What Constitutes a Sellable Asset?

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Torreya Insights Professional Services

Innovation in life sciences continues to be synonymous with biotechnology start-ups, and commercialization or marketing capabilities with large pharma. Although there are several exceptions to this rule, most biotechs are truly Research and Development (R&D) organizations focusing on asset-specific value construction. Those who have made the strategic decision to remain independent and commercialize their asset do so at significant risk, especially when developing a product for a large patient population. The increased risk-to-reward ratio for biotechs is further compounded by the fact that funding is always finite and contingent upon clinical advancement in a world where failure is the norm, rather than the exception. For a biotech company to become a commercial organization and shift its focus away from R&D to clinical and marketing operations constitutes a cultural evolution, nothing short of a change to its very fabric.

Many early to mid-stage life sciences companies in 2015 experienced an unprecedented number of successful Initial Public Offerings and Merger and Acquisition (M&A) transactions. This trend, combined with reductions in internal R&D programs at pharma and heightened interest in in-licensing new drug candidates, emphasizes the fact that asset partnering for biotech companies remains a viable value-creation option. The majority of licensing deals still occur either during preclinical development or in Phase 2 (Thomson Reuters, 2015 Life Sciences Deal Trends; EP Vantage, February 11, 2014). With respect to the former, executing a licensing agreement early in development may provide much needed capital to a growing biotech company. However, it may also delay fully recognizing the value of an asset, as the sense of urgency for asset development may not be shared by its buy-side partner. Generally, value is directly proportional to asset maturity and can first be truly recognized when an asset reaches Proof-of-Concept (POC; Phase 2, but in oncological and orphan indications this could be as early as Phase 1b). By observation, the ultimate value is only recognized when a drug becomes approved, reimbursed, prescribed by physicians and used by patients.

Pharmaceutical companies face several challenges as they consider pipeline expansion, including whether to invest in internal vs external innovation. For instance, a program that was brought in from the outside may effectively de-prioritize one that has been pursued internally. Currently, more than 75% of late stage assets in big pharma's pipelines come from external sources, i.e. those very biotech companies looking for a viable partner, a clear trend in R&D externalization. Other pressures faced by pharma that put additional constraints on what should be considered and what is achievable within the

time frame of a strategic business plan (i.e.: generally 5 years) include world-wide price controls, intellectual property violations and growing global markets. The era of block-buster, multi-billion dollar pharmaceuticals is over. The goal now is to become either “best in class”, or “only in class” (especially for orphan indications). Furthermore it has become obvious that the healthcare system is moving towards a patient-centric, value-based approach and pharma is experiencing increasing pressure from regulators, payers, providers and patients. It is thus pertinent to select and then develop a product that has the biggest potential to address these challenges and the cleanest path to market.

Most pharma business development groups are flooded with incoming requests, reviewing several hundred opportunities annually. Prioritization is thus paramount. From a biotech perspective, it is important to understand what a potential pharma partner may be looking for, how their existing pipeline can be complemented, whether they are active in deal-making, or tend to simply “shop around” to gain a deeper understanding of the competitive landscape. Another critical factor is to determine who might be the internal “spokesperson” (generally a business development professional or head of an R&D group) for discussions between the parties.

Just like a resume needs to stand out in order to land a job interview, so does the data supporting an asset in order to have pharma agree to that first in-person meeting with key stakeholders. During the initial asset assessment stages, pharma looks for compelling animal data in a model that closely recapitulates human disease state, human tissue or genetic data, transcription expression-related data, and, most importantly, differentiation from standard of care should a program move forward successfully in the clinic. It is expected that a biotech will be able to answer the following fundamental questions:

