Managing the Strategic Evolution of a Bioscience Platform Company

By Robert Thong, January 2015



What are bioscience platform companies? And how do they eventually develop into fully-fledged product businesses? Most industry participants, commentators and investors recognize and understand start-up biotech companies at one extreme, and fully-fledged pharmaceutical and medical device product companies at the other. However bioscience platform companies are neither. They can be very healthy businesses, yet bereft of product revenues or even profits for many years. And for much of their lifecycle, they can be very difficult for investors to pin a precise valuation on.

In this paper, we define what the term "bioscience platform company" encompasses. We also outline the key evolutionary stages of such a company and describe what drives the transition from one stage to another. Last but not least, we highlight for each stage the relevant key success factors that need to be borne in mind. Our aim with this paper is to help the management teams and investors of bioscience platform companies to more effectively navigate their strategic journey.

Author's Note: The content in this paper synthesizes (i) my own experiences working with bioscience platform companies, and (ii) perspectives volunteered by the leaders of such companies. For example, the quotations which appear in this paper emerged from an informal workshop I chaired in the summer of 2014 with the leaders of four drug discovery platform companies. Since the meeting took place in a pre-competitive setting under the Chatham House Rule, neither the individuals, their companies nor the quotation attributions can be identified. Nevertheless, all assertions and opinions in this paper are mine alone and do not necessarily reflect the views of the four individuals nor their companies.

What is a Bioscience Platform Company?

The phrase "bioscience platform company" typically refers to an enterprise whose business model includes three important aspects:

- 1. The company is built around certain comparatively scarce scientific core competencies.
- 2. These compentencies are deployed to enable the generation of a succession of new therapeutic or diagnostic product candidates.
- 3. Application of these compentencies is spread across a wide range of therapeutic areas and specific indications.

While such a company is often centered around a branded proprietary technology (hence the phrase "platform technology company"), there are also platform businesses based just on unique know-how, for example, companies skilled in therapeutic peptides, fragment-based drug discovery or protein engineering. In this latter situation, the companies usually do not brand their platform and many do not even see themselves as having a "platform" per se. Branded or otherwise, both sub-types exhibit similar business characteristics and are included in the scope of this paper. What we do *not* include under this definition is the "classic" biotech/medtech start-up focused on a single very high potential application. Nor do we include here those companies providing contract services which are essentially commoditized.

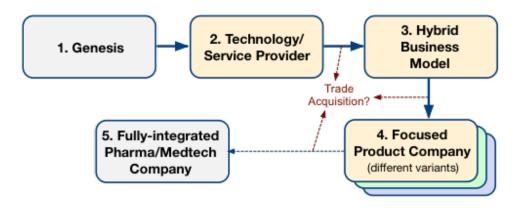
Today, our state of knowledge in biotechnology and how diseases can be diagnosed and treated is comparatively far behind that of for example information technology or aerospace technology. Platform companies provide the **critical mass of people, time, funding and application breadth** for new untested bioscience technologies to develop and be eventually applied against the widest range of potential healthcare applications. This is something which arguably neither large established pharmaceutical and medical device corporations, nor start-up biotech companies, are best set up to handle.

For consistency of narration, this paper adopts a drug discovery flavor. Nevertheless, everything here applies to other stages in the new drug life cycle, as well as to devices and diagnostics, for example inhaler devices, bio-materials, modified release dose formulation, and so forth.

Key Stages in the Lifecycle of a Bioscience Platform Company

A bioscience platform company evolves through several key stages:

Lifecycle Stages of a Bioscience Platform Company



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The company typically has its **genesis** as an academic start-up or a spin-off from an existing pharmaceutical or medical device business. It could have been conceived from day one as a platform company. Or it could have started life as a "classic" biotech seeking to take a single product idea to market, only for that original idea to run aground or for the company to run out of cash. In either case, the company needs to generate revenue by providing its platform to other companies.

