WHITE PAPER

Why therapeutic platforms could provide a powerful innovation model

The promises and unique value drivers for large pharma

By Anna Kataki, Andrew Chiang, Nan Wang and Olivier Lesueur

Since the early 2000s, large pharma companies have shifted to inorganic growth as their major tool to drive innovation and portfolio expansion. In line with this trend, their investment focus has been on later-stage single assets that could potentially reach blockbuster status. But this asset-driven model may not be sufficient to sustain innovation and revenue growth. Meanwhile, R&D ROI is expected to continue to decrease due to the increasing challenge of providing substantial benefit over the standard of care. Overall, large pharma companies are looking for alternative models to drive de-risked growth.

Therapeutic platforms present an enticing model to source innovation in a large pharma portfolio. A therapeutic platform is a common base of technologies leveraged to produce multiple novel therapeutics with reduced asset-specific add-on technologies. These platforms may offer synergy, add flexibility to develop assets for many indications and enable faster scaling.

Given the therapeutic platform model's potential to help pharma companies navigate key risks and challenges, we set out to explore its promises, its unique value drivers and why large pharma should embrace it.

Exploring the promises of the therapeutic platform model for biotech

To understand the impact of the therapeutic platform approach on overall R&D productivity, we evaluated timelines and costs across companies that have embraced a platform model versus those that have not. We chose to restrict our analysis to biotech companies to avoid confounding factors associated with large pharma, such as external sourcing, broad portfolio priorities and others. To evaluate the group with a platform strategy, we divided the top 60 biotech companies by market cap (Figure 5) into two sets and analyzed them across multiple dimensions, including the average time for follow-on assets to enter the clinical setting and R&D costs per phase 1 equivalents (see end notes). The first set, which we defined as "platform companies," included biotech companies that rely on a unified therapeutic platform to generate portfolio assets. The second set included biotech companies that do not rely on a single platform but instead a therapeutic-area or asset-driven strategy.

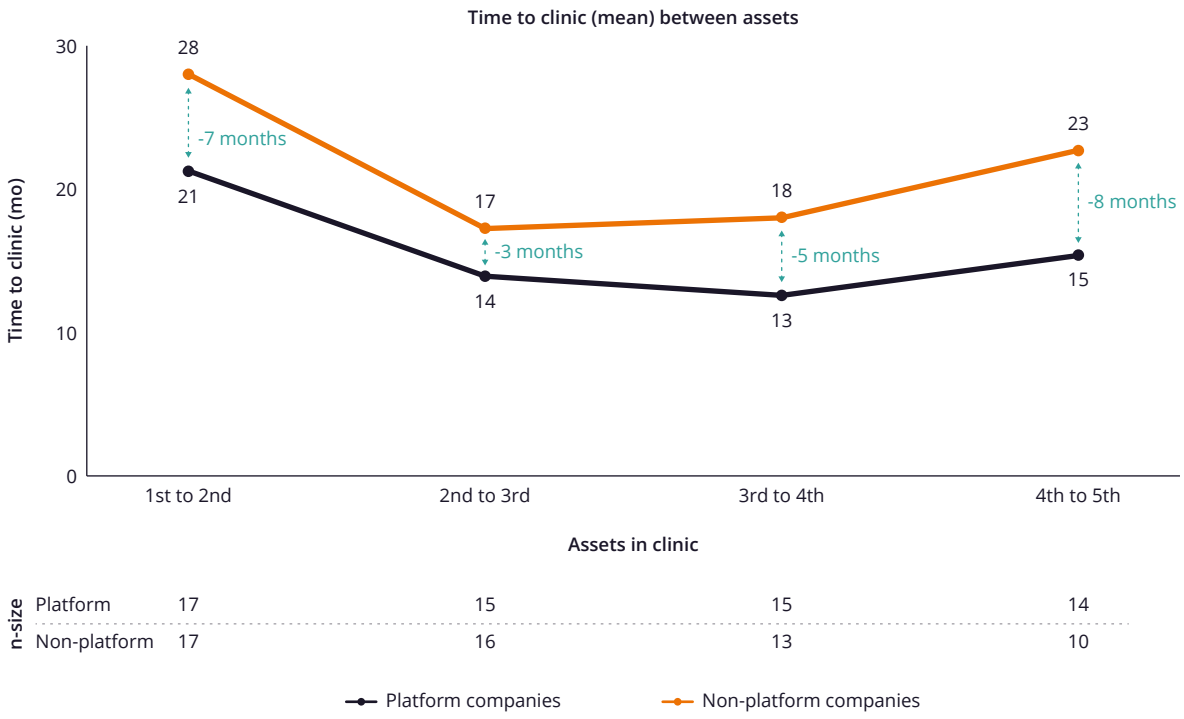
Platforms offer a scalable engine to a portfolio that can generate "more shots on goal" and faster proof-of-concept realization. They offer the synergy and optionality for pharma to pursue multiple smaller indications simultaneously. The therapeutic platform model is an intriguing addition to the changing drug development and market landscape that opens up opportunities for de-risked innovation and revenue growth.