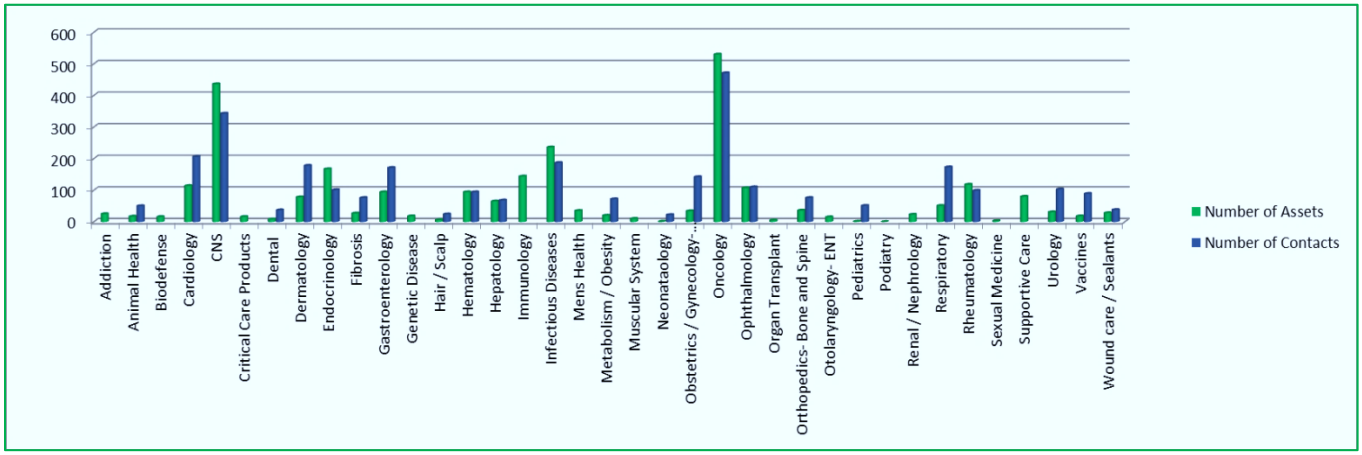
- Is my company offering disruptive technology or platform?
- Is the biology novel? What is the mechanism of action?
- What is the unmet medical need? What are the current treatment options and how satisfied is the target patient population with the current treatment?
- What benefits does the asset provide?
- How does it stack up against the competition (efficacy, safety, dosing, and cost)?
- What is the future potential target audience or customer for this product?
- Even if at early stage of development, can an insight be offered into clinical development, commercial strategy and payer’s perspective?

In our experience, the fastest way to derail any partnering discussions is to use poor animal models to demonstrate preclinical proof of principle, clinical endpoints that simply are not well thought out, a patient population that is poorly defined and selected for a trial, lack of intellectual property strategy or protection, and grandiose, unsupported claims of future market potential.

Addressing some of these points may seem like a daunting task, but groups like ours at Torreya Insights have been formed precisely in order to assist life science companies in navigating the murky waters of asset acquisition, review and partnering. We advise on how to communicate an opportunity in a clear,

concise and compelling fashion. We help to leverage key attributes of an asset in conversations with potential co-development or strategic partners. We provide more hands on deck for all business development functions and activities. We track more than 30,000 individuals in our industry and have personal connections with many of them. This enables us to identify key contacts that could become internal champions for our Client's programs and later deal negotiation. Once these connections have been re-established and interest is expressed, we can then leverage the strengths of an asset by analyzing the competitive landscape, market potential, as well as current and future financial value (taking into account the ever-changing reimbursement and henceforth marketing landscape).

To provide an illustrative example, the graph below shows the number of assets and individual contacts (not companies) per therapeutic category as listed currently in our database of Available Pharmaceutical Assets, AvaRx (www.avarx.com) and our proprietary Customer Relationship Management database, Licensing Dynamics. It is not surprising that the number of assets and individual contacts representing those assets is not uniformly distributed across all therapeutic areas, but correlates with the recently-reported data on M&A and licensing events (Thomson Reuters, 2015 Life Sciences Deal Trends). Our internal assessment is on point with the fact that deal making in 2015 was mainly driven by assets in the area of Oncology, Central Nervous System (CNS) and Infectious diseases. Additionally, judging by the number of assets and interested parties developing treatments that are yet to find a home in the form of a partner or buyer, the same therapeutic areas are likely to continue to drive deal making in 2016 and beyond.



Business development experts at both biotech and pharma companies are faced with a daunting task as competition increases for the best biology, platform technology and molecules. The quality and efficiency of a partnering process can be augmented with dedicated tools, expertise and thorough analysis.