If it can generate revenue consistently, it will establish itself as a credible **technology and/or service provider** i.e. Stage 2 in the diagram above. At this stage, it is still very much an awkward adolescent, trying to find its way in the world where others seem to hold all the cards. Its business comprises multiple technology and/or service provision contracts with a portfolio of customers who use the platform for their own projects and products. Licensing (if the platform involves proprietary technology) and contract R&D work is the usual business arrangement. There may be a risk-reward sharing element in these arrangements e.g. milestone payments and small single digit royalties on future sales. But nevertheless their customers are the ones who own, fund and control the direction of the projects.

At Stage 3, the company moves into young adulthood. It starts to originate, fund and direct proprietary projects of its own until they are much closer to the market. While in most cases, these proprietary projects will still eventually be partnered with commercial collaborators at a much later stage, at this point, the company makes the decisions, bears the risks and captures the rewards.

Since the company now requires even more cash flow for funding its proprietary projects, it will continue or even expand its portfolio of technology/service provision deals. Hence it operates a **hybrid business model**, continuing the type of business arrangements which were the staple of the previous stage while simultaneously pursuing its own proprietary projects.

Eventually, the company reaches maturity at Stage 4, when its proprietary projects begin to dominate management attention and enterprise man-hours. As a **focused product company**, its valuation by investors is now founded on anticipated revenues from one or more specific commercial products which are close to (or already on) the market. Operationally, the company continues to concentrate in-house on certain key activities, relying on outsourced service providers and collaborators for other aspects of its value chain. As we will see later in this paper, there are a few variants to how this business model can be realized at this stage.

During the second, third or fourth stages, there is always the possibility of the company being acquired by a customer, collaborator or competitor – the probability increasing rapidly as it evolves through stages three and four in particular. Acquisition could happen for both positive or negative reasons. In the former case, one or more of the projects develops huge commercial value and becomes strategically essential for another player to control. In the latter case, the failure of certain projects leads to a loss of investor confidence, enabling an opportunistic acquirer to buy the company and gain control of its platform.

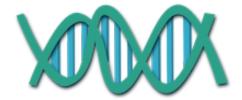
If the company is not acquired by a trade buyer, its original owners will typically engineer a stock market listing via an Initial Public Offering ("IPO") sometime during the second or third stage, depending on the attractiveness of both the capital markets and its customer and project portfolio at the time.

In a few cases, the company may make it all the way to Stage 5 and become a **fully-integrated pharmaceutical or medical technology company**, conducting the bulk of its manufacturing, marketing and sales in-house, in addition to continuing the R&D activities which were its roots. Having said that, many companies and their investors find it makes sense to remain as focused product companies i.e. sustained growth in its valuation is not tied to having a complete in-house value chain.

For the remainder of this paper, we will discuss Stages 2, 3 and 4, concentrating on the:

- Key success factors at each stage.
- Drivers for the transitions from Stage 2 to 3, and from Stage 3 to 4.

Stage 2 - Service/Technology Provider



Unless there is a hugely compelling investment case, a new bioscience platform company cannot usually afford to fund its own projects at the outset. And the platform often needs additional investment for further development. The company thus **needs to generate reliable cash flow by providing its platform to other companies** (its customers) via business-to-business ("B2B") collaboration arrangements. Transition from Stage 1 to 2 is therefore a matter of survival.

Each successive collaboration deal increases its credibility and reputation, enabling it to secure increasingly more high profile collaborators and increase its effective pricing level. Multiple projects with the same collaborator create economies of scale and scope. While a broad portfolio of collaborators is essential to manage risk and to ensure a solid bargaining position in the marketplace. If for example, 12 running projects represents what is required to sustain and grow the company, then 4 collaborators with 3 projects each is much better than either 1 project each with 12 collaborators, or 12 projects with 1 collaborator.

"A broad portfolio of partnered projects spreads corporate risk, ensures business stability and strengthens our bargaining position."

