Bio Biotechnology Innovation Organization

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The State of Emerging Biotech Companies: Investment, Deal, and Pipeline Trends

June 17, 2025

Biotechnology Innovation Organization

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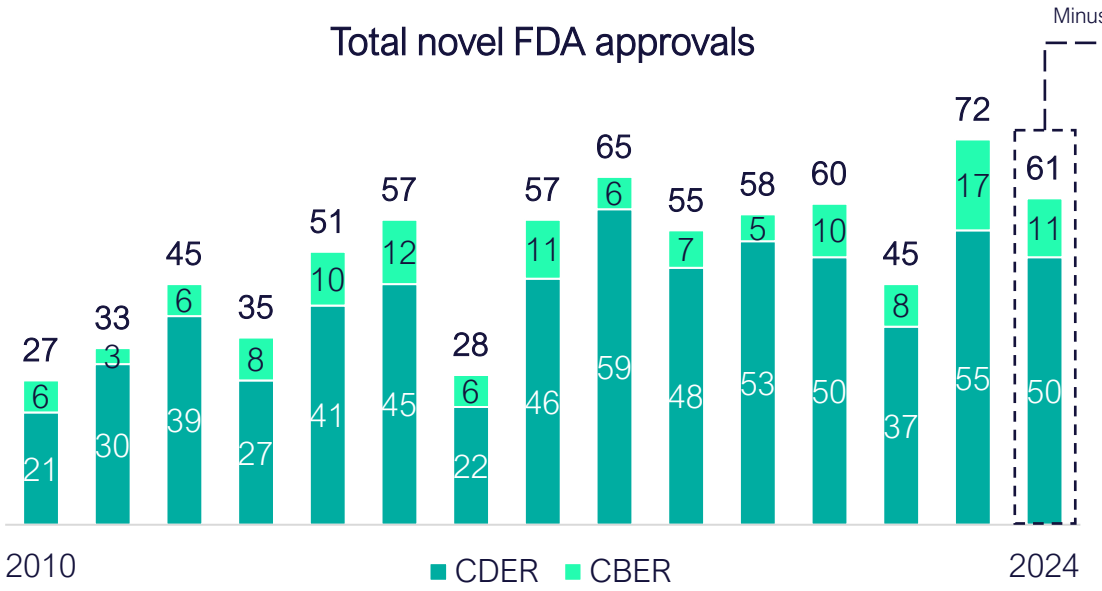
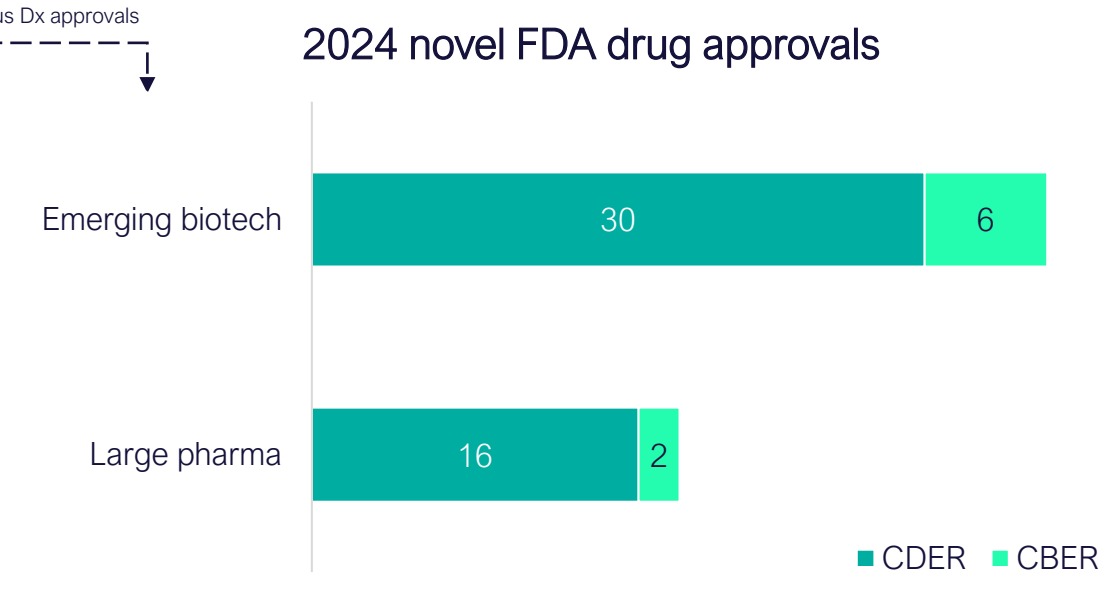
New drug approvals

Biotechs behind two thirds of novel approvals in a slightly down year at FDA





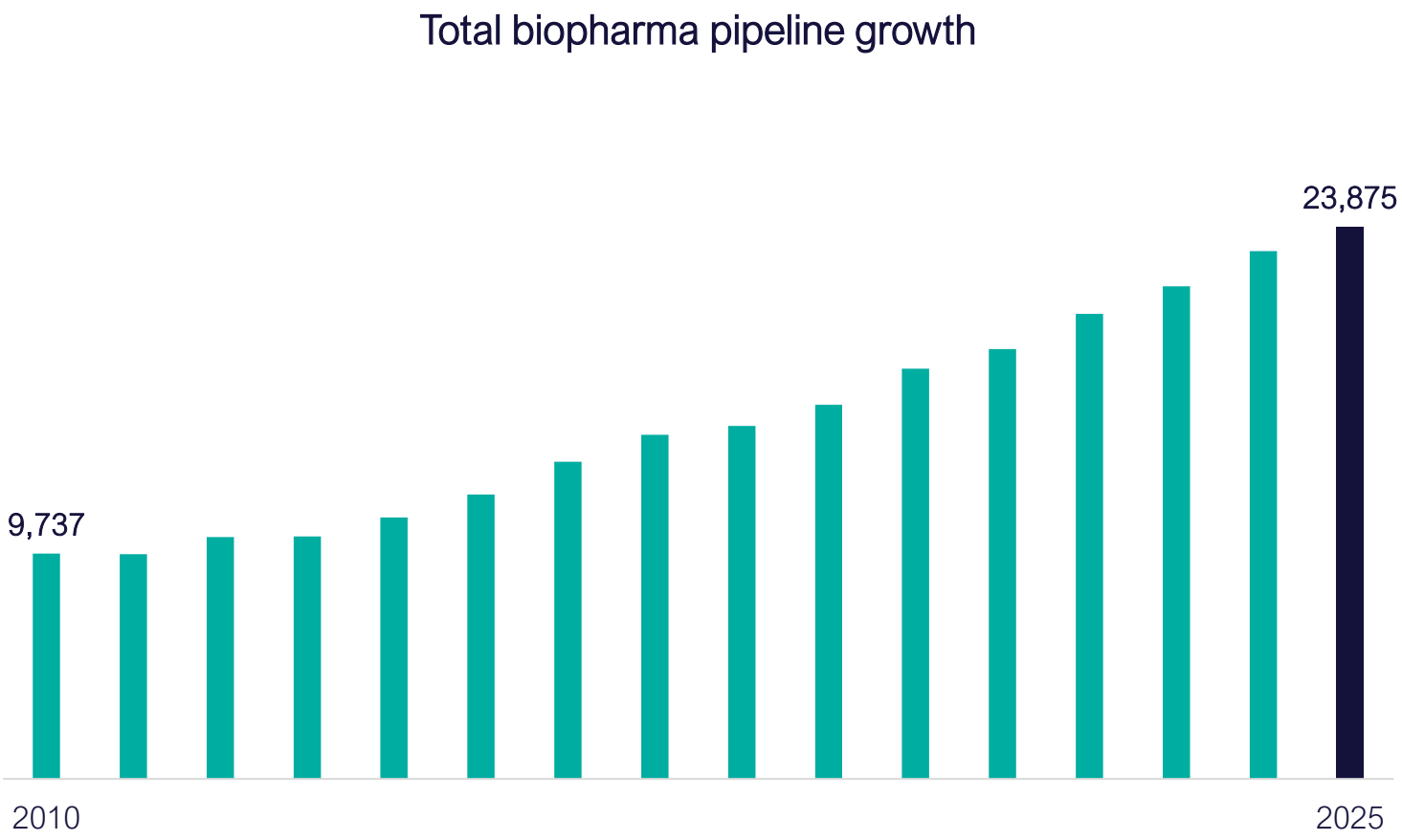
- Novel approvals mirror R&D pipeline with long-term growth trend
- 2024 slower than record 2023, but aligned with recent average of ~60 new drugs per year
- Fluctuations at both CDER and CBER
- 2025 YTD stands at 21 (15 + 6)

- Emerging biotechs behind 36 out of 54 novel drug approvals at FDA last year
- 24 out of 54 (44%) were BLAs
- 32 approvals for rare diseases (59%)
- 10 accelerated approvals (19%)

Source: Citeline, The Pink Sheet

Global biopharma R&D pipeline

Total size has grown by 4.6%; 23,875 drugs under development



Long-term growth in R&D pipeline continues

- Overall growth of 145% since 2010
- 2024 expansion rate (4.6%) subsided slightly, trailing 5-year average of 6.7%

Rate of new asset discovery slows

- 4.5k new candidates identified, balancing attrition of 3.5k exiting the R&D pipeline
- Prior years have consistently seen 5-6k new drugs added to our databases

New drug statistics

- 39% of new assets were anticancer
- 20% for rare diseases
- 14% neurologicals

Source: Citeline, Pharmaprojects

Drug development universe

Rapid expansion of new drug developers; YOY growth of 11.4% to 6,823 companies

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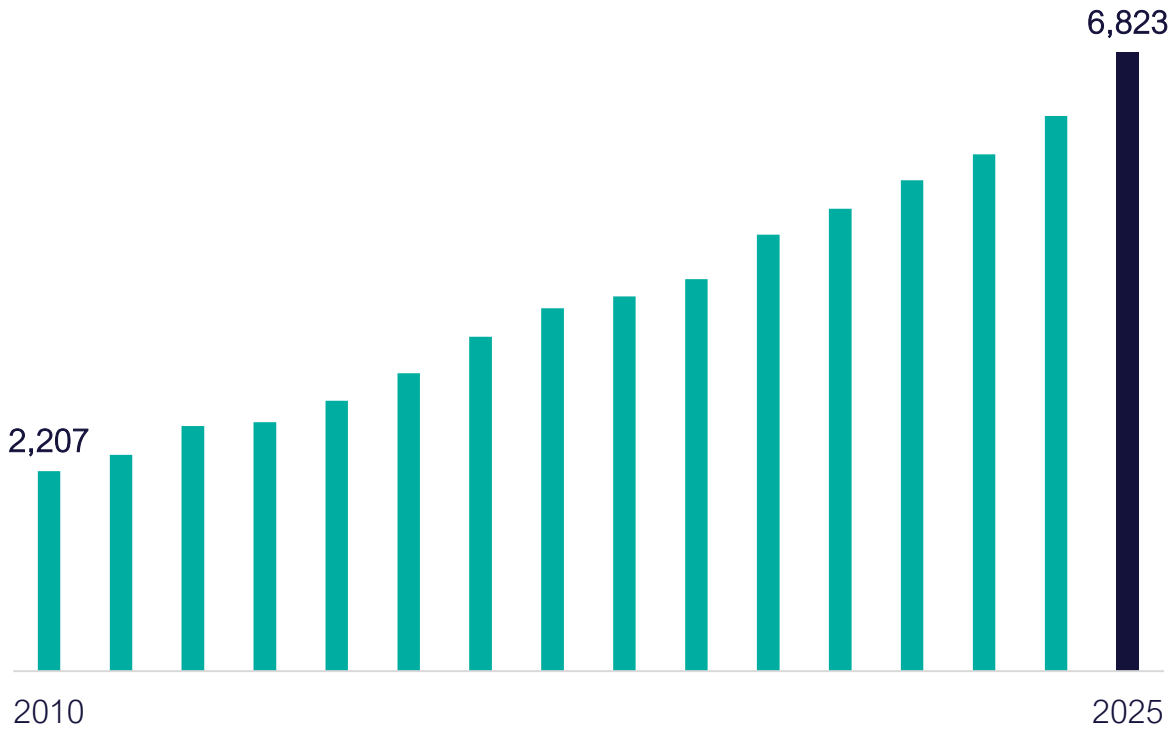
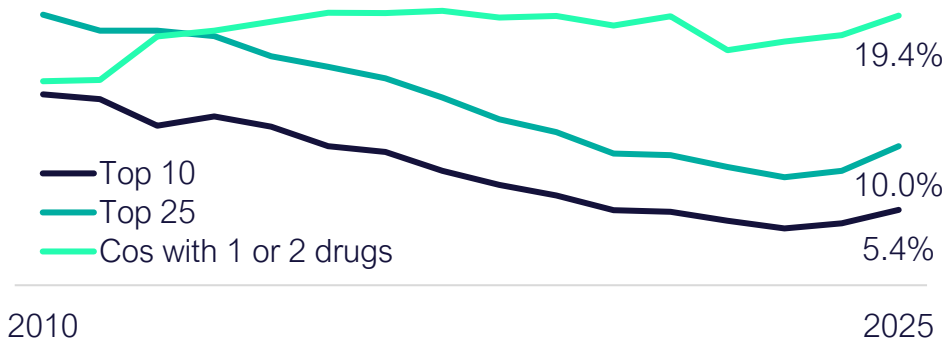
Biopharma pipeline developer universe



Share of pipeline by company size



Pipeline growth driven by new and emerging biotechs

- Almost 7k companies in the drug developer universe
- Rate of growth strongly exceeds pipeline count as R&D is increasingly democratized

Large pharma's share of the pipeline is diminishing

- Overall share of pipeline has steadily dropped
- Top 10 retains just 5% of programs; top 25 just 10%

Source: Citeline, Pharmaprojects

Current Clinical Pipeline

7,557 last year → 7688 Drug Programs in 2025 (+1.7%)