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This is a detailed check list of due diligence items that a pharma partner is generally looking for:

General information

- Technology
- Candidate/Program
- Developmental stage
- Final product formulation/Target Product Profile
- Pedigree
- Biological rationale
- Mechanism of action
- Primary disease target
- Secondary disease target
- Strategic fit

IP Landscape

- Patents
- Exclusivity/Third party involvement
- Freedom to Operate
- Lifecycle

Clinical/Regulatory

- Manufacturing/COGS/Formulation
- Clinical trial design (primary and secondary endpoints)
- Size of the population/trial
- Patient enrollment rate – length of the trial
- Pricing
- Sales potential
- Reimbursement
- Post-approval marketing

Marketing

- Cost of marketing post approval
- Sales force
- If an asset has been shopped around, it may be difficult to get a marketing partner,
  clever positioning may be needed

Market/Competitive Intelligence

- Size
- Competitive Landscape
- Market need
  - Number of Patients
  - NCE
  - Orphan exclusivity
- Clinical endpoints
- Efficacy
- Safety
- Time to POC
- Time to Approval/Commercialization
- Regulatory Incentives

Financial Analysis

- NPV
- Target population/Pricing/Profit Margin Royalty
- Investment to POC
- Investment to Market
- Third party support opportunities
- Alternatives to Partnering

The below cases studies are examples of the typical projects that Torreya Insights conducts:

Case Study: Asset Search

Situation Overview
- Midsize pharmaceutical company with no approved assets hired Torreya Insights to search for available assets for their pipeline
- Areas of interest included hematology, cardiovascular and autoimmune diseases. The Client was interested in assets from Preclinical to Phase 3 stage of development

Activities
- Torreya Insights team worked closely with the Client to better understand their strategy
- We held weekly teleconferences to discuss the status of the project and to tweak the search
- We screened the universe of potential targets and generated summary data of assets fitting Client's criteria

Results
- Torreya Insights initially presented nearly 120 asset ideas
- Through numerous discussions, 30 assets of interest were selected
- The team then created one-page profiles for selected assets, which included biological target, mechanism of action, clinical stage and indication, just to name a few of the criteria.
- Torreya Insights and the Client discussed all the assets and categorized them by level of interest
- The former Client is currently assessing internally how to best pursue selected ideas.

Case Study: Market Study

Situation Overview
- Torreya Insights was engaged by a global pharma company with a market cap >$5b developing a portfolio of acute care hospital products utilizing a proprietary technology that enhances efficacy and safety of known molecules via the 505(b)(2) regulatory pathway
- The Client has made a strategic decision to raise funds to complete clinical development of these assets, rather than continue to invest internal resources, due to pipeline reprioritization and the need to keep R&D spend in check.
- The Client has requested to anonymously evaluate the potential for a financial transaction to fund the Phase 3 development of the acute care products.

Activities
- The team at Torreya Insights and the Client held numerous discussions to gain a better understanding of the value proposition of the assets, the market and treatment potential in a hospital setting.
- With Client's input we developed an interview questionnaire and identified selective investors for outreach.
- We conducted the interviews, collated raw findings, and analyzed all responses

Results
- The results of interviews with various financing sources indicated that project financing of the portfolio is both feasible and achievable.
- Together with this data and the results of the most recent Phase 2 trial the former Client is considering spinning out the assets under a New Co.

Case Study: Financial Valuation

Situation Overview
- Torreya Insights was engaged by a Client with a Phase 2 asset for the potential treatment of a neurodegenerative disease
- The Client needed a 3rd party objective analysis of the asset value, as well as its competitive positioning

Activities
- Torreya Insights and the Client held numerous discussions to gain a better understanding of the asset, the current marketplace and treatment potential
- Our tem conducted in-house research to create assumptions and generated a comprehensive valuation model, which included detailed revenue and costs forecasts
- Torreya Insights also researched recent comparable transactions and companies with similar products and/or go to market strategy in order to affix value based on the activities in the market

Results
- NPV and rNPV sensitivity analyses were generated and "raw" excel model was provided to the Client
- White paper was written to accompany the valuation, which outlined the methodology used and provided market and competitive positioning in the context of the results
- The former Client is currently completing a second Phase 2 trial and is preparing for a transaction.