Therapeutic platforms accelerate early asset development for biotech

Our analysis of biotech companies found that platforms improve pipeline throughput and reduce the time it takes to generate the next clinical asset. Average time to clinic was shorter for generating follow-on assets in the platform company sample. Synergies in investigational new-drug-enabling activities, such as validated target and technologies, prior learnings in indication selection, lead optimization and preclinical activities, might explain the ability for biotech companies with a platform model to accelerate time to clinic (Figure 1).

FIGURE 1:

Average time to clinic of second through fifth pipeline assets for platform and non-platform biotech companies



Source: Evaluate Pharma, AdisInsight, Pitchbook, Clinicaltrial.gov, Trialtrove, Biomedtracker

Specifically, there was an average of three- to eight-months difference in time to add clinical assets between platform and non-platform companies, which translates to about two-to-three additional clinical-stage candidates every five years when relying on a

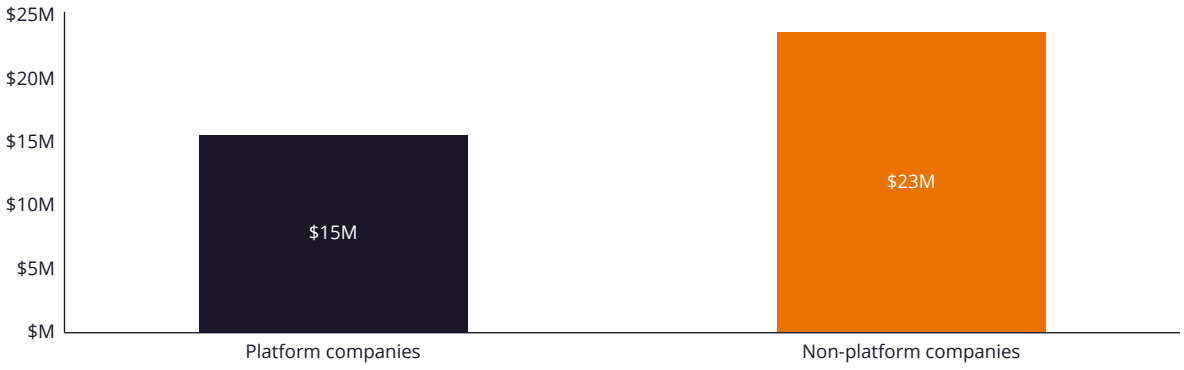
therapeutic platform. Interestingly, time to clinic for the lead asset (or the time between when the company is formed and the lead asset enters the clinic), was also faster for platform companies (approximately 10 years as compared to more than 12 years), potentially due to unrelated factors such as founding team experience or early stage funding, among others. Our analysis found that platforms are able to accelerate the pace of new clinical-stage asset generation, allowing companies to have "more shots on goal," potentially richer late-stage pipelines and easier expansion into new indications.

Platforms deliver cost synergies for early pipeline assets

Our analysis showed that the average R&D costs per phase 1 equivalents was lower for platform companies, potentially due to synergies in R&D technologies, materials and infrastructure, along with accelerated asset development (Figure 2). To understand R&D cost associated with phase 1 equivalents in 2021, we analyzed historical R&D investment (2012-2018) to account for time lag between investment to clinical stage output. We used phase 1 equivalents (see end notes) to mitigate the higher clinical development costs of late-stage assets and to account for differences in infrastructure costs between maturing and emerging platforms and companies.

