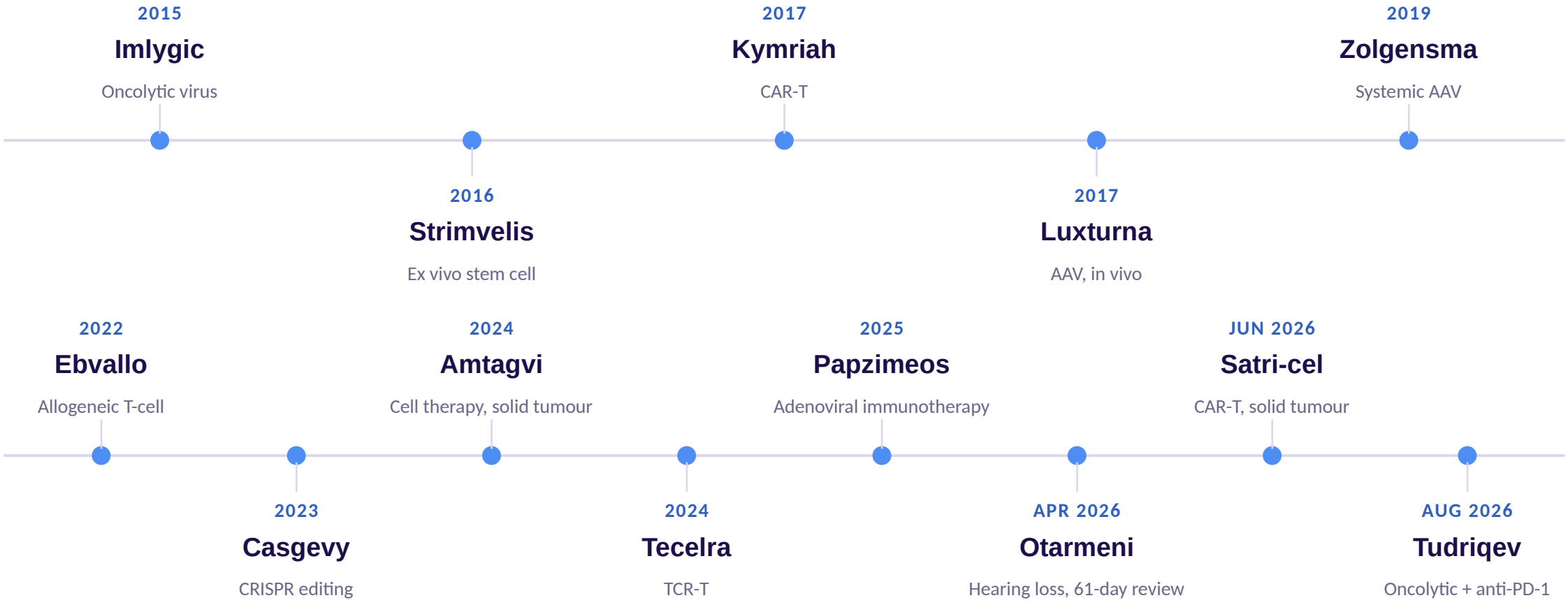
More than half of Europe's eighteen arrived in the last five years.

Seven have since been withdrawn or not renewed — Glybera, Provenge, Zalmoxis, Alofisel, Zynteglo, Skysona, Beqvez.

ASGCT–Citeline Q2 2026 approvals tables, counted once per market. Gene therapy includes genetically modified cell therapies such as CAR-T; tissue-engineered products excluded. China's two were Gendicine (2004) and Oncorine (2005).

Two modalities became a dozen

First-in-class approvals. Five firsts in the five years to 2019 — eight since 2022, three of them since April.



Still unapproved anywhere: allogeneic CAR-T, in vivo CAR-T, base-edited therapies.

Source: FDA, EMA and NMPA records. Rails show sequence, not linear time.

And the clinical wins have kept coming this year

Five readouts and filings from 2026 alone

uniQure
2 Sept 2026

BLA filed for the first gene therapy in Huntington's. 75% slowing of progression at three years vs matched external control.

Intellia
27 Apr 2026

First positive Phase 3 for in vivo CRISPR. 87% reduction in hereditary angioedema attacks, p<0.0001. 62% attack- and therapy-free.