Disease	Total	%
Oncology	3495	45%
Neurology	787	10%
Infectious Disease	643	8%
Other	519	7%
Immunology	472	6%
Endocrine	308	4%
Metabolic	288	4%
Ophthalmology	284	4%
Cardiovascular	216	3%
Psychiatry	197	3%
Respiratory	181	2%
Gastrointestinal	188	2%
Hematology	110	1%
Total	**7688**	**NA**
Percent	**100%**	**100%**

"Other" Category
Allergy
Dermotology
Obstetric/Gynecology
Renal
Rheumatology (non autoimmune)
Urology

- *Company drug-indication clinical programs intended to eventually reach FDA approval*
- *Data as of April 2025*

Source: Biomedtracker, BIO Industry Analysis, 2025

Current Clinical Pipeline

84% of all Programs are Early Clinical-Stage

Disease	Total	%	P1	P2	P3	NDA/ BLA
Oncology	3495	45%	1432	1745	297	21
Neurology	787	10%	274	356	143	14
Infectious Disease	643	8%	239	271	121	12
Other	519	7%	146	261	98	14
Immunology	472	6%	155	208	96	13
Endocrine	308	4%	92	162	46	8
Metabolic	288	4%	83	137	56	12
Ophthalmology	284	4%	46	170	63	5
Cardiovascular	216	3%	51	100	52	13
Psychiatry	197	3%	64	86	40	7
Respiratory	181	2%	58	94	24	5
Gastrointestinal	188	2%	63	101	23	1
Hematology	110	1%	21	53	30	6
Total	**7688**	**NA**	**2724**	**3744**	**1089**	**131**
Percent	**100%**	**100%**	**35%**	**49%**	**14%**	**2%**

- *Company drug-indication clinical programs intended to eventually reach FDA approval*
- *Data as of April 2025*

Source: Biomedtracker, BIO Industry Analysis, 2025

Current Clinical Pipeline

15% of Clinical Programs are for Rare Disease

Disease	Total	%	% Rare Disease
Oncology	3495	45%	40%
Neurology	787	10%	28%
Infectious Disease	643	8%	16%
Other	519	7%	18%
Immunology	472	6%	61%
Endocrine	308	4%	14%
Metabolic	288	4%	67%
Ophthalmology	284	4%	22%
Cardiovascular	216	3%	25%
Psychiatry	197	3%	2%
Respiratory	181	2%	56%
Gastrointestinal	188	2%	20%
Hematology	110	1%	57%
Total	**7688**	**NA**	**NA**
Percent	**100%**	**100%**	**15%**

- *Company drug-indication clinical programs intended to eventually reach FDA approval*
- *Data as of April 2025*

Source: Biomedtracker, BIO Industry Analysis, 2025

Current Clinical Pipeline

Emerging Companies account for 55% of Programs

Disease	Total	%	% Small Company
Oncology	3495	45%	62%
Neurology	787	10%	54%
Infectious Disease	643	8%	48%
Other	519	7%	46%
Immunology	472	6%	44%
Endocrine	308	4%	46%
Metabolic	288	4%	54%
Ophthalmology	284	4%	53%
Cardiovascular	216	3%	41%
Psychiatry	197	3%	53%
Respiratory	181	2%	49%
Gastrointestinal	188	2%	49%
Hematology	110	1%	52%
Total	**7688**	**NA**	**NA**
Percent	**100%**	**100%**	**55%**

- *Company drug-indication clinical programs intended to eventually reach FDA approval*
- *Data as of April 2025*

Source: Biomedtracker, BIO Industry Analysis, 2025

Current Clinical Pipeline

57% Unlicensed – Down from 69% in 2024

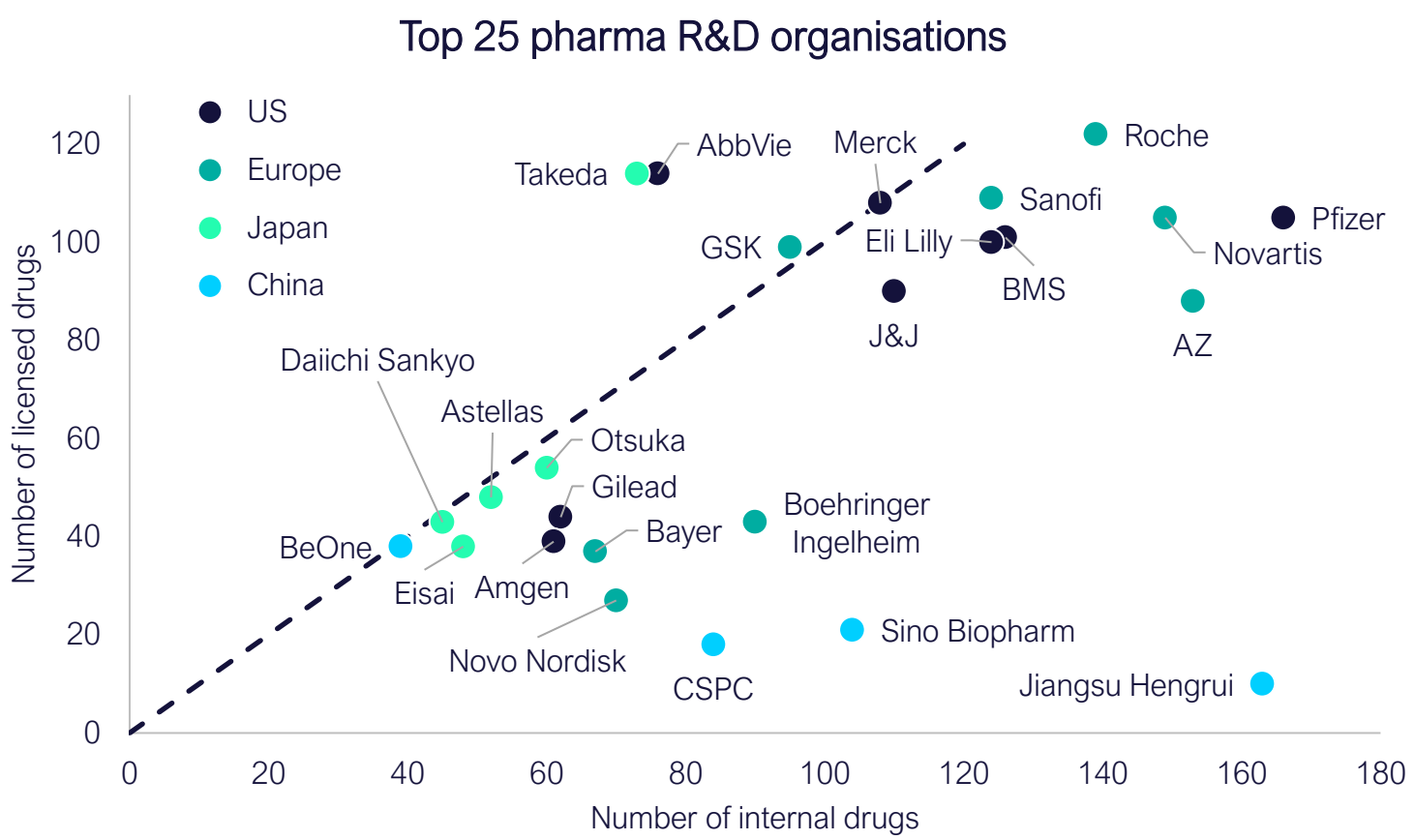
Disease	Total	%	% Small Company	% Licensed
Oncology	3495	45%	62%	46%
Neurology	787	10%	54%	37%
Infectious Disease	643	8%	48%	42%
Other	519	7%	46%	41%
Immunology	472	6%	44%	45%
Endocrine	308	4%	46%	37%
Metabolic	288	4%	54%	44%
Ophthalmology	284	4%	53%	40%
Cardiovascular	216	3%	41%	41%
Psychiatry	197	3%	53%	49%
Respiratory	181	2%	49%	40%
Gastrointestinal	188	2%	49%	48%
Hematology	110	1%	52%	38%
Total	**7688**	**NA**	**NA**	**NA**
Percent	**100%**	**100%**	**55%**	**43%**

- *Company drug-indication clinical programs intended to eventually reach FDA approval*
- *Data as of April 2025*

Source: Biomedtracker, BIO Industry Analysis, 2025

Large pharma portfolio origin

An approximate 50:50 balance between internal and external innovation remains the goal



Dotted line shows 50:50 portfolio of internal and in-licensed pipeline candidates

Large pharma generally sources as many drugs externally as internal candidates

- Majority of the top 25 biggest pipelines are split in or close to 50:50
- Slight variation according to strategic focus and internal capabilities as a small cohort favor outsourcing

The largest pipelines require the biggest internal R&D support

- Pfizer, Novartis and Roche are 2025's top 3 and trend the furthest to the right

China biopharma model is unique

- Emphasis on internal R&D and out-licensing for international markets
- BeOne (former BeiGene) is the exception with a global footprint and track record of in-licensing assets

Source: Citeline, Pharmaprojects

Pharma R&D spend

Approx. $300bn budget – driven by large pharma – and growing, albeit at reduced rate

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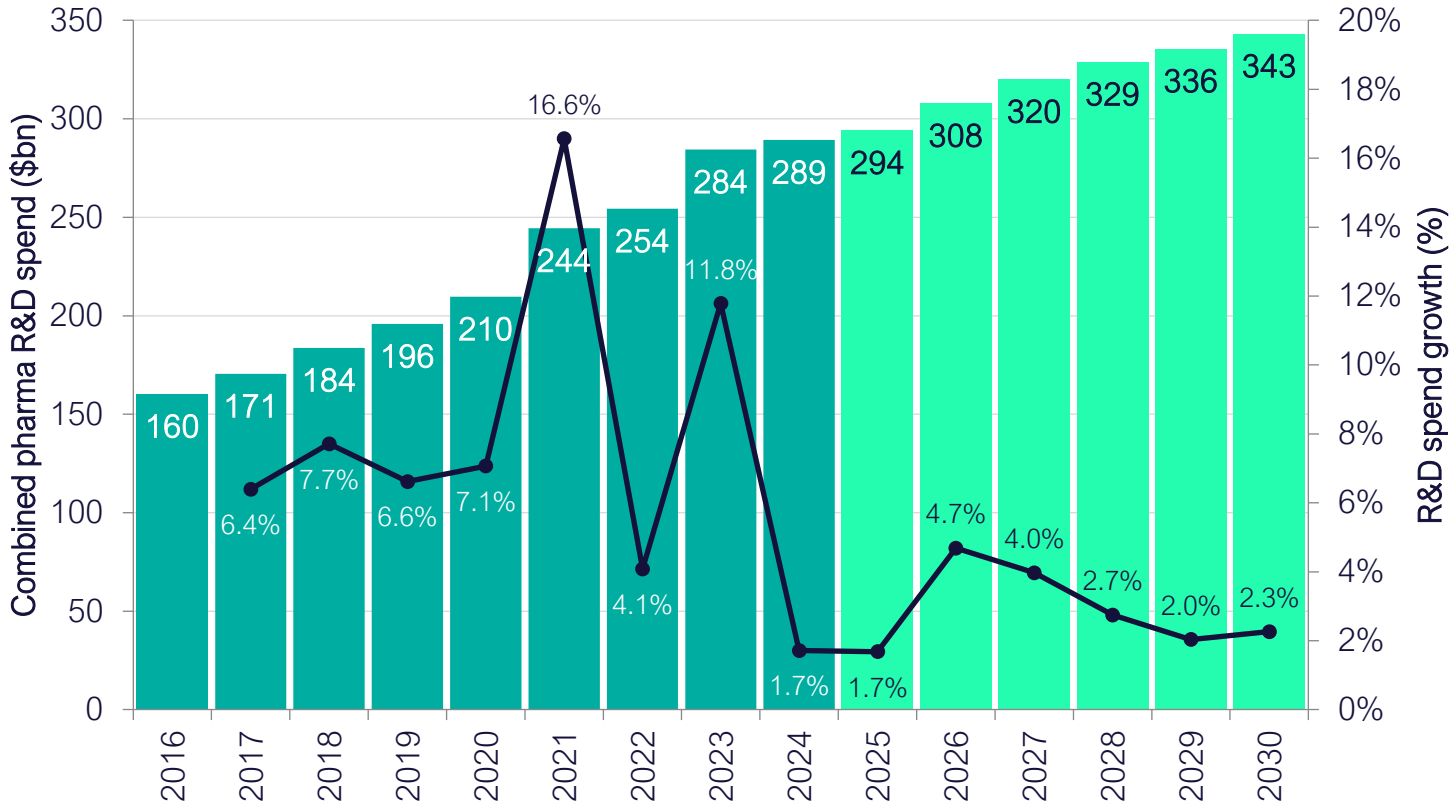
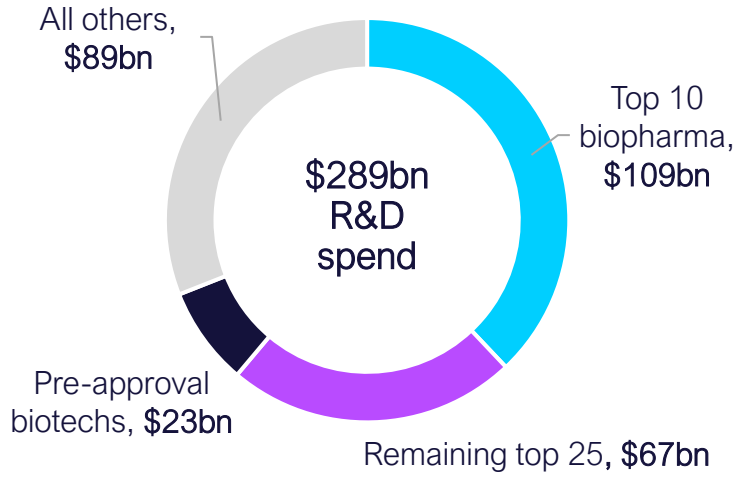
Worldwide total pharmaceutical R&D Spend