FIGURE 2:

Mean R&D cost (2012-2018) per phase 1 equivalent in 2021 for platform and non-platform biotech companies



Sources: Evaluate Pharma, AdisInsight, Pitchbook, Clinicaltrial.gov, Trialtrove, Biomedtracker, ZS internal analysis and company websites

We aimed to understand whether platforms can lower the investment required per asset potentially arising from scientific, formulation and manufacturing cost synergies with lead assets and higher throughput. We observed lower R&D costs per phase 1 equivalent for platform companies. Interestingly, the decreased R&D costs were agnostic to therapeutic area and modality.

Offering increasing savings on R&D costs, along with higher throughput of subsequent assets, platforms provide a powerful value proposition to fill in the early pipeline of clinical candidates. Platform companies may then have more opportunities to partner faster and de-risk their portfolios. By scaling preclinical activities faster, a platform company with a $100 million R&D budget may generate approximately seven subsequent assets compared to a non-platform company generating four assets.

How large pharma is using therapeutic platforms

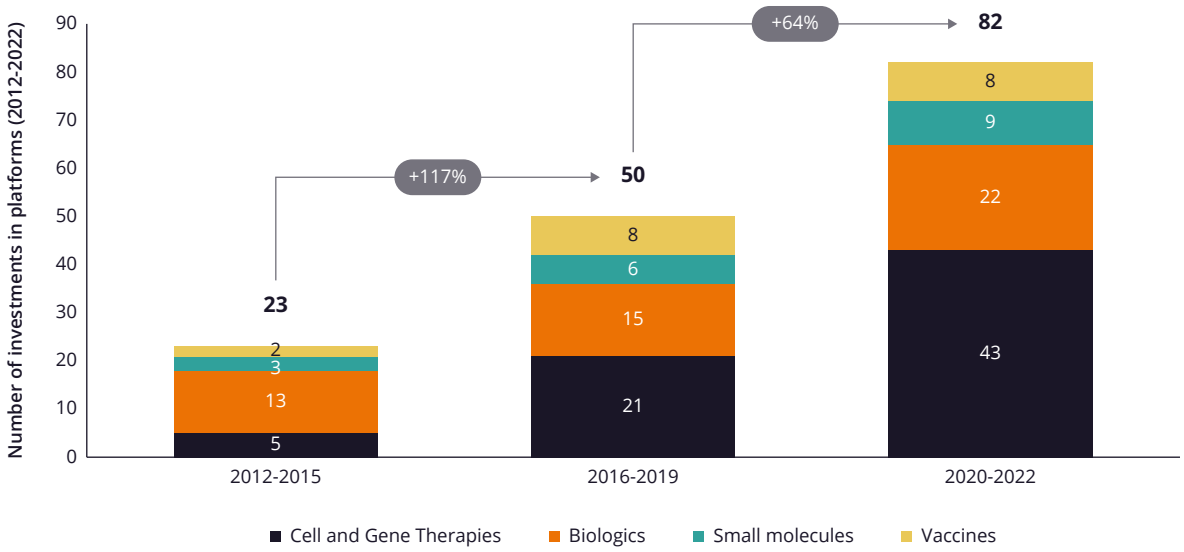
After identifying the advantages of a platform model for biotech companies, we explored how those advantages translate for established large pharma companies.

We analyzed internal and external investments (in-house development, collaborations, licensing deals and acquisitions) related to a therapeutic platform for 2012-2022 to identify the exposure of the 18 largest pharmaceutical companies to platforms. Large pharma investment in platforms grew dramatically across therapeutic modalities between 2012-2015 and 2016-2019 (Figure 3) and continued to increase in recent years (2020-2022). We observed an upward trend in cell and gene therapy platform investments coinciding with FDA approval of the first cell therapies (2017).

By scaling preclinical activities faster, a platform company with a $100 million R&D budget may generate approximately seven subsequent assets compared to a non-platform company generating four assets.

FIGURE 3:

Large pharma internal and external investments in therapeutic platforms (2012-2022)



Note: Data reflects the number of investments in each therapeutic modality out of all platform-related deals and internal investments large pharma has made from 2012 to July 2022.