Moderna + Merck
19 Aug 2026

First Phase 3 win for an individualised neoantigen mRNA therapy. Met both RFS and DMFS in 1,137 resected melanoma patients.

Beam
8 Sept 2026

First in vivo correction of a disease-causing mutation, now shown durable. Z-AAT down 84%; protein above the protective threshold out to 18 months.

CRISPR Therapeutics
28 Aug 2026

ANGPTL3 base editing, published in NEJM. LDL −53%, triglycerides −48% at one year from a single infusion.

Clinical success does not mean approval. Approval does not mean commercial success.

In advanced therapies that gap is wider than anywhere else in pharma.

Thirty years of science, nine billion dollars of revenue

Worldwide net product revenue, FY2025, bottom-up from company reports

CAR-T

$6.0bn

Seven approved products. Carvykti $1.90bn, Yescarta $1.50bn, Breyanzi $1.36bn, Abecma $0.43bn, Kymriah $0.38bn, Tecartus $0.34bn, Aucatzyl $0.07bn

Gene therapy, non-CAR-T

$3.1bn

Zolgensma $1.2bn, Elevidys $0.9bn, Vyjuvek $0.4bn, Luxturna, Hemgenix, Roctavian, Casgevy — the rest below $0.2bn each

GROWING			SHRINKING		
Carvykti	$1.90bn	+97%	Yescarta	$1.50bn	−5%
Breyanzi	$1.36bn	+82%	Kymriah	$0.38bn	−14%
Casgevy, H1 2026	$119m	+151%	Tecartus	$0.34bn	−15%

Two CAR-T products out-sell thirty years of everything else.

Legend reached Carvykti profitability; Aucatzyl, the only European-owned CAR-T, guides to $120–135m this year.

Source: Company FY2025 and Q2 2026 reports; speaker's aggregation.

The failures were structural, not scientific

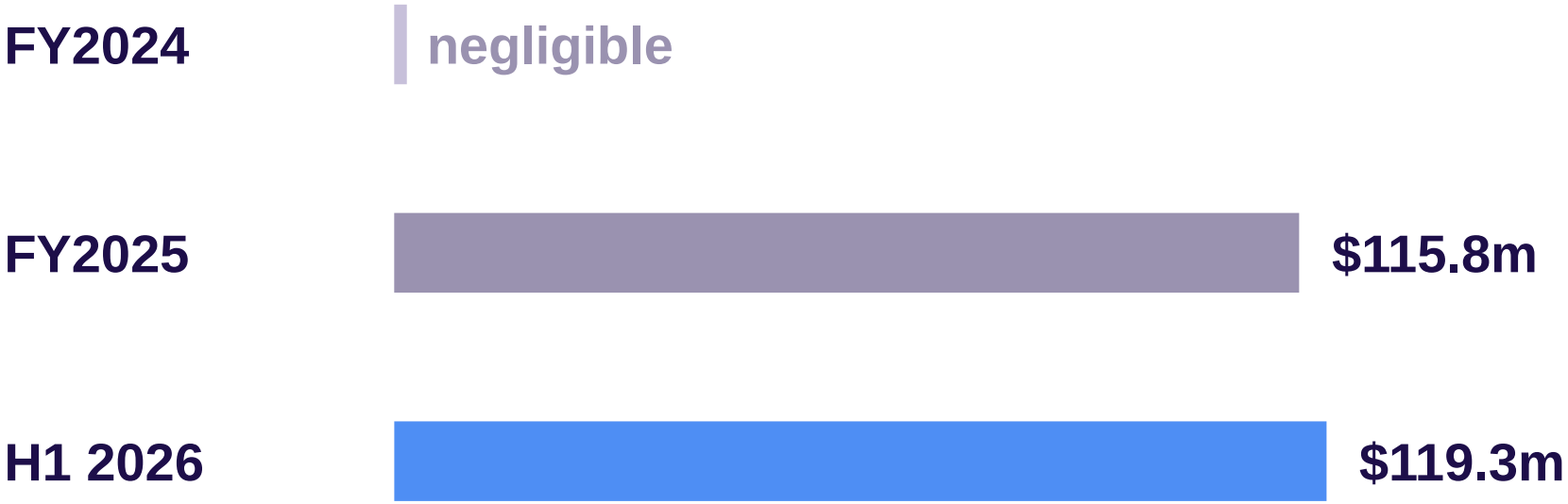
Sangamo	Chapter 11, June 2026. Estate sold for $164m.	Pfizer walked away six months after positive Phase 3 data.
bluebird bio	Taken private for ~$29m.	Once valued near $10bn, with three approved products.
Elevidys	Two deaths, boxed warning, label narrowed.	Sarepta cut 36% of its workforce.
Roctavian · Beqvez	Withdrawn, and discontinued.	No buyer found; no sales ever disclosed.