R&D spend by cohort



Consensus suggests that year-on-year R&D budget growth will slow

- 8% CAGR (‘16–24) forecast to drop to 3% (‘24–30) as R&D budgets pressured and rationalized across many companies
- Overall dynamic driven by large pharma, which dominates R&D spending (see above)
- Biotech budgets should outperform; hinges on availability of financing

Source: Evaluate Pharma

R&D engines

Tripolar innovation ecosystem as China's emergence squeezes Europe into a distant third

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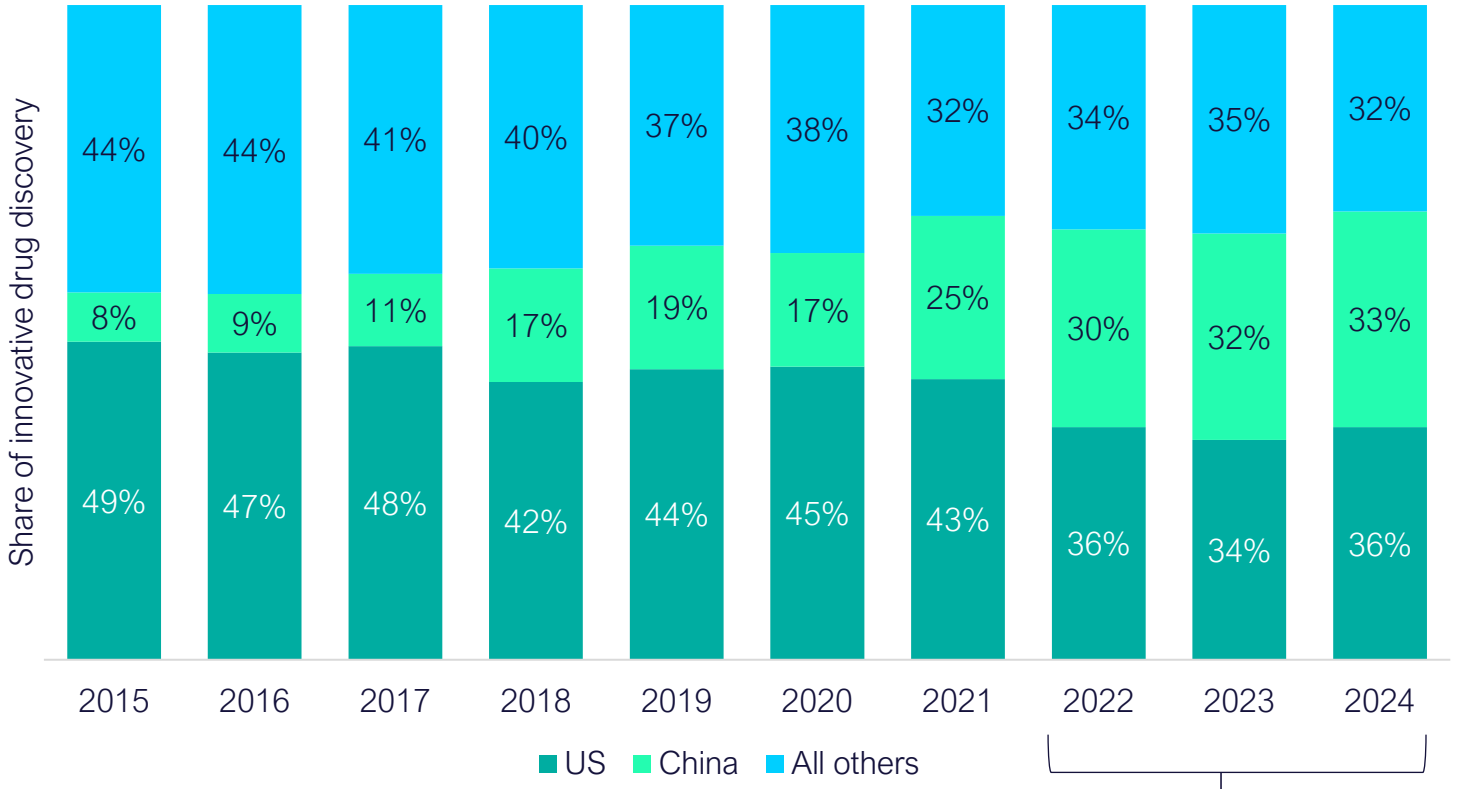
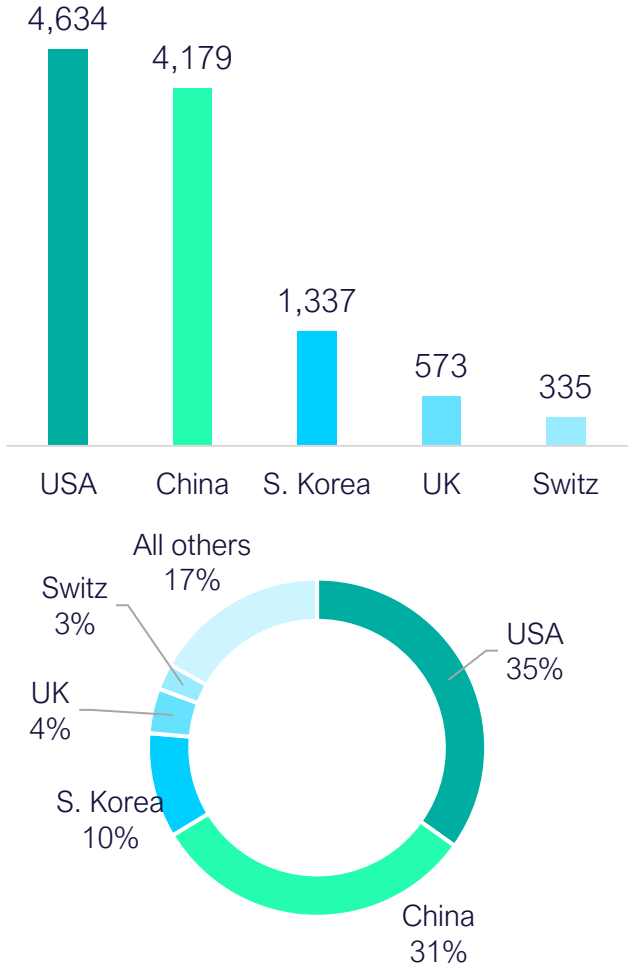
New-to-pipeline drugs by corporate HQ location



Top 5 R&D engines



New-to-pipeline drugs characterized as innovative NCEs or NBEs beginning preclinical or clinical-stage development in given year

Source: Citeline, Pharmaprojects

Measure of innovation

But US and Europe still lead on translation of targets into first-in-class drug candidates

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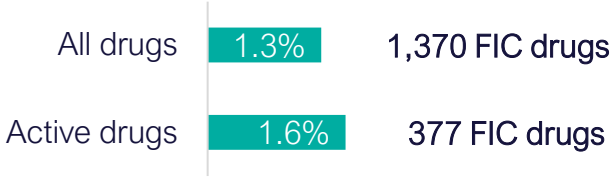
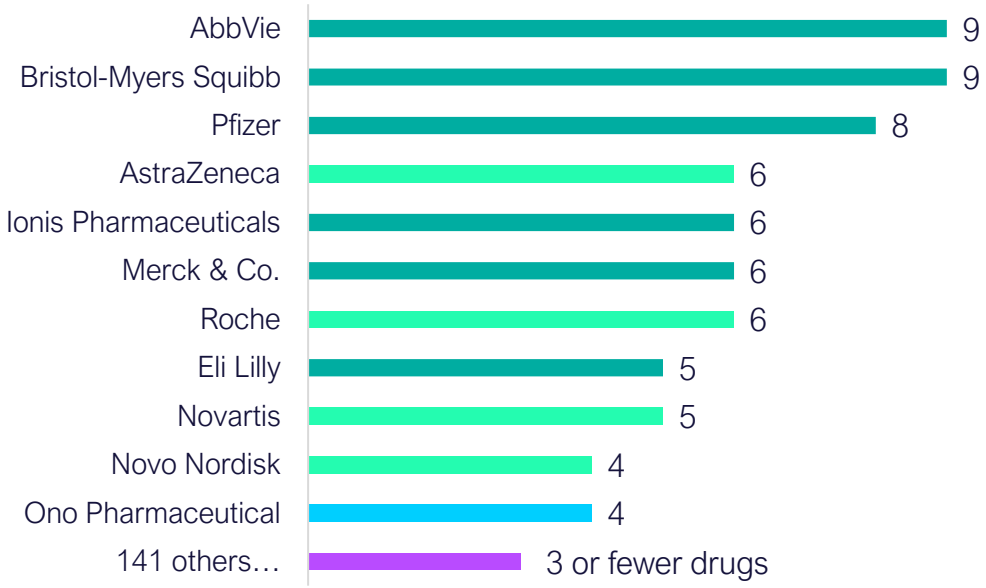
How many drugs are first-in-class against their target?



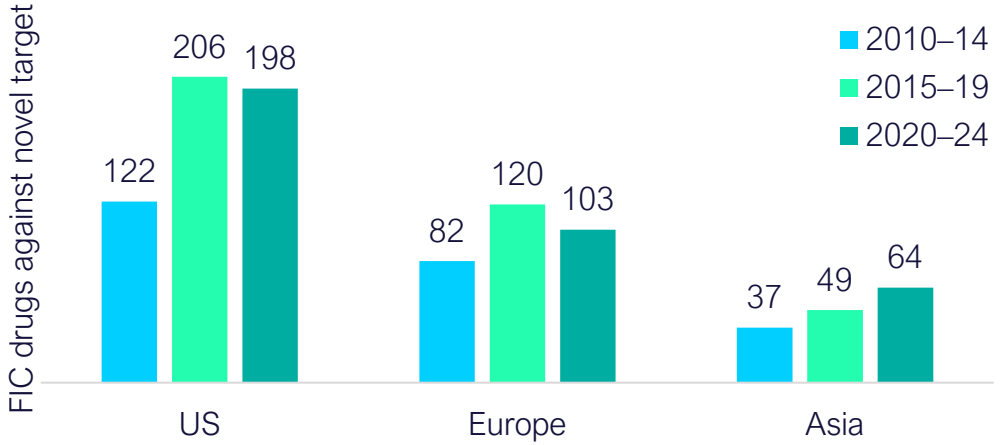
Drugs added to Citeline's database that required a **new target field**

- A measure of first-in-class-*ness* and degree of innovation
- Differentiation, but no guarantee of clinical success
- US ~50% share of all active FIC assets; Europe ~30%; China just 3%

Who is really innovating scientifically?



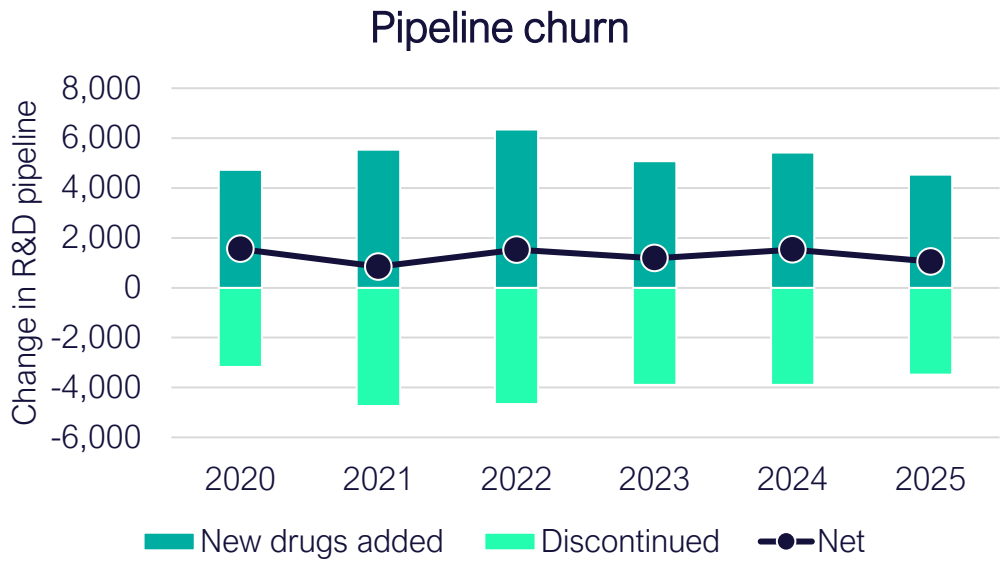
How is the distribution of innovation changing?