A strong **business development** capability is a critical enabler to assembling this portfolio and becoming an established technology/service provider. In particular, the "**B2B marketing**" aspect of business development is crucial i.e. positioning and differentiating the platform as one which provides unique value in the eyes of the customer. A classic error is to focus on the technical uniqueness of the platform rather than on the unique benefits to the customer that enable the latter to differentiate the resulting end-user products in the customer's own marketplace. This is an important principle in any B2B marketing situation i.e. unique and sustainable benefits in the customer's end-user marketplace are what the customer wants, not the unique science or know-how of the technology/service provider. Hence the platform company's competitors are not just suppliers with a similar platform but also those who can provide the same benefits to the customers in some other way.

"Our scientists sometimes forget that we essentially sell our projects to pharma companies – our projects need to enhance our prospective collaborator's business."

Each successive project broadens the range of situations where the platform can be applied, enhances the efficiency of the platform's delivery owing to the experience curve

effect and triggers further platform-enhancing innovations. A critical enabler is a strong capability in **collaboration structuring and execution**, with respect to every individual project as well as the overall alliance with a collaborator over multiple projects. Working with another company to deliver a project together is much harder than doing it all in-house, the inherently unpredictable nature of bioscience R&D projects further exacerbating this challenge – for a further explanation, see: "Hidden Reasons for Collaboration Failures".

"Collaborations are the lifeblood of our business."

"You have to fit in with your collaborator in some way, especially since many of them are large multi-nationals with their own established processes. And if you're working with several different collaborators, it can get somewhat schizophrenic! Nevertheless, you also have to somehow maintain your own identity and culture, otherwise you lose your own strengths."

Over time with technology platforms, it is not uncommon to introduce "version 2.0" of the platform – to overcome the shortcomings of the original version and to counteract improvements that competitors have made. Alternatively, or in parallel, it could also make strategic sense to stretch the existing platform to a somewhat different application. Furthermore, as the company establishes strong relationships with its collaborators, it will also make strategic sense to widen the portfolio of platforms aimed at the same target customer segments, either through organic development or acquisition.

Key Success Factors for a Technology/Service Provider

- Business Development B2B Marketing and Deal Finding/Making
- · Collaboration Design/Structuring and Execution Management
- Platform Development
 - Version 2
 - New Application Areas
 - Additional Platforms

Stage 3 - Hybrid Business Model

At some point after the platform company has become an established technology/service provider able to sustain its own existence, pressure starts to build to initiate proprietary projects of its own. In some cases, this pressure comes from within, when the management team seeks to control their company's own destiny and maximize the return from their platform. In many cases though, the pressure comes from its owners. Many professional investors fund platform start-ups or spin-outs with expectations of very

high returns if they succeed, commensurate with the high failure rates of similar new companies. The usual way to capture such huge returns is via proprietary projects which eventually lead to large revenue flows from commercial products.

"Investors want the possibility of the 'big pop' when a proprietary drug succeeds"

"There are not many industries where valuations can explode to the extent that they can in ours. Investors can hedge their risk by placing investments across a portfolio of investments. But what they want is the possibility of a company whose valuation explodes. And all the successful biotech case studies out there are of companies who eventually developed their own product."

"At the end of the day as CEOs, we're measured on valuation. And the reality is a service/technology provision business is valued much less than a 'Dreams' business. If you start to talk about own projects and products, the shareholders take a completely different view of your company."

Whatever the reasons, the company now starts looking for new projects where it has at least 50% control and ownership. A critical prerequisite for success is a **deeper business** and corporate development capability, not just in the business development function but embedded into the other parts of the organization that need to be involved to find, evaluate and select the right proprietary project opportunities. Typically, new proprietary projects come from some combination of:

- 1. Identifying internally new potential applications of the platform that it can initiate on its own.
- 2. Initiating 50/50 co-funded collaborations with existing technology/service provision customers, with the option to continue as a 50/50 owner in the later stages.
- 3. Acquiring or in-licensing assets to which the platform can be applied to generate new product candidates.
- 4. Acquiring or merging with a company that has a complementary capability that, when combined with the original platform, enable internal creation of new product candidates.