In almost every case the biology held and the business did not.

Source: SEC filings; company announcements; EMA CAT June 2026.

Casgevy shows how long the operating model takes to build

Two flat years, then an inflection once centres, labels and reimbursement existed



+151%

Q2 2026 revenue, year on year.

39 countries approved. German reimbursement, May 2026.

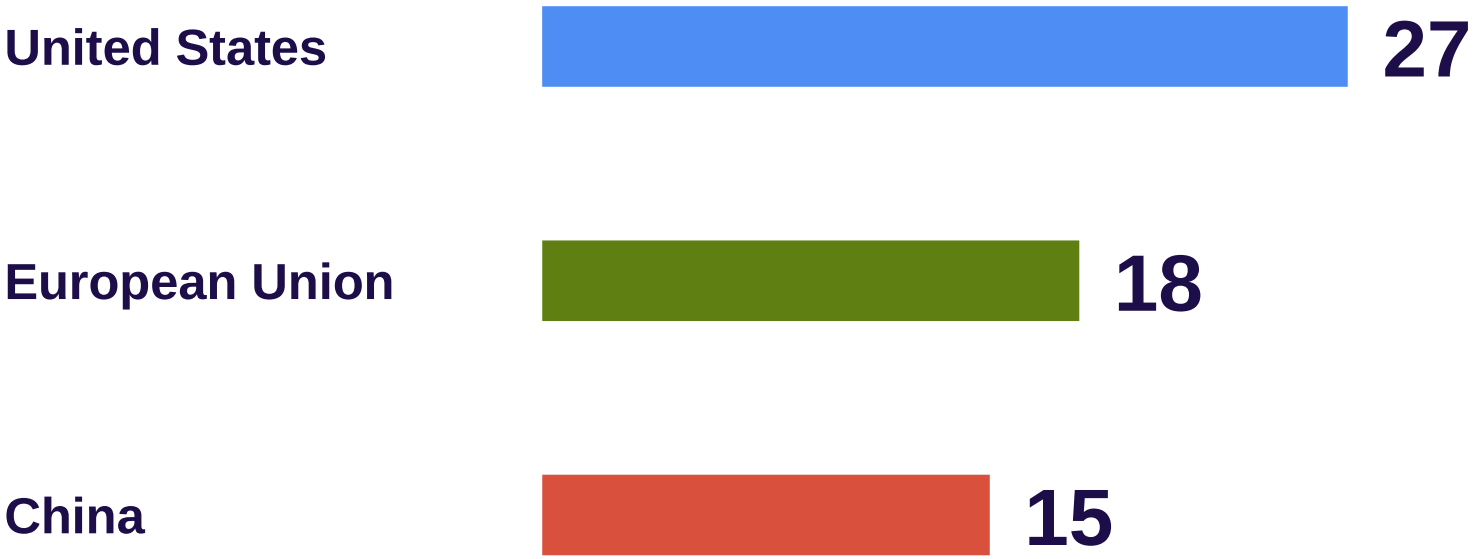
The delay was never the editing. It was apheresis, conditioning, centres and reimbursement.

Vertex and CRISPR stopped disclosing patients collected, patients infused and centre counts this quarter.

Source: Vertex Q2 2026 and FY2025 results.

Where advanced therapies are approved has changed

Of the 46 approved gene therapies worldwide, by market



LAST QUARTER

5

gene therapies approved worldwide in Q2 2026

China	2
United States	1
Japan	1
South Korea	1
Europe	**0**

Europe's eighteen is two-thirds of the American number. It is not out of the game — but it approved nothing at all last quarter.