Source: Citeline, Pharmaprojects

Pipeline churn

Change in research priorities as rare diseases, cardiovascular rise in prominence



Slight drop in churn keeps pipeline growth afloat

- New drug creation at lowest level so far this decade

Churn allows reprioritization of therapeutic area focus

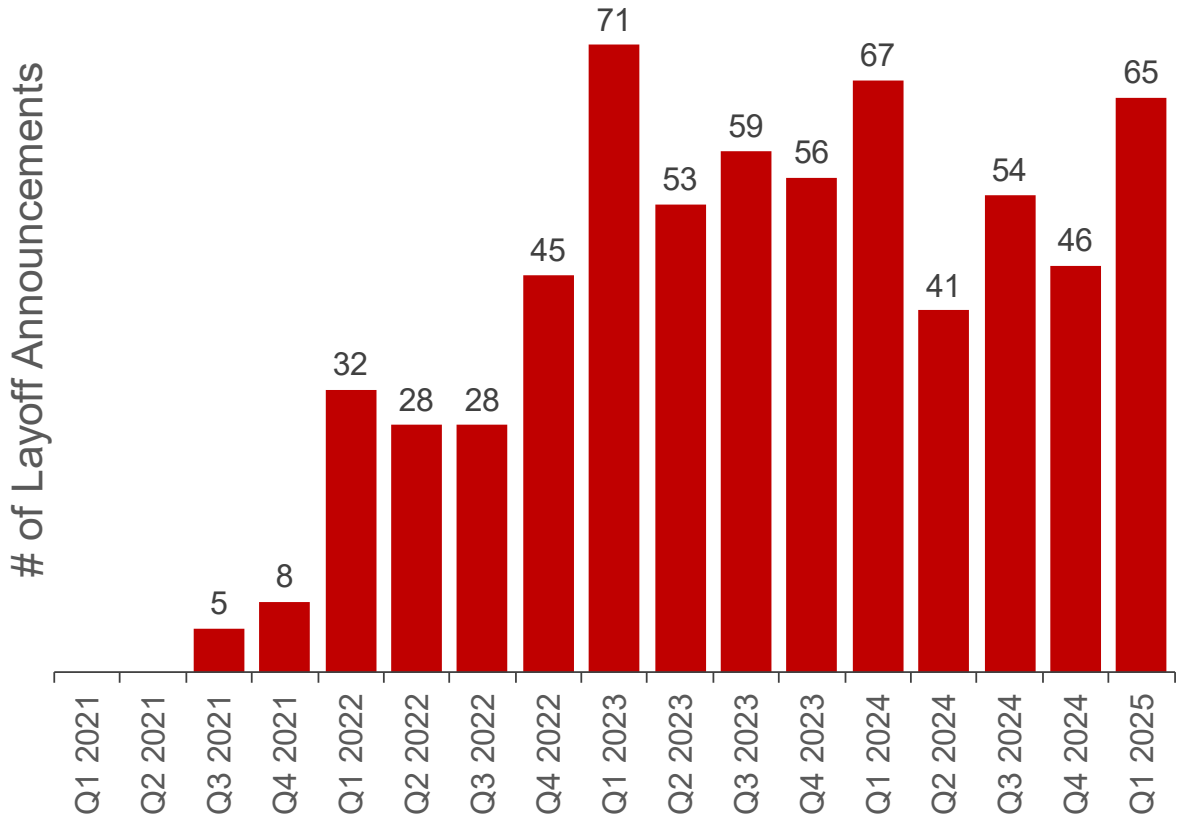
- Rare disease and cardiovascular are fast growing, while other TAs also outpace the 4.6% average
- Oncology grows at below-average rate for first time

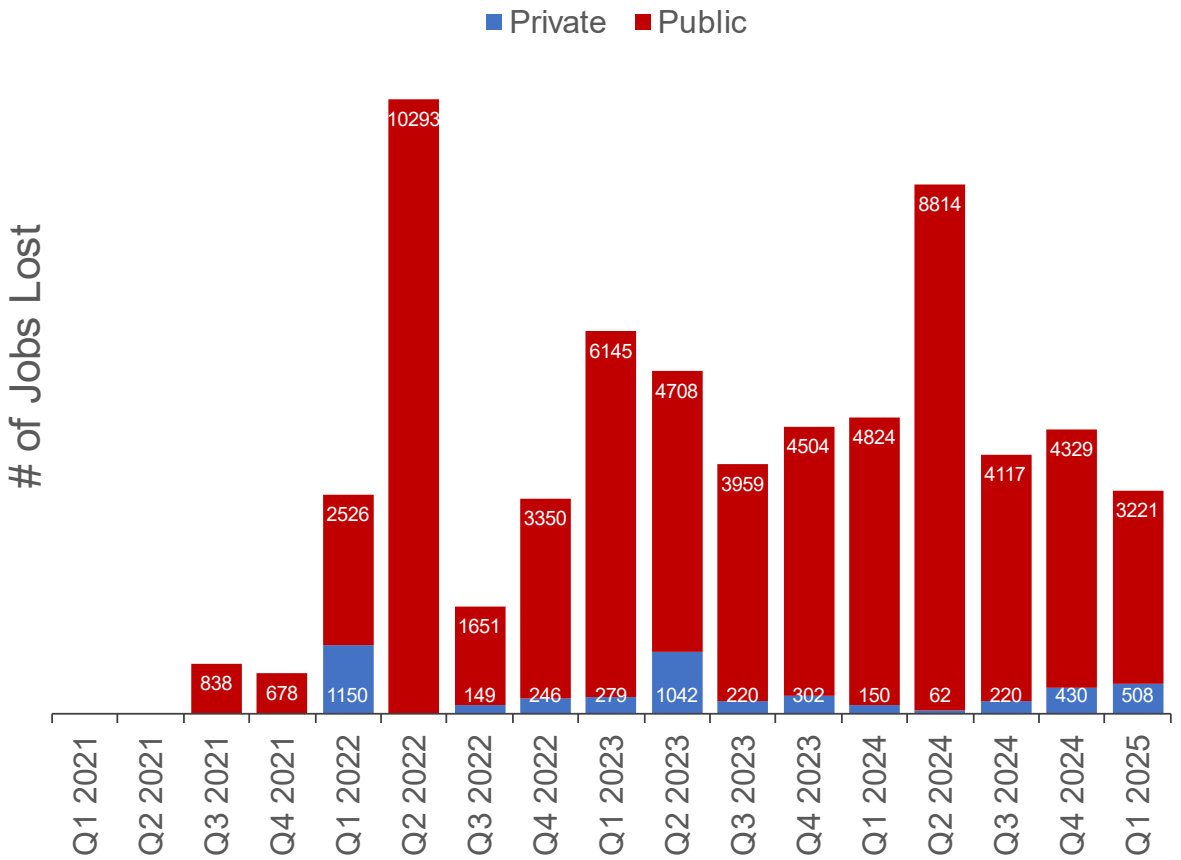
Therapeutic group	Added during 2024	Discontinued	Net change	Relative growth
Anticancer	1,763	-1,429	334	3.7%
Rare diseases	**908**	**-378**	**530**	**7.4%**
Neurological	626	-471	155	4.2%
Metabolic	477	-482	-5	-0.2%
Anti-infective	368	-440	-72	-2.4%
Cardiovascular	**214**	**-94**	**120**	**11.0%**
Immunological	208	-192	16	1.1%
Musculoskeletal	205	-122	83	4.0%
Sensory	**185**	**-88**	**97**	**8.0%**
Dermatological	**162**	**-54**	**108**	**8.9%**
Respiratory	126	-111	15	1.3%
Genitourinary	**112**	**-47**	**65**	**7.9%**
Blood and Clotting	101	-73	28	3.6%
Antiparasitic	13	-16	-3	-2.7%
Hormonal	8	-10	-2	-0.7%

*Therapy areas in **bold** denotes an above-average growth rate*

Source: Citeline, Pharmaprojects

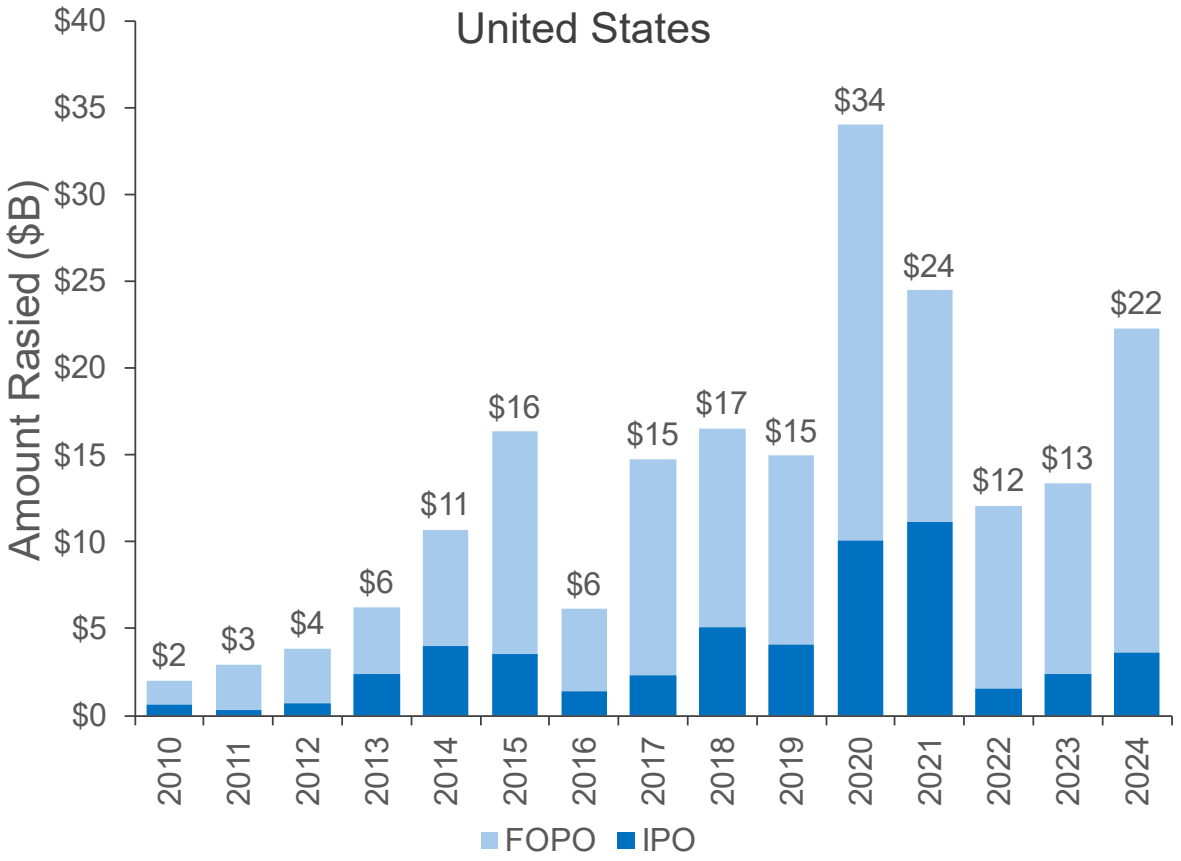
Layoffs at BioPharma Companies



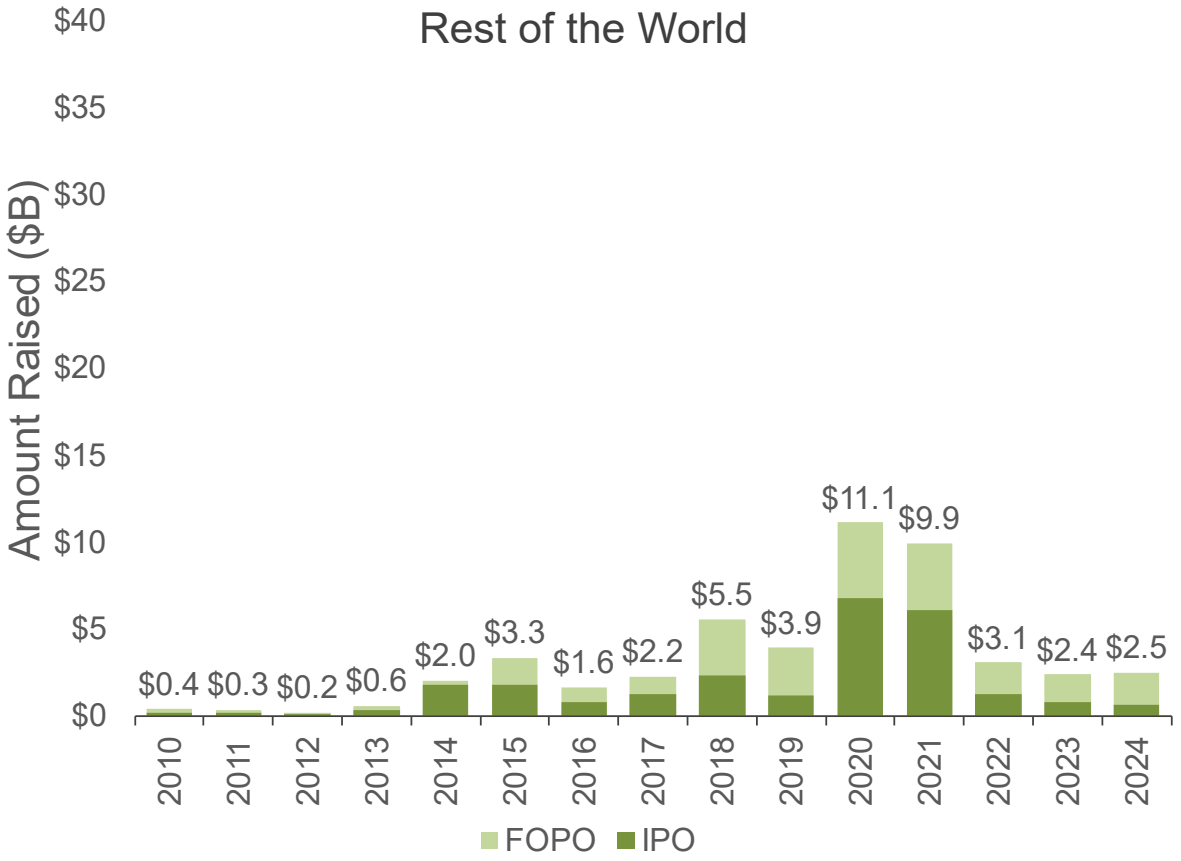


Source: Company Press Releases, Biospace, EndPoints, Biopharmadive, Fierce

Public Investment into R&D-Stage BioPharma Companies



U.S. companies continue raising money through follow-on offerings while IPO funding has been slowly growing



The amount raised in IPOs outside the U.S. has continued to drop over the past three years while follow-on offerings has stayed roughly the same