Sources: Biomedtracker and ZS secondary research

Additionally, we took a deeper dive into which companies have invested in platforms and to what capacity. Across our large pharma sample set, we found two archetypes of companies investing in platforms between 2014 and 2022 (Figure 4). The first archetype, "early movers," invested in platform capabilities prior to 2018. The second archetype, "late movers," invested in the second wave of therapeutic platforms and are still catching up to the "early movers."

FIGURE 4:

Large pharma investments in therapeutic platforms and platform capabilities

Large pharma companies	2014-2018	Platform capability focus	2019-2022	Platform capability focus
Novartis	3	100%	5	40%
Roche	3	100%	2	-
Merck	6	67%	11	55%
Lilly	3	67%	10	30%
Sanofi	6	50%	9	78%
Johnson & Johnson	6	50%	6	17%
Gilead	5	40%	4	25%
Takeda	6	33%	10	60%
AstraZeneca	3	33%	4	50%
Bayer	0	-	3	100%
Amgen	2	-	4	75%
Astellas	1	-	6	50%
AbbVie	0	-	3	33%
Biogen	0	-	4	25%
Pfizer	2	-	5	20%
Bristol Myers Squibb	2	-	5	20%
GlaxoSmithKline	2	-	2	-
Novo Nordisk	2	-	1	-

Early movers

Late movers

Sources: Biomedtracker, Fierce Biotech, Endpoint News, BioPharma Dive, Pitchbook

Note: The number of deals represented are all deals (acquisition, collaborations and licensing) with biotech platform companies. A company's platform capability focus was defined as investment in platform biotech companies that allowed use and integration into large pharma R&D capabilities.

As we examined the type of investments these large pharma companies were making, we differentiated between capability-focused deals, where large pharma gained access into the platform's know-how and R&D infrastructure, and asset-focused deals, where large pharma invested solely in one or more platform-derived assets (Figure 4). For example, Eli Lilly

invested in the Akouos therapeutic platform and intellectual property around viral vectors for gene therapy delivery in the central nervous system, incorporating these technologies into its existing gene therapy platform. On the other hand, Lilly placed an asset-driven bet with MiNA Therapeutics, where Lilly would oversee preclinical and clinical development without using potential synergies from the MiNA saRNA platform.

A significant portion of the "early movers," including Novartis, Roche, Merck, Eli Lilly, Johnson & Johnson and Gilead, slowed down their capability-focused deals in the second wave and increased ad-hoc asset-driven deals. Most "early movers" have transitioned their platforms to in-house development, including Eli Lilly's Institute for Genetic Medicine after its acquisition of Prevail Therapeutics.

On the other hand, "late movers," including Bayer, Amgen and Astellas, are catching up in platform capabilities by following a similar trend toward in-house development in the second wave of platform technologies. And some "late movers," such as BMS, Pfizer, Biogen and AbbVie, are still slow to embrace platform capability integration and continue to invest in targeted platform-derived assets. Further analysis of deal size and its correlation to large-pharma portfolio strategy will reveal the potential of platforms for large pharma.

Future strategic options for the platform model

For biotech companies, platforms offer synergies and enable faster scaling, delivering advantages in key value drivers, including lower R&D costs and accelerated asset development timelines. Despite large pharma's initial investments in platforms, it's unclear at this time and worth evaluating whether commercial organizations can retain these advantages.

When considering a platform play, large pharma should be prepared for the long haul, knowing that upfront costs may be high and initially payoffs to the pipeline may be slow. For example, large pharma may need to make multiple early stage acquisitions to progressively build platforms in house or bring multiple products to clinic to see cost reduction across its pipeline.

When developing a platform, pharma must strike a balance between following the science to determine the best application and the broader commercial strategy of the company. For example, one "early mover," Novartis, has built an internal radiotherapy platform with two successful launches, multiple late-stage assets and expansion into a theranostic for oncology. To realize a platform's full potential also requires crossing the silos between therapeutic areas that restrict synergies to assess the platform's applicability across the organization. Large pharma needs to grow "new muscles" that may not align with its core portfolio strategy. In Novartis' case, its radiotherapy platform, likely part of the company's early stage asset strategy, may require it to adapt its internal resource allocation and corporate governance to allow systematic partnering-out for opportunities and expansion beyond oncology.