Ten of China's fifteen are approved in China and nowhere else.

Source: Speaker's count of the approvals table in ASGCT–Citeline Gene, Cell & RNA Therapy Landscape Report, Q2 2026. Products are counted once per market.

China has bought speed. The question is what it costs.

WHAT SPEED DELIVERED

Satri-cel, June 2026

The world's first CAR-T approved for a solid tumour. In China, not here.

990 vs 916

Cell and gene therapy trials in 2025 — Asia-Pacific ahead of North America for the first time.

WHAT IT COST

Three deaths, disclosed in 2026

In investigator-initiated trials. None reported at the time. One ethics board judged a death treatment-related.

A diligence question

Was it registered? Was toxicology reviewed? Was escalation governed? Were events disclosed?

Three cases are not a verdict on a country. They are a permanent change to a diligence checklist.

Source: CARsgen; ASGCT–Citeline; congressional correspondence to FDA, 21 August 2026.

One week in August contained both halves of the field

27 AUGUST

First CAR-T trial in rheumatoid arthritis

- Six refractory patients
- Three of six in remission, at ACR70
- Grade 1–2 CRS only; no ICANS
- Published in Nature Medicine

31 AUGUST

Novartis halts eight autoimmune CAR-T trials

- Three deaths from IEC-HS
- Lupus, scleroderma, myositis, RA, gMG, MS
- BMS paused its own within days

Same modality. Same disease area. Four days apart.

One hypothesis for the deaths: faster manufacturing may drive greater expansion in the patient, and with it the toxicity.

Sell-side, hedged by its own authors. Mechanistically plausible; causally unestablished. Neither the company nor the regulator has endorsed it.

Source: Nature Medicine, 27 Aug 2026 (COMPARE, NCT06475495); Novartis and BMS statements, 31 Aug–1 Sept 2026.

The hard problems are no longer only biological

Patients	Manufacture	Reimburse	Regulate	AI
Find and reach the right ones	Fast, consistent, at what cost	Who pays, where, on what evidence	An authority in transition	Where in the chain it actually pays

More generous on paper than ever, and less predictable

There is still no Senate-confirmed leadership over any of it

FDA Commissioner

Acting since May. Heidi Overton nominated 19 Aug; no hearing scheduled.

CBER Director

Acting. Third this year.

Office of Therapeutic Products

Vacant since June.

Jun 2025	CAR-T REMS eliminated
Jul 2025	Rejection letters published
Sep 2025	Rare Disease Evidence Principles
Feb 2026	Plausible mechanism pathway
Apr 2026	Otarmeni approved in 61 days

You can now read why a competitor was rejected. In full, within days.

Tudriqev: two rejections, an advisory committee, three resubmissions — then approval in August.

Acting is not hostile: policy has never been more accommodating. The variable is whether last year's answer still holds.

Source: FDA leadership pages, press announcements and Federal Register, to September 2026.

The public window has been shut for two years

0

IPOs in advanced therapies in the last 24 months.

So companies are reaching the public market another way.
Three of the eight acquisitions last quarter were reverse mergers:
Obsidian, Remix and Serapha Bio.