IPOs & FOPOs raising $10M+; Sources: SEC, BCIQ

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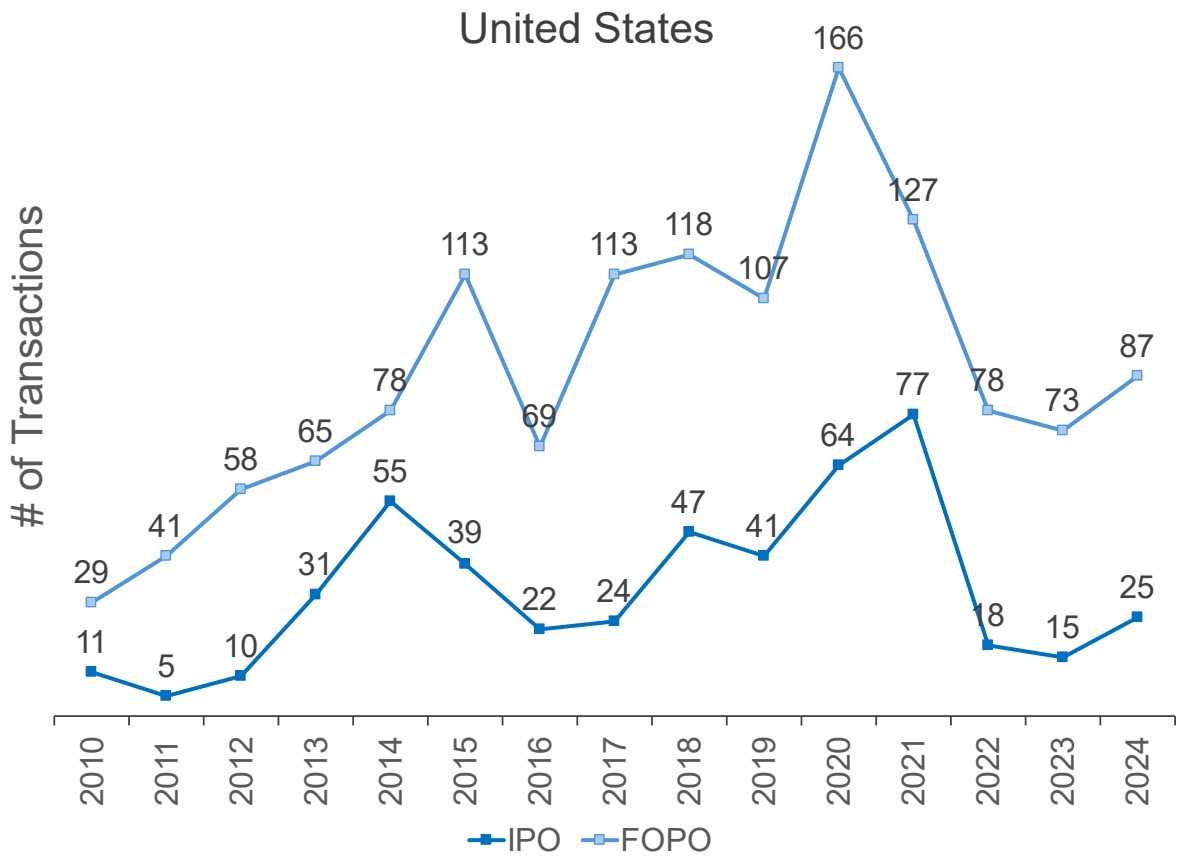
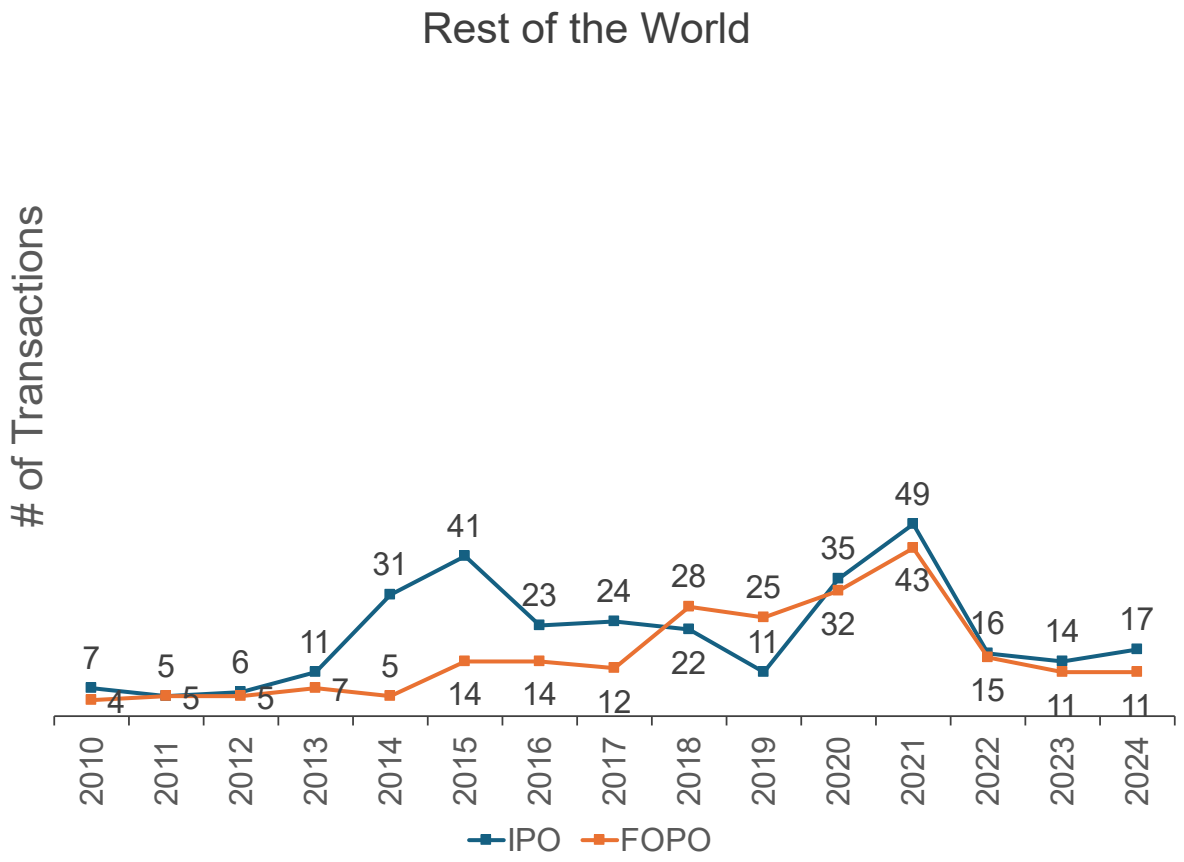
Public Investment into R&D-Stage BioPharma Companies



The number of transaction for both IPOs and FOPOs have increase in 2024, but still below the numbers seen between 2018-2021



The number of FOPOs and IPOs transactions has been relatively flat the last three years, well down from the peak in 2021

IPOs & FOPOs raising $10M+; Sources: SEC, BCIQ

Deal-making snapshot

Mixed picture to 2025; drop in financing coincides with increased partnerships and M&A

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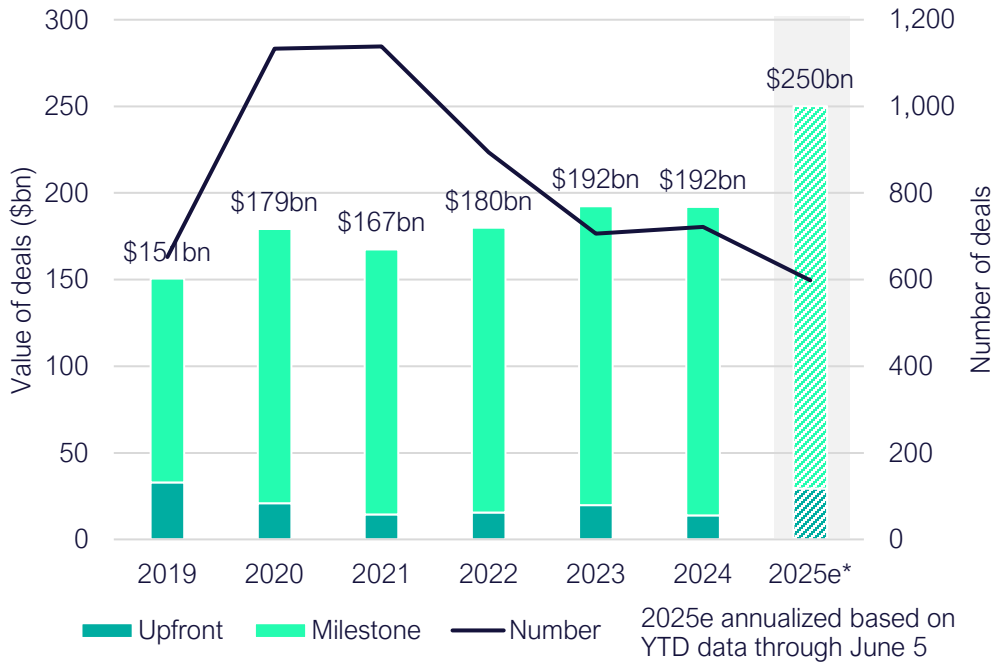
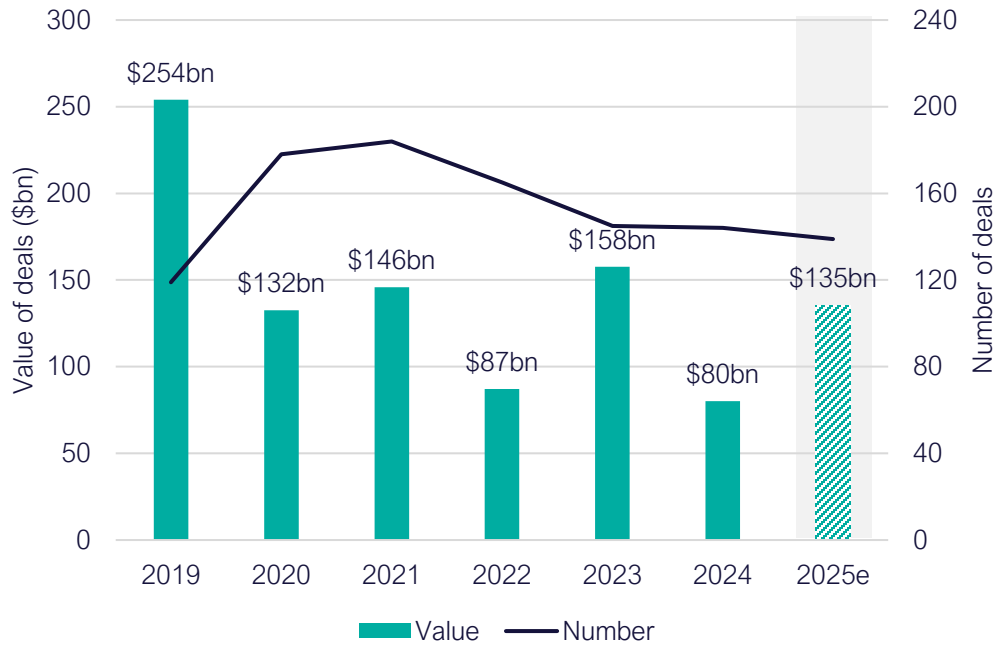
Alliances



Acquisitions



- Continued demand for alliances to access external innovation
- Strong start in 2025 with numerous blockbuster obesity transactions
- Share of deal value tied up in milestones continues to rise

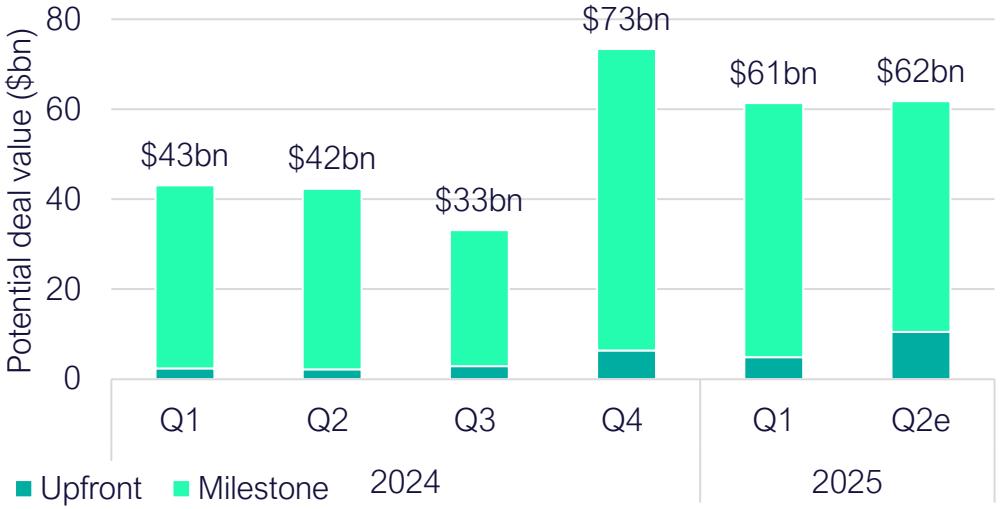
Source: Citeline and Evaluate, Biomedtracker

- Large slowdown in pharma capital allocation via M&A during 2024 as no single deal >$5bn concluded
- Deal-making bounce in 2025 with January M&A flurry and continuing cadence of bolt-on deals
- Intra-Cellular, Blueprint the highlight take-outs; trend towards private biotech exits