As the foray into proprietary projects begins, the company will typically maintain (or even expand) its traditional portfolio of technology/service provision collaborations, to spread risk and provide the additional cash flow needed to fund its own projects. Hence the company now operates a hybrid business model.

"We'll have to carry on with technology/service provision collaborations for quite some time, at least until we can build up a broader portfolio of proprietary programs and the funding for them." "Need to build up funds to take our proprietary programs to Clinical Phase 2 PoC, as we'll never raise the hundreds of millions we'll need for this purely from investors."

An important prerequisite for the hybrid model to work well is sufficient **synergy between the proprietary projects and the core platform**. This ensures scale economies and a focused internal operating mindset. Adding proprietary projects that have no synergy with the core platform usually leads to a poor return on investment as there is no mechanism to mitigate the high cost of creating or bringing in these projects. A comparison can be made here with another kind of hybrid business model created in the reverse fashion i.e. starting with a classic cash-burning proprietary product development biotech and adding a cash-generating contract R&D, manufacturing or sales business. Such companies do exist but those that prosper are the ones that create a high degree of synergy across the two parts of the business by deploying the service capabilities to competitively enhance the proprietary projects.

Another important prerequisite for success in the hybrid model is adding the new capabilities required to deliver the proprietary projects. The typical capabilities that need to be added for a drug discovery platform company include clinical development, formulation development, clinical trial supply and regulatory/commercial strategy ("strategic marketing") in the first wave, and at later stages also, manufacturing and sales. Adding a full suite of in-house capabilities across the value chain is too expensive and time consuming for most companies, notwithstanding the risk of adding fixed costs if the projects are delayed or fail. So the usual approach is to add capabilities in a focused and semi-virtual way i.e. a small core group of in-house leadership and technical expertise to manage outsourced service providers. What the company had learnt in collaboration structuring and execution with its platform partners can now be extended and applied in reverse to get the most out of its outsourced collaborators for the new capabilities being added.

"We need CROs so that we can focus on our core and remain lean"

"It's not simply 'outsourcing' – people always underestimate the management time"

The new capabilities also need to be **added in a culturally-sensitive way** as the mindset, priorities and working language of the new people added are usually very different to those of the original team. Such differences need to be recognized and managed to avoid two or even three separate "camps" emerging in the company. Another cultural aspect that needs careful management is **ensuring continued internal ownership and commitment to the partnered projects**. There can be a tendency when the company starts to grow its proprietary projects for internal attention and energy to shift to them at the expense of the partnered projects. At this stage in its evolutionary lifecycle, it still needs the cash flow and the market credibility from its technology/service provision projects – a level internal playing field needs to be maintained.

Key Success Factors for the Hybrid Business Model

- Business & Corporate Development for Partnered & Proprietary Projects
 - Embedded across the organization
- · Synergy between Proprietary Projects and Core Platform
 - Scale economies
 - Focused internal operating mindset
- · Adding New Capabilities
 - For delivering proprietary projects
 - Using a focused and outsourced operating model
- · Collaboration Structuring and Execution
 - With customers/partners
 - With outsourced capability providers
- · Managing Internal Culture
 - Adding new capabilities in a culturally-sensitive way
 - Ensuring continued ownership and commitment to partnered projects

Overall, it can be quite challenging to run a hybrid business, but it is important to realize that this stage in the evolutionary lifecycle is absolutely necessary – there are too many financial and management risks when trying to leap directly from being a technology/service provider to being a focused product company.

"Our hybrid model works, at least for a certain time. It's just a tough and strange one to manage."

"We're a little bit schizophrenic. We're not pure service companies. We have our aspirations for our own programs. Technology collaborations and other services are a means to an end, some of them can be quite large and quite complicated relationships, very financially beneficial. Whereas our investors think 'this is just paying the bills' and the real business is creating our own products. Requires constant management of the expectations of both our investors and our own people."