MEANWHILE, SEED AND SERIES A INTO GENE AND CELL THERAPY

$250m

Q1 2026 · 10 companies

$414m

Q2 2026 · 16 companies

+65%

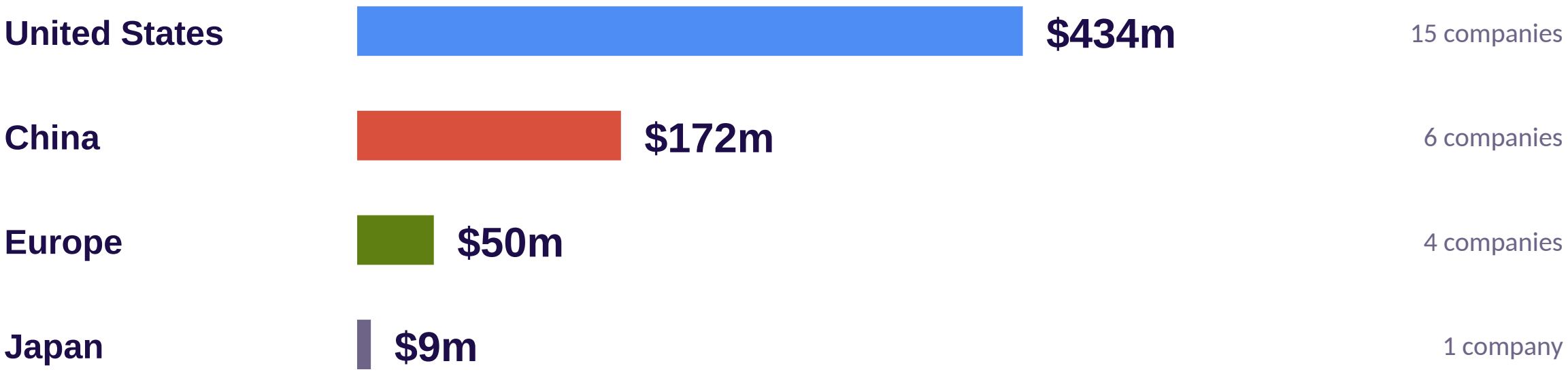
quarter on quarter

Early-stage dollars have recovered. The exit has not.

Source: ASGCT–Citeline Q1 and Q2 2026, gene and cell therapy financings only; BioPharma Dive IPO tracking. Reverse mergers are not IPOs.

And Europe is not where the new companies are being funded

Seed and Series A into gene and cell therapy companies, 2026 year to date



Europe took 8% of the world's start-up capital — four companies in eight months.

ORCHARD THERAPEUTICS

UCL and Great Ormond Street science. Strimvelis, Europe's first ex vivo stem-cell gene therapy. Lenmeldy at $4.25m, the most expensive drug in the world. Sold to Kyowa Kirin for $478m — and Strimvelis handed to a charity, because no company could sustain it.

Source: Speaker's analysis of the seed and Series A financings listed in ASGCT–Citeline Q1 and Q2 2026, RNA-only companies excluded; disclosed values only, $665m across 26 companies.

Meanwhile pharma is buying, and the pipeline keeps growing

Announced acquisitions in gene and cell therapy, 2026 year to date

Feb	**Gilead / Arcellx** BCMA CAR-T	**$7.8bn**
Apr	**Lilly / Kelonia** In vivo CAR-T	**$7.0bn**
Feb	**Lilly / Orna** In vivo CAR, circRNA	**$2.4bn**
Apr	**UCB / Neurona** Allogeneic cell therapy	**$1.15bn**

$18.4bn across four acquisitions, January to August 2026.

Half of it is in vivo cell engineering — a modality with no approved product anywhere in the world.

GENE THERAPY PIPELINE, Q2 2025 → Q2 2026

Phase I	361 →	**427**
Phase II	330 →	**387**
Phase III	45 →	**59**
Total programmes	2,155 →	**2,217**

Phase III up 31% in a year.

Innovation is not the constraint. It grew through the worst quarter this field has had.

Source: Company announcements; ASGCT–Citeline Landscape Report, Q2 2026. RNA-only acquisitions excluded.

Where AI is most likely to pay is late, not early

85–90%

of drugs still fail in the clinic. The bottleneck was never the speed of inventing molecules — and discovery has the longest validation loop we have. A target chosen today is judged in eight years.

WHERE THE FEEDBACK LOOP IS MEASURED IN WEEKS

Asset scouting

Find the asset before the market prices it

Due diligence

Interrogate a dossier in hours, not weeks

Competitive intelligence

Track every rival in every language, continuously

Launch excellence

Find the patients, the centres and the payers

Agents do this faster and more accurately than we can — over messy, multilingual, unstructured data. And they end up informing decisions worth billions of dollars.

If the constraint has moved downstream, so should the tooling.

Full disclosure: I am head of science at Bioptic, which builds AI agents for exactly these four things.

The only certain thing in our industry is uncertainty.

We cannot cancel it. We can learn to navigate it.

Everyone in this room is better equipped to do that
than any group I know.

Let us use these three days for what they are for:
getting medicines to the patients who need them.