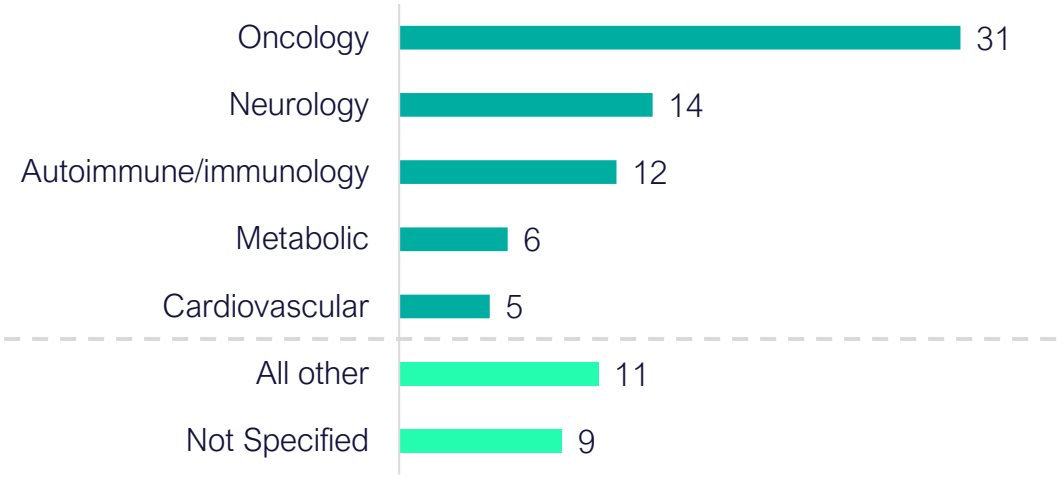
Alliances

Preferred method to access external innovation, while structuring deal to manage risk

Biopharma alliances by payment structure



2024 alliances >$1bn by therapy area



Alliance activity holding up well

- Important source of funding, validation, and innovation
- Total potential deal value rising as upfront contribution shrinks
- Obesity race creating several high-value partnerships in early 2025: Roche-Zealand, Novo-United Biotechnology, AbbVie-Gubra, Lilly-Olix, Verdiva-Sciwind

Strong activity in usual large pharma therapy area focus areas

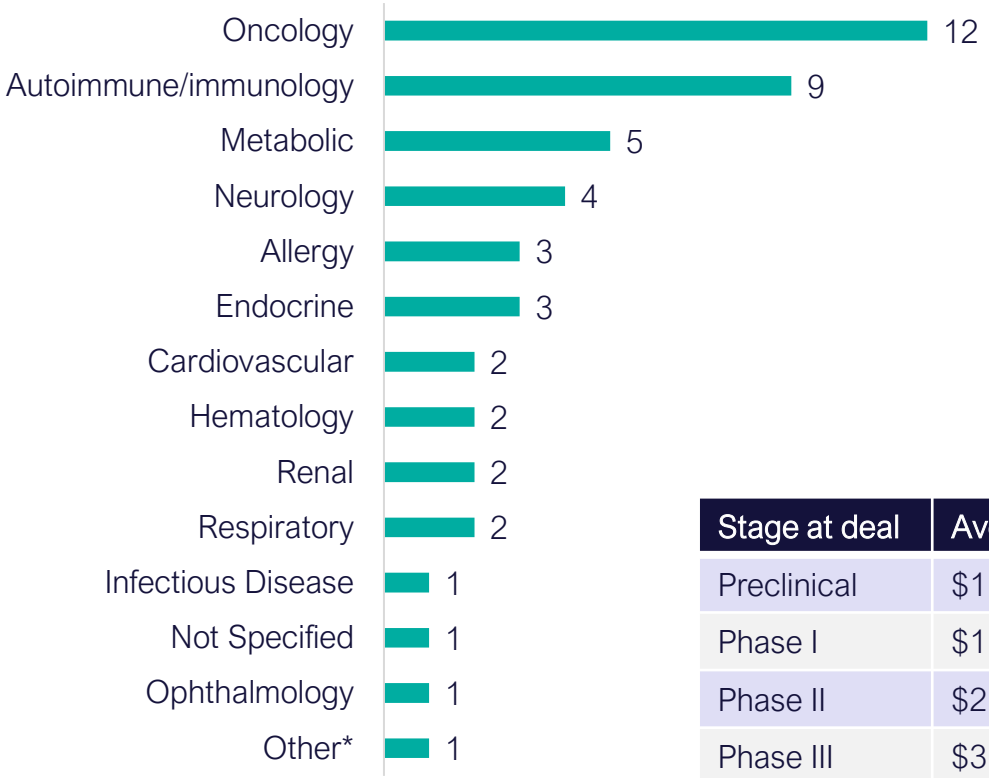
- Oncology, neurology, immunology are the perennial top three TAs for pipelines and investment
- Cardiovascular rising as large pharmas, led by AstraZeneca, Novartis and Novo Nordisk, strengthen their portfolios

Source: Citeline and Evaluate, Biomedtracker

Acquisitions

2024 an outlier as buyers return to de-risked, late-stage or commercially proven assets

2024 M&As >$1bn by therapy area

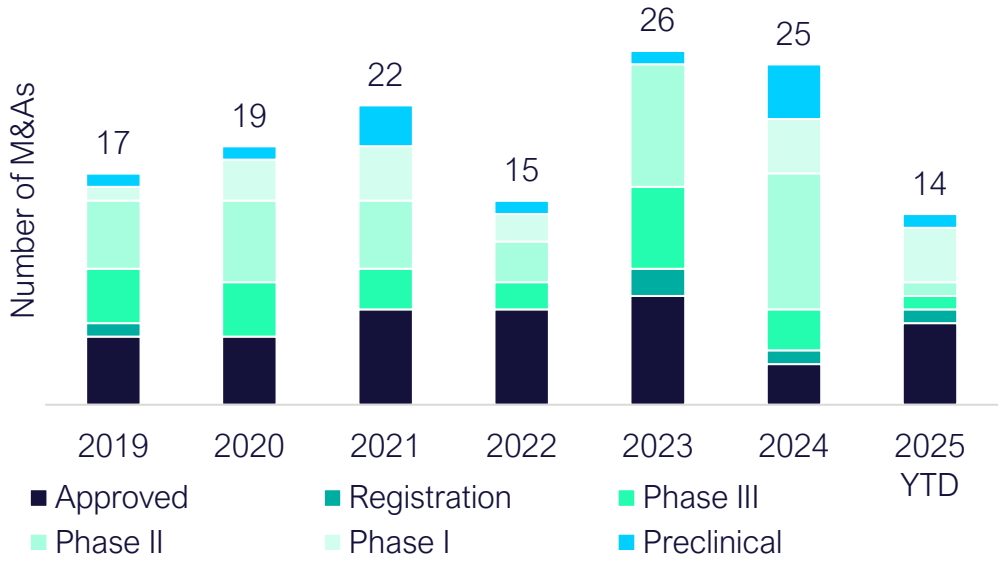


Stage at deal	Ave. value*
Preclinical	$1.4bn
Phase I	$1.5bn
Phase II	$2.7bn
Phase III	$3.9bn
Registration	$5.2bn
Approved	$10.5bn

for asset-driven M&As >$1bn

Source: Citeline and Evaluate, Biomedtracker

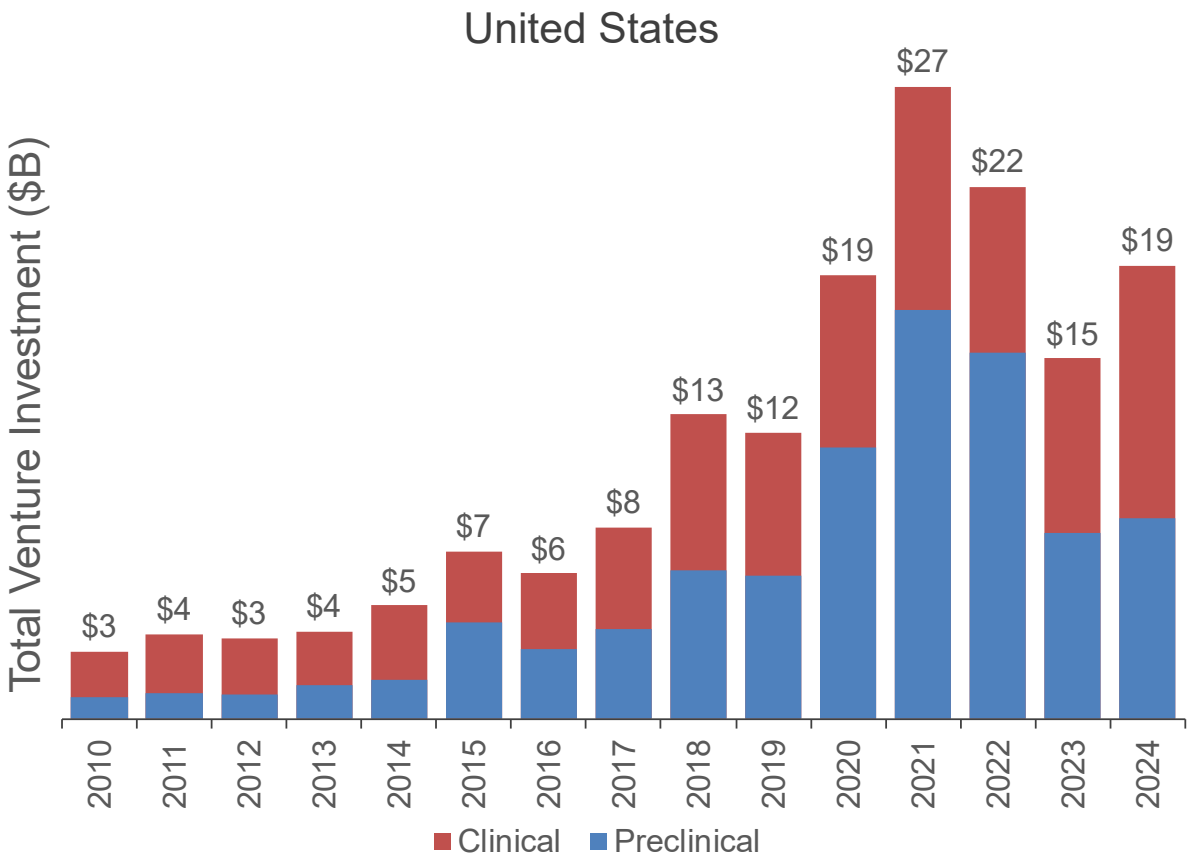
Asset-driven M&As >$1bn



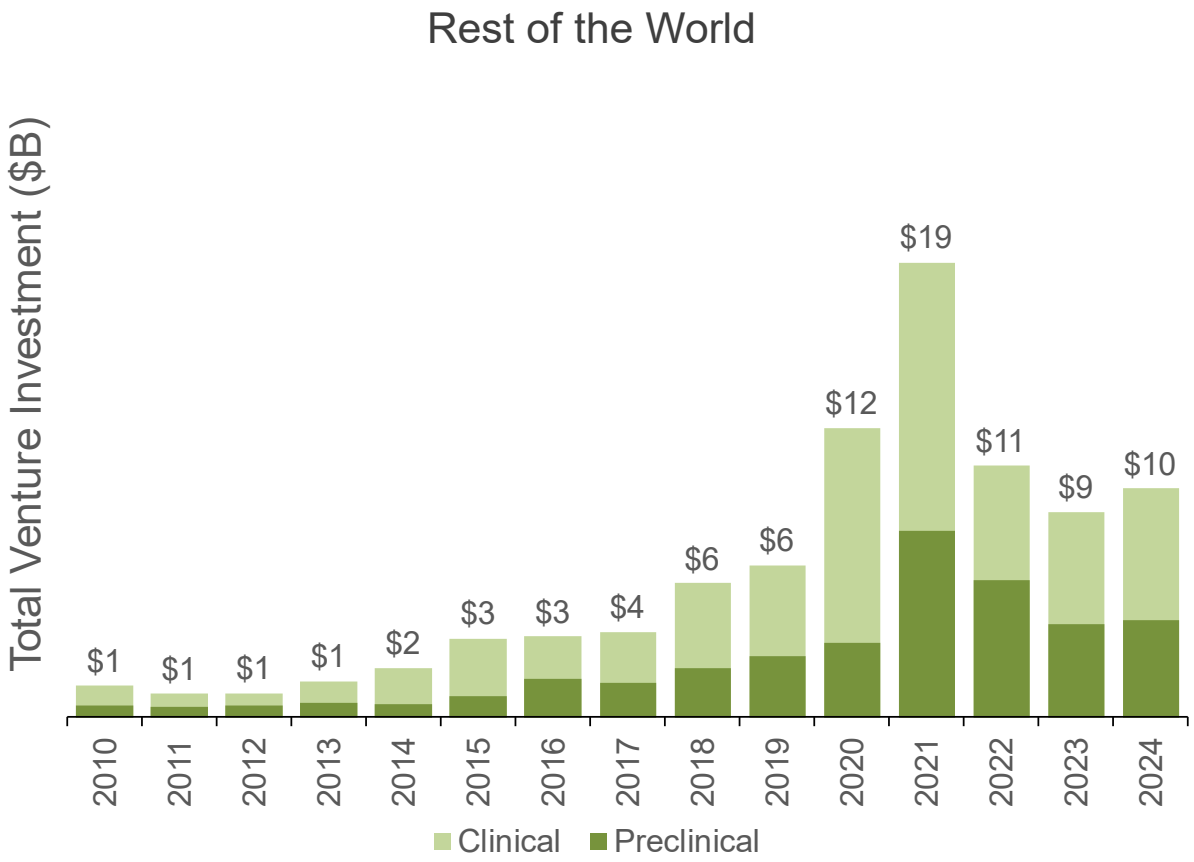
Bolt-on M&A driven by near-term commercial growth drivers

- While alliances are trending earlier (>50% preclinical), M&A remains biased towards de-risked, late-stage assets
- Clinical validation drives much higher valuations
- 2024 an exceptional year as deals trended much earlier (and at lower valuations)

Venture Dollars Investment into BioPharma Companies



Venture funding levels remain strong in 2024, although the number of transactions are down from the previous three years