Stage 4 - Focused Product Company



As the company progresses, one or two of its projects start to exhibit a high probability of reaching the market in the not-too-distant future. Often these are proprietary projects, but they could also be technology/service provision collaborations with substantial future royalty streams. Since the company by this stage is typically already listed on the capital markets, investment analysts begin ascribing the majority of its valuation to these "lead" projects. This in turn drives the company's owners and management team to focus their efforts on the lead projects, investing in new capabilities to ensure their success and if necessary, diverting resources from the other projects when funds are short. Such focus makes it easier to raise new funding from the capital markets to support the investment, creating a virtuous circle that drives the transition to a product company focused in a particular application area. This **pressure to become a product company is inevitable once the lead projects attain high visibility** – the investor community finds it difficult to value a company with small pieces of the pie in many disparate application areas.

"We have a lot of investors who say of our broad portfolio of programs, 'It's too much, I can't get my head around it. My methodology is to look at single compounds, take a deep dive, analyze the market, the competition, and I can put a value on that asset. But 10, 20 or even more assets, I'm not even going to start, it's too much work'."

"Analysts will only focus on one or two of the programs, so they disproportionately skew the company in terms of its valuation around these one or two programs. That's a real challenge for a platform company. You haven't got a label. What are you? Are you an oncology company? Are you a diabetes company? What are you?"

Notwithstanding the pressure to become a focused product company, there are several variants to how this business model can be realized:

- Development Company which develops and registers a portfolio of products within one or two specific therapeutic areas, for marketing through global distribution and selling partners.
- Specialty Marketing Company which markets high margin niche products in one or two specific therapeutic areas with its own salesforce across a selected set of geographic markets.
- 3. Combination of (1) and (2).

Being a *Development Company* makes sense if the company's **platform continues to generate new opportunities**, especially if the targeted therapeutic areas are "mass market" ones with lots of prescribers e.g. diabetes or asthma. In this variant of the focused product company model, the critical success factors revolve around **integrating decision making and project management** in the later stages of the value chain such as clinical development, technical product development, manufacturing scale-up, regulatory strategy, physician positioning and pricing/reimbursement strategy. Since it will not make economic sense to maintain a full suite of in-house operations all of these areas, the company will typically leverage outsourced service providers in many of them e.g. clinical trial monitoring. At the same time, the company will need to contract and work with one or more marketing partners globally for each product. Typically these will be funded co-development arrangements initiated after Phase 2 clinical studies in those cases where the cost of taking the product to regulatory approval is excessive for it to bear alone. The ability to **structure and execute collaborations** thus continues to be a critical success factor, with marketing partners as well as outsourced service providers.

"Over the last five years, of those companies that have brought their first cancer product to market, those that have co-commercialized have very clearly been the most successful. Those that have tried to do it on their own, or who have given it all away, have done the worst."

Being a Specialty Marketing Company makes sense if the targeted therapeutic areas are of a specialist nature that require a comparatively small sales force e.g. oncology or an orphan disease, especially if the company's platform is becoming commoditized and is less likely to continue reliably generating a slew of unique new opportunities. In this variant of the focused product company model, the same capabilities in decision making and project management integration across development and strategic marketing mentioned above will again be a critical success factor. Furthermore, the ability to set up and manage local marketing and sales units will also be essential to success. A typical approach when initiating this strategy is to in-license or acquire a portfolio of products already on the market in the target therapeutic areas so as to build up prescriber and payer relationships and capture market insights in anticipation of the lead projects in the pipeline. In this case, the sales and marketing capabilities need to be built very early on. Nevertheless, since the company is unlikely to have a local presence in every market, marketing partners will still be required in certain geographies. Collaboration structuring and execution will thus continue to be important for these marketing partners as well as for outsourced service providers.

