

Leveraging the IBE to Fuel Pipeline Growth at a Growing Biopharmaceutical Company

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Growing biotechnology and bio-pharmaceutical companies require a sustained flow of innovative product candidates to fuel corporate growth. These candidates come primarily from the Research organizations within these companies that are charged with understanding the biological basis for unmet medical need and designing therapeutic agents that can effectively modulate that biology to provide improved patient outcomes.

In such companies, a major impediment to the flow of innovative product candidates into clinical development is the lack of understanding by research scientists within these organizations as to the components of a compelling commercial product concept. Almost universally, the scientists who are recruited to these organizations have graduated from post-doctoral training programs at academic institutions that are focused on basic research and technology development, and not on the discovery and development of novel therapeutic products. As such, these scientists develop highly sophisticated understanding of fundamental biological concepts and basic research techniques but are never exposed to the other disciplines that are required to define a compelling product idea. These other disciplines include an understanding of how to define product commercial potential, including a crisp understanding of the unmet medical need, current treatment options, competitive landscape, and the impact of health economics on drug access and pricing. They are also generally unaware of the process of assessing freedom to operate and securing intellectual property rights, and of the key steps in the discovery and development process required to translate an idea into a commercially-viable product.

The Institute for Biomedical Entrepreneurship was established to enhance the flow of biomedical innovations from research to commercialization. In particular, the IBE provides educational programs to allow researchers and entrepreneurs to develop an understanding of the execution processes involved in developing ideas into commercial success.

At Momenta Pharmaceuticals, we have leveraged the IBE to expose select scientists and other staff members to a formal training in how to develop innovative therapeutic product concepts that represent compelling opportunities for Momenta's internal pipeline.

Momenta was founded in 2001 based on technology discovered and developed at the Massachusetts Institute of Technology (MIT) for the precise sequencing of complex sugar molecules. Momenta is a leader in the analysis, characterization, and design of complex pharmaceutical products. We are applying our innovative technology to the development of generic versions of complex drugs, biosimilar and potentially interchangeable biologics, and to the discovery and development of novel therapeutics for autoimmune indications. Momenta has launched two complex generic products in the US market and has multiple product candidates in development in two key areas, biosimilars and novel autoimmune disease therapeutics. As a growing company, MNTA needs further innovative product opportunities. The company has a Research staff of over 50 scientists, with more than half

of them with PhD's or advanced degrees and invests approximately \$15M annually in novel drug discovery research.

From November 2016 to March 2018, the company has supported the participation of 9 scientists in the IBE Educational Program, at a cost of \$45,000. These scientists were selected based on their scientific subject matter expertise in the disease areas of interest, their aptitude for identifying potential drug targets that might underlie unmet medical need, and their potential to develop as drug discovery project leaders. In other words, these are the scientists with potential to develop as what we refer to as "Product Champions", the inventor and visionary lead for a novel drug. Each scientist takes to the IBE program a specific product concept relevant to their internal work. In general, the scientist has developed the product idea based on their knowledge of a biology, and drug target, associated with the disease process and a proposal that modulating this drug target will alter disease cause and address unmet medical need in the targeted patient population. These proposals are typical of the state of maturity and depth of thinking of most novel product concepts in Biotechnology and Pharmaceutical companies. The scientist, or product champion, is intensely focused on the biological rationale and supporting experimental data for their drug target but is largely unaware of what it will take to advance the idea to a commercially viable product opportunity, and how to secure endorsement by management and build a team to advance the product concept through discovery and development.

Each product idea is appropriately anonymized to avoid disclosure of any proprietary company information. Actually, we have found that anonymization of the product concept does not deduct from the educational experience at all, as the concepts being developed as part of the IBE training experience are not focused on a deep assessment of biological rationale or specific disease indications, rather they are focused on the principles of assessing commercial potential, building the product development plan, securing intellectual property and funding, developing the presentation skills to describe the opportunity, and building the team who will execute on the product opportunity.

In general, 2-3 scientists attend each program to ensure the opportunity to not only advance their understanding of biomedical entrepreneurship, but also to facilitate relationship building within the Momenta team. This also ensures a steady flow of maturing product ideas that can be further developed within Momenta.

Over the last 18 months, a total of 8 product concepts have been assessed within the IBE course setting, and multiple others have been assessed within MNTA using the principles learned at the course. The scientists who have participated in the IBE training program have been role models for their colleagues back at Momenta and have transferred many of their learnings to others. Overall, the standard of product conceptualization and the depth of rigor in assessing potentially compelling product opportunities has significantly increased. In this way, the core group of IBE course participants have been able to transmit their knowledge to others with MNTA and expand the number of opportunities that can be rigorously assessed.

From these efforts, 6 novel product concepts have been presented to Momenta executive management and have been supported for investment within the company. Each of these product concepts has been well-developed, with a clear assessment of the unmet medical need, the scientific and therapeutic rationale for the novel product, a draft target product profile that defines the key performance attributes required for success of the product, and a detailed business case for the investment. These product opportunities represent significant commercial product opportunities for MNTA, with potential revenues ranging from approximately \$200M to greater than \$1Bn annually. Importantly, each product proposal also contains a detailed drug discovery