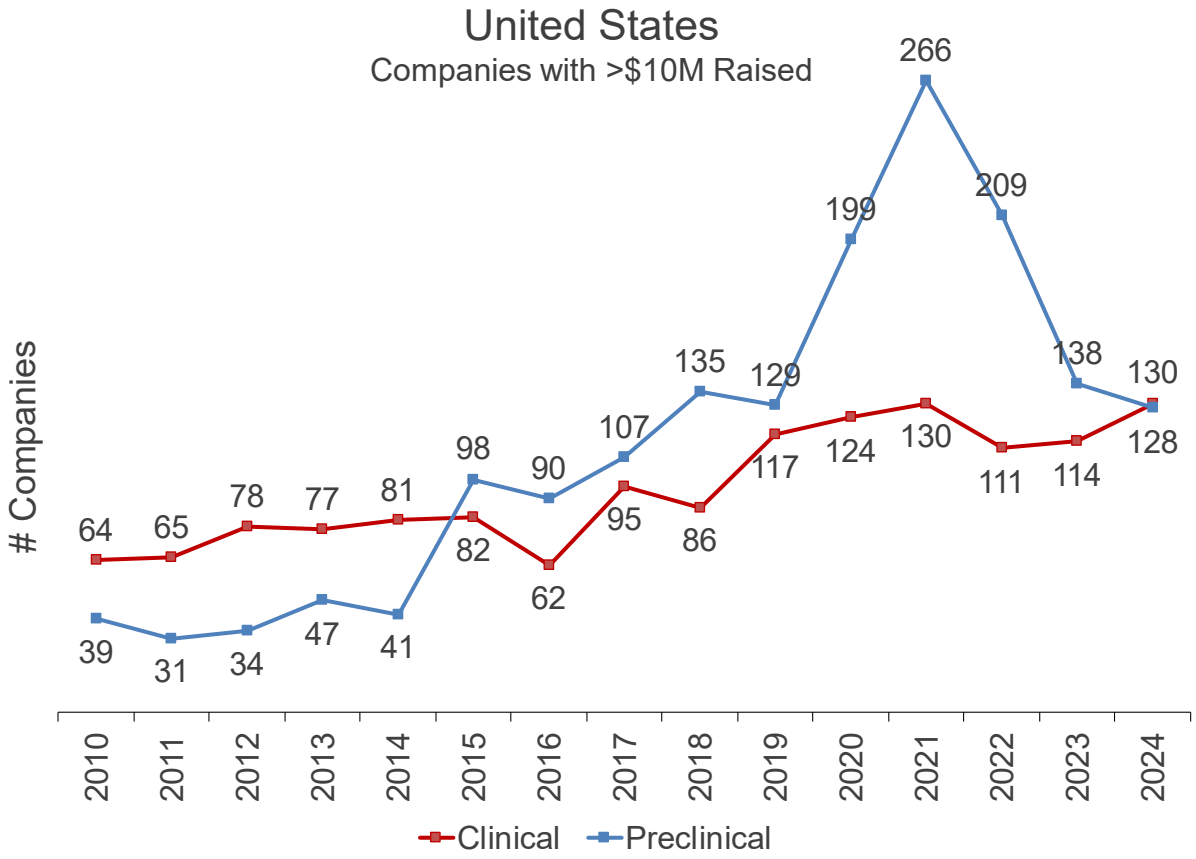


Top 2 countries to raise VC funding in 2024 outside of the U.S. is China with $2.6 billion & UK with $2.4 billion

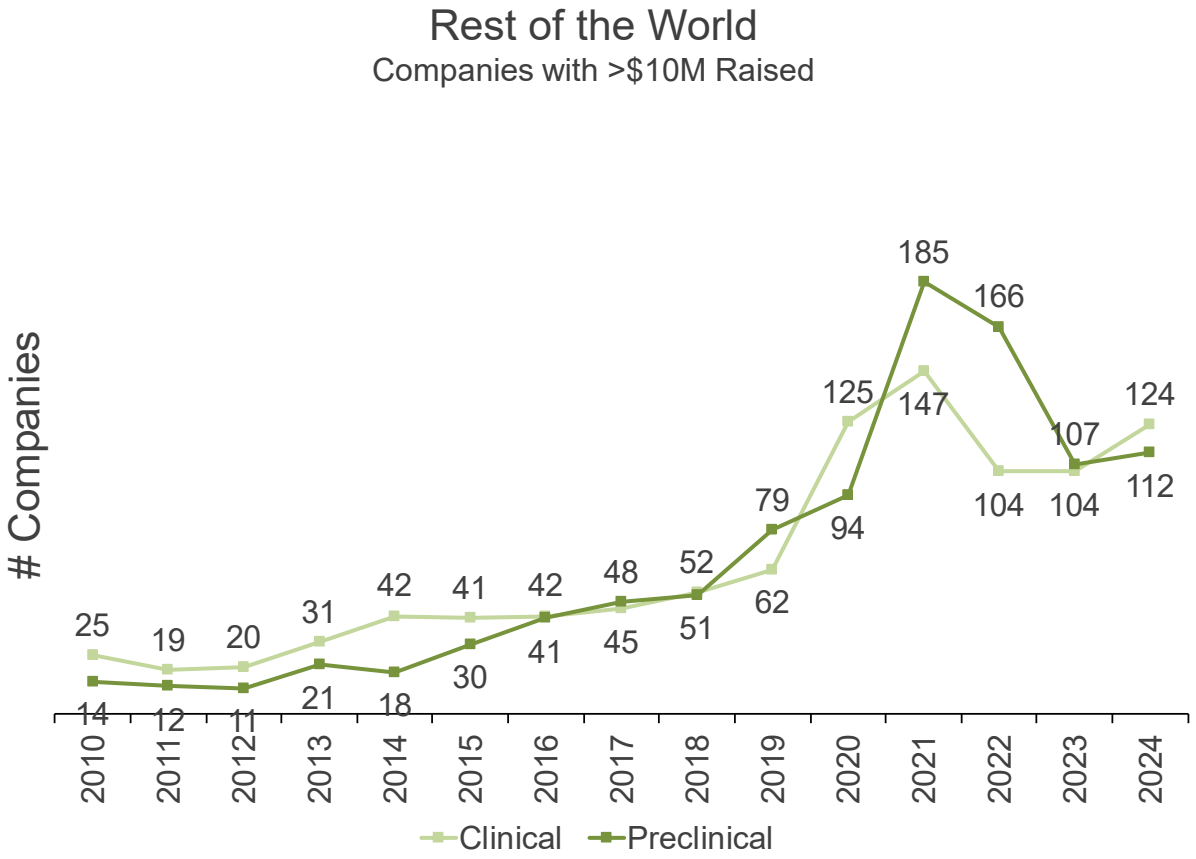
Databases used: Pitchbook (primary), Biocentury BCIQ , Cortellis/TR, EvaluatePharma

More @ www.bio.org/iareports

Venture Capital Transactions into BioPharma Companies



In 2024, VC transaction for clinical stage companies has surpassed preclinical stage companies for the first time since 2014

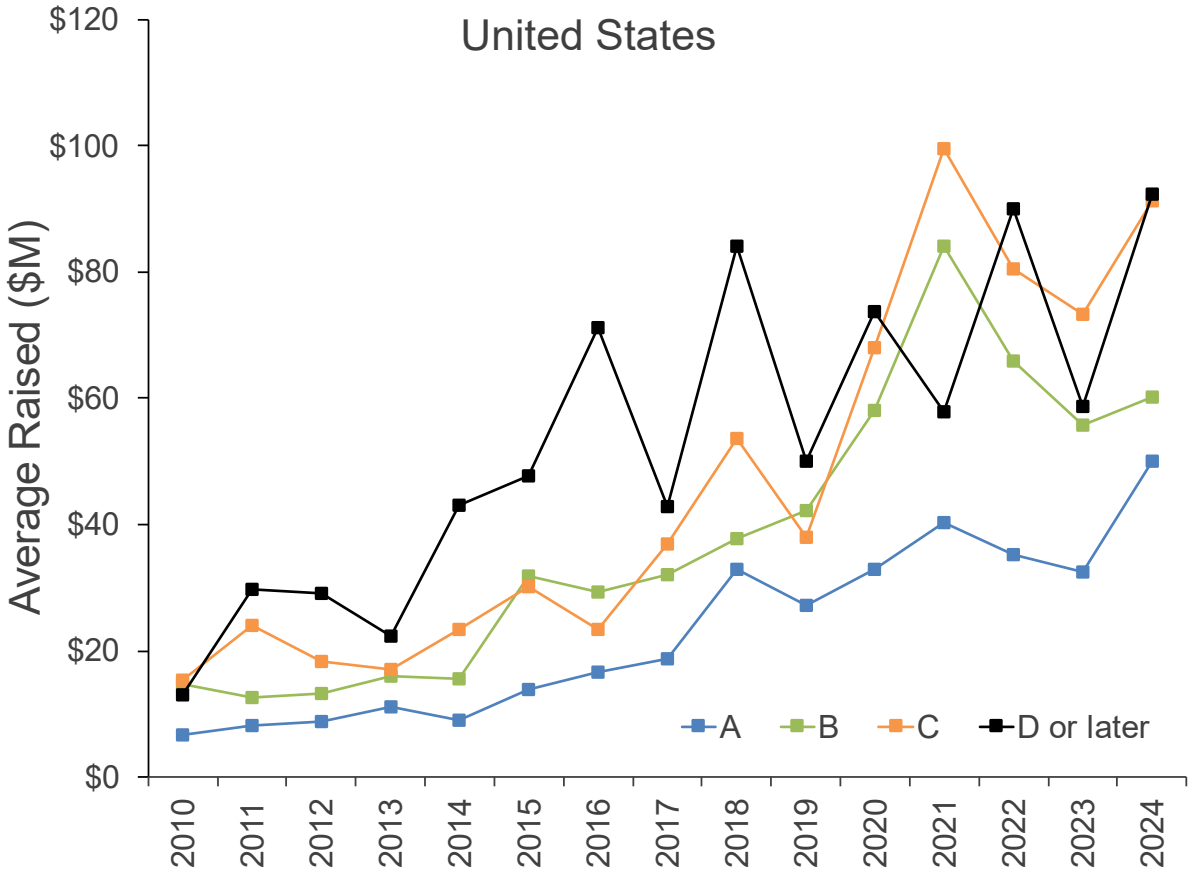


The number of transaction outside the U.S. has continue to grow since 2012 with the majority coming from early-stage companies

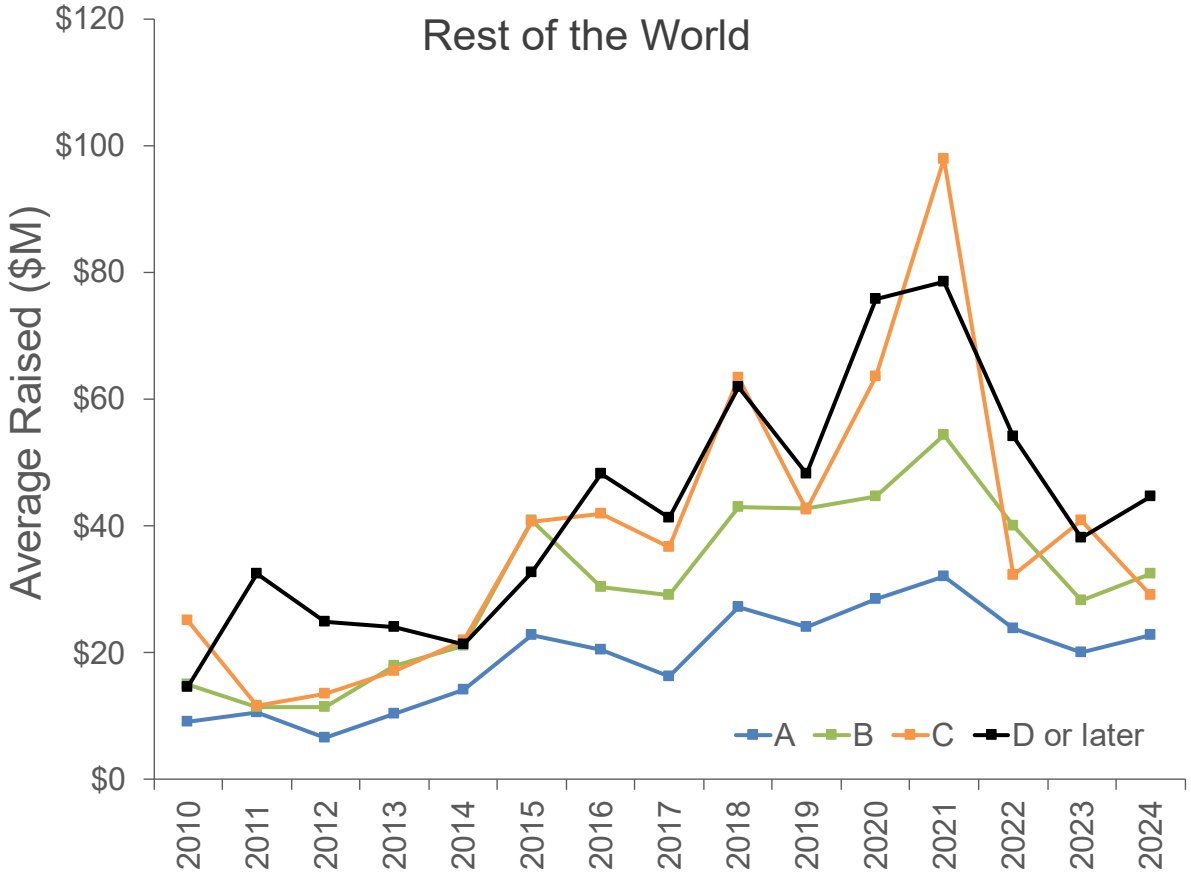
Databases used: Pitchbook (primary), Biocentury BCIQ , Cortellis/TR, EvaluatePharma