In the future, it's worth exploring the approach to best use platforms to identify the most attractive model for overall therapeutic area and portfolio strategy. Either through acquisitions, licensing and collaborations or in-house development, large pharma has multiple options to build platform capabilities to expand its portfolios or to strengthen its therapeutic area leadership. Thorough value and risk-correlation analysis is needed to understand where a therapeutic platform fits and how it could grow to maximize its potential. And after large pharma companies integrate therapeutic platforms, they should continue to explore solutions to sustain the superior productivity they can achieve by monitoring their platforms' value build-up, while continuing to invest in platform life cycle management through in-house R&D or external innovation. Overall, a decision to invest in a therapeutic platform should come after cross-functional deliberation, risk-benefit analysis across therapy areas and willingness to grow new capabilities within the organization.

End notes

1. Given market conditions and investor appetite, our sample included a high number of cell and gene therapy companies.

2. Phase 1 equivalents were calculated using internal ZS formulas on a per indication basis to reflect variation in clinical development protocols and costs.

3. List of "platform" and "non-platform" companies used in time-to-clinic (Figure 1) analysis and R&D cost (Figure 2). Note: Final n-size of companies used was based on data availability and is outlined in figure legends. We defined platform companies as biotech companies that focused on and solely relied on their integrated platform rather than on a series of incremental technologies or platforms in a broader portfolio strategy (for instance, Seagen's focus on oncology).

FIGURE 5:

List of platform and non-platform companies included in our analysis

	Platform companies	Non-platform companies
1	AbCellera	ALK-Abelló
2	Akeso Biopharma	Allakos
3	Alkermes	Apellis Pharmaceuticals
4	Alnylam Pharmaceuticals	BeiGene
5	Argenx	Beijing Tiantan Biological Products
6	Arrowhead Pharmaceuticals	BioMarin Pharmaceutical
7	Beam Therapeutics	BridgeBio Pharma
8	BioNTech	Cerevel Therapeutics
9	Blueprint Medicines	Chongqing Zhifei Biological Products
10	China Medical System	Denali Therapeutics
11	CRISPR Therapeutics	Evotec
12	CureVac	Gan & Lee Pharmaceuticals
13	Fate Therapeutics	Horizon Therapeutics
14	Galapagos	I-Mab Biopharma
15	Genmab	Innovent Biologics
16	Intellia Therapeutics	Mirati Therapeutics
17	Kodiak Sciences	Organon
18	Legend Biotech	Sana Biotechnology
19	Moderna	Sarepta Therapeutics
20	Novavax	Seagen
21	Regeneron	Tonghua Dongbao
22	Ultragenyx	Vir Biotechnology
23	-	Yuhan
24	-	Zai Lab

About the authors

Anna Kataki is an associate consultant in ZS's Los Angeles office. Anna has executed unstructured problems for clients, bringing together expertise in research, technology transfer and business development to influence business decisions with cross-functional leaders. She is also a member of the portfolio and business development and cell and gene therapy groups, responsible for thought leadership on investment strategy, gene therapy technologies, clinical development and commercialization.

Andrew Chiang is a decision analytics associate who works in portfolio and business development, leading clients through significant qualitative and quantitative analyses. Andrew has experience supporting clients in emerging pharma, large pharma, medical technology and private equity on business development initiatives that include opportunity assessments and commercial due diligence. Andrew leverages strong modeling and quantitative skills, together with qualitative analysis, to deliver holistic and meaningful results.

Nan Wang is a manager in ZS's Philadelphia office. Nan leads matrixed teams in solving complex problems for clients, bringing together scientific expertise and business insights to influence business decisions among cross-functional leaders. She is also a core member of ZS's portfolio and business development group, generating thought leadership advising clients how to leverage innovative business development and licensing approaches to achieve their portfolio ambitions.

Olivier Lesueur is a principal in ZS's New York office, focused on portfolio and pipeline strategy, asset strategic planning and business development and licensing search and evaluation. Olivier has more than 15 years of experience in the life sciences consulting field supporting key pharma and biotech clients address cross-functional challenges at the corporate, franchise, portfolio and brand levels. His interests focus on encouraging collaborations across the care continuum to improve patient outcomes and health equity.

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