"Adding Marketing & Sales creates completely different classes of problems to overcome, as well as a major enforced culture change. It's a much bigger management shift than sticking with managing technical/scientific issues."

| Key Success Factors for a Focused Product Company | |
|---|---|
| Development Company | Specialty Marketing Company |
| Platform Continues to Generate New Opportunities | Setting Up and Managing Local Marketing & Sales units |
| Collaboration Structuring & Execution Marketing Partners Outsourced Service Providers | Collaboration Structuring & Execution Marketing Partners Outsourced Service Providers |
| Integrating Decision Making & Project Management across the value chain | Integrating Decision Making & Project Management across the value chain |

A Combination of Development and Specialty Marketing is not impossible of course, and some companies do seem to go down this route. But the underlying cultures and business values of the two variants are quite different, with the former being very product/platform-oriented and the latter being very physician/market-oriented. Hence this approach is probably difficult to carry off without one variant being the major driving force. For example, it can make sense to be primarily a development company, with a salesforce in say one or two large markets (or perhaps just in the home market) as a way of capturing market intelligence and increasing profitability without taking on undue risk or diluting the company's culture. Conversely, one could be primarily a specialty marketing company with a focused development arm to exploit know-how and increase company valuation.

An important point to note is that there will be companies successfully operating the above business models who did *not* arrive there from a platform history. The newly-transitioned platform company will be judged against a different set of benchmarks i.e. other focused product companies – not just by investors and prospective collaborators, but also by regulators, payers, physicians and patient lobbying groups.

Forever Platform?

One question that is often asked is whether the company could remain a technology provider or hybrid company forever, instead of taking on the cost and risk of adding new capabilities and in essence gambling on the success of the lead projects. An oft-quoted example is that of ARM Holdings plc ("ARM"), the dominant global player in mobile technology for smartphones and tablets. ARM focuses on creating and licensing intellectual property – its chip designs are used in almost every mobile phone and tablet but it does not manufacture any of them itself and neither does it have an end-user sales force. Nevertheless, it has a market capitalization of nearly US\$ 20 billion with an operating income in excess of 20% of sales. However one key difference is that the application

market for ARM is very specific and clearly defined whereas a bioscience platform company with 10 to 20 separate projects for applications in say diabetes, alzheimer's, pain, arthritis, asthma and cancer is much harder for the investor community to get their arms around since the underlying demand drivers are very different. And so the valuation focus always narrows in on the lead projects closest to market.

To overcome this valuation challenge, one possibility might be to create spin-off entities for each project (or group of closely-related projects) once a certain stage in development in reached, with separate investors for each entity according to their preferences for the different end-user markets, risks, returns and timelines. Each such project-centric entity would initially operate a virtual model, contracting in the relevant management and technical capabilities from the "mother" company. Over time, the successful project-centric entities would evolve into listed focused product companies of their own or be acquired by trade buyers. While the mother company would continue to use its platform to generate and nurture new opportunities in a diverse range of areas.

"If someone says to me I love that asset, I want to see it in a separate vehicle, I want to put in tens of millions to give it a chance of working, ultimately that model stands a better chance of success because they will always look at that asset differently as a separate entity than an asset within a hybrid organization."

"A possibility might be single asset joint venture spinout, take it out, fund it elsewhere, we put in half the stake, the rest from outside, but effectively it's still our drug."

Concluding Remarks

Platform companies are a critical component of the bioscience ecosystem. They can add tremendous value across a range of therapeutic areas. And they provide both the critical mass of know-how, and the incubation time, for certain innovative technologies to be fully developed and exploited. Not everything being spun out of academia or an existing business is best exploited via the virtual project-centric model that many venture capital firms seem to be favoring these days. And in any case, value can often be maximized via a diversity of applications rather than focusing too early on just one.

Platform companies are also a good base to spawn new sustainable product companies who can deliver increased innovation and improved outcomes across the healthcare system. The market pull for the successful platform companies to evolve into focused product companies is almost inevitable despite the management challenges involved. Nevertheless, there may be some creative mechanisms for having the best of both worlds, by spinning off single asset entities for commercialization and marketing, while retaining the platform core in the original mother company.