More @ www.bio.org/iareports

Average Venture Investment into BioPharma Companies

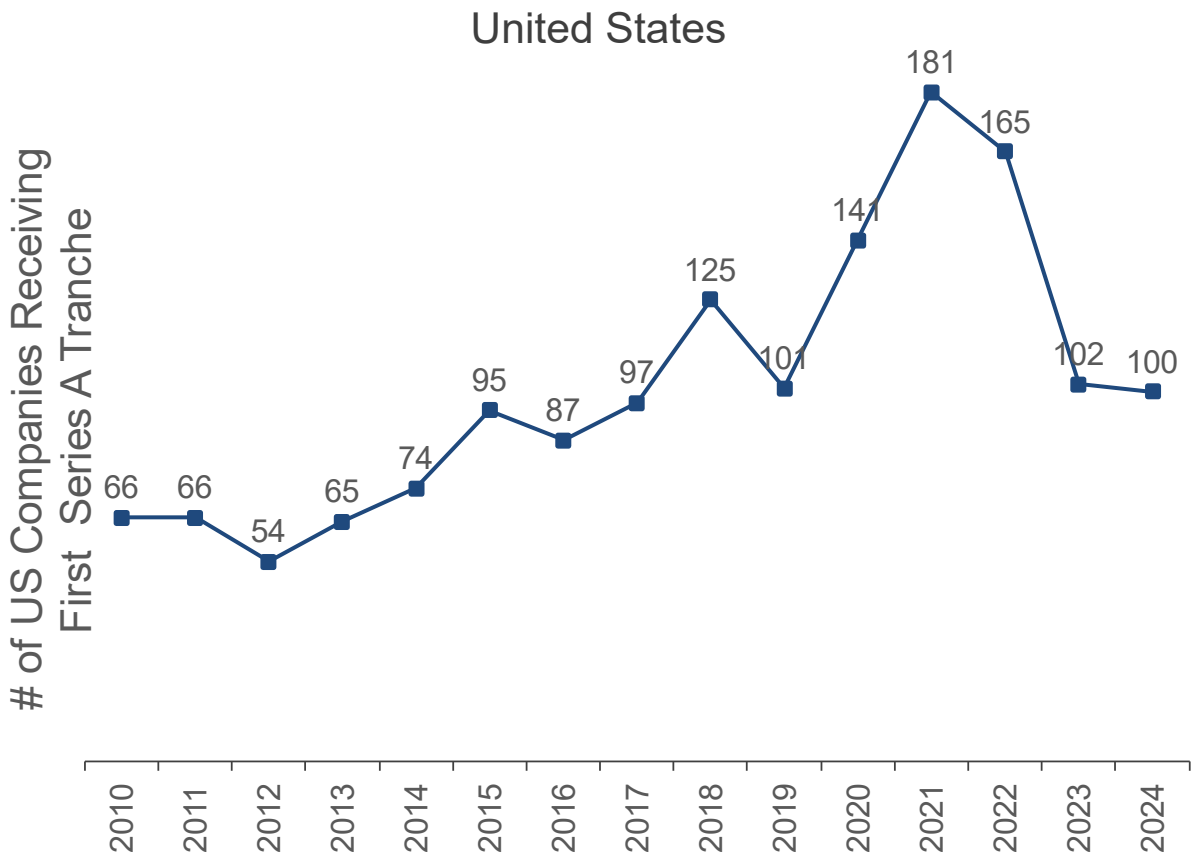


The average size of transaction in the U.S. has continued to grow over the past 15 years for all funding rounds.

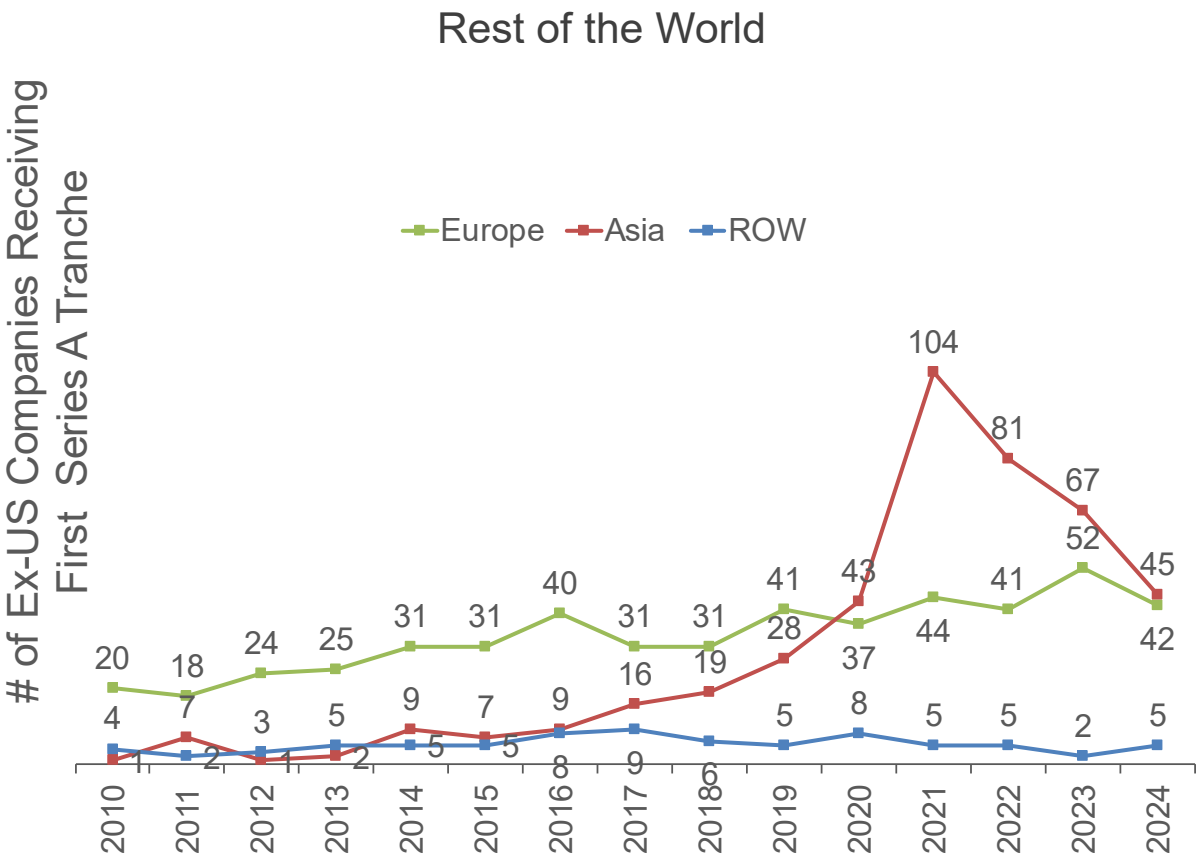


Compared to the U.S., the average funding size for the rest of the world is growing at a slower rate, with the last three years being significantly down from the 2019-2021 timeframe

Series A-1 Investment into BioPharma Companies

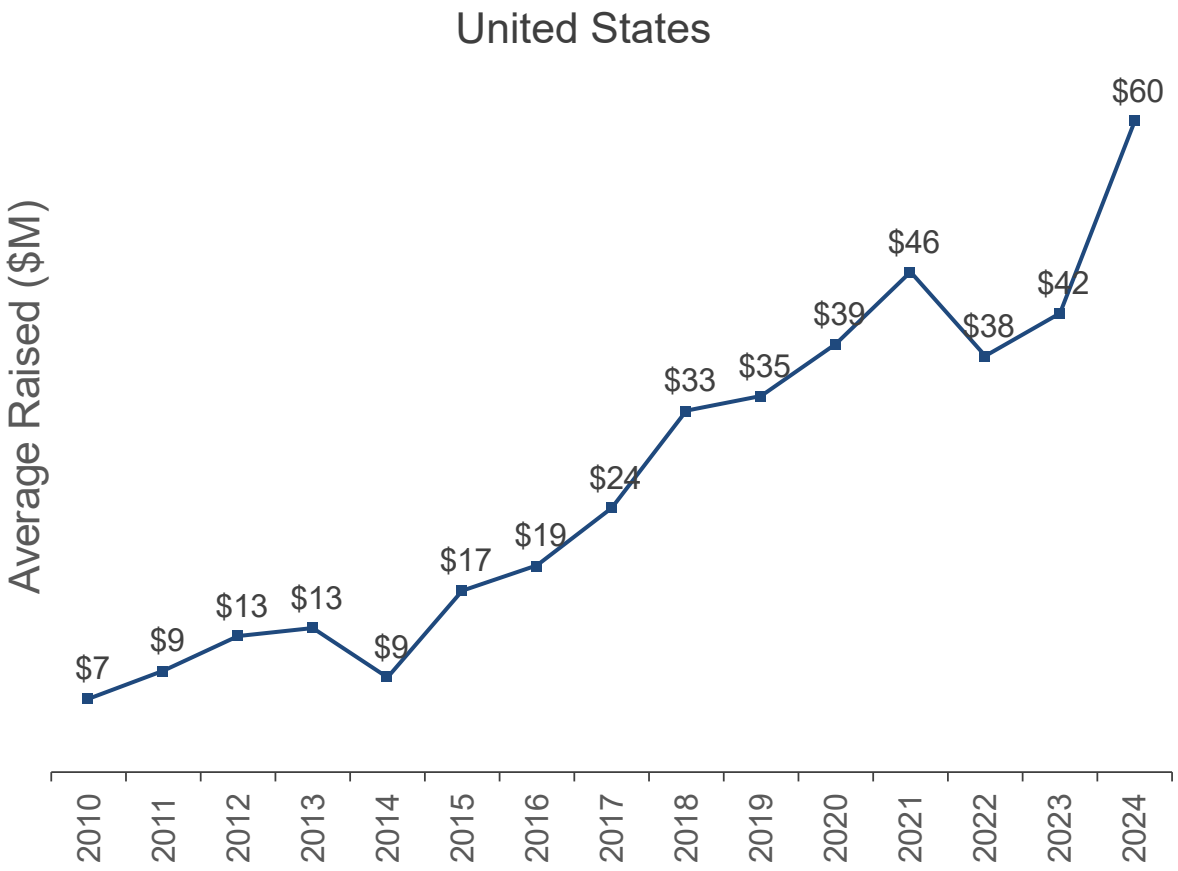


The amount of new A-1 rounds is flat between 2023 & 2024, down to levels last seen prior to COVID

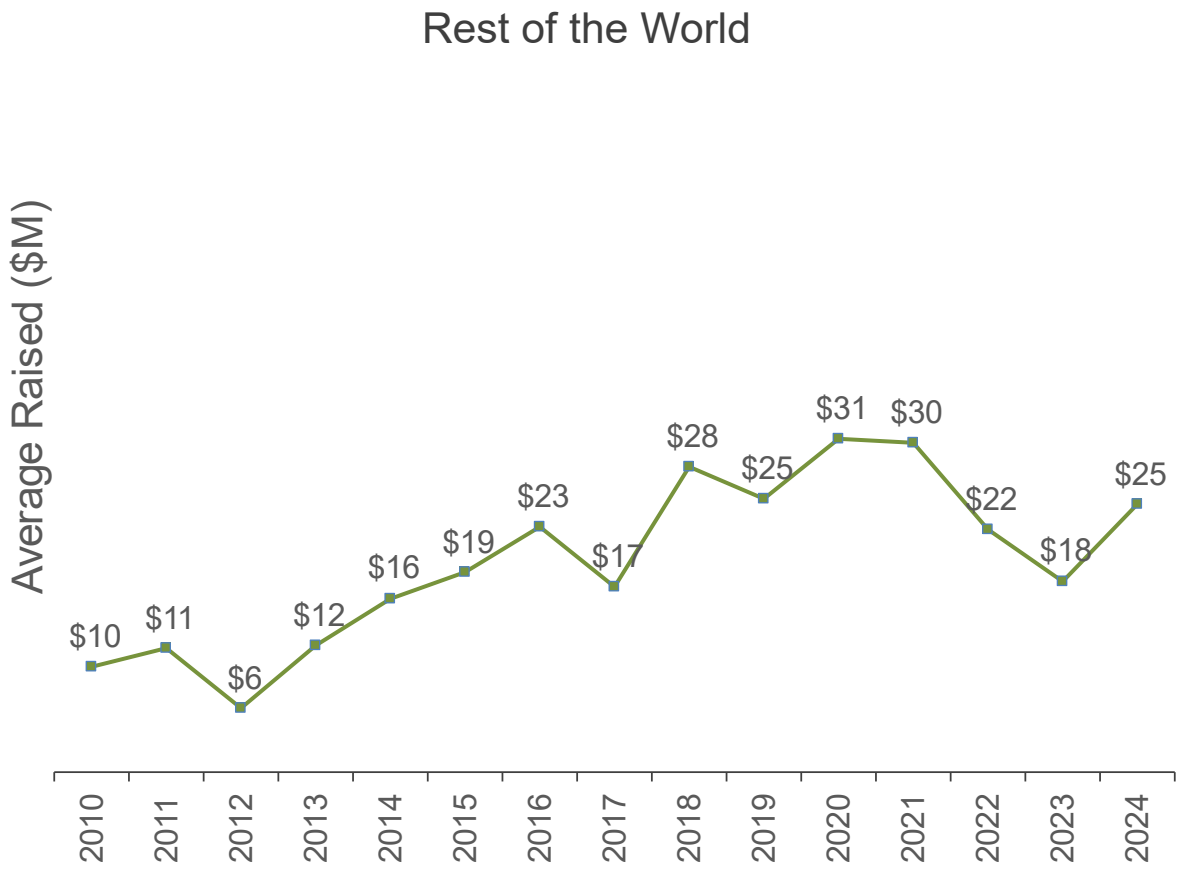


A-1 transaction has been relatively flat for rest of the world with Asia being the exception, largely due to the increase of Chinese transactions since 2019.

Average Series A-1 Investment into BioPharma Companies



The average amount for A-1 transactions in the U.S. have had a remarkable increase over the past 15 years with an over 700% increase



The rest of the world has been much more modest in the average amount invest in A-1 rounds with a 150% increase over the past 15 years

Databases used: Pitchbook (primary), Biocentury BCIQ , Cortellis/TR, EvaluatePharma

For a copy of todays slides please visit the BIO Industry Analysis Homepage

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Questions? Reach out to us at cwessel@bio.org and daniel.chancellor@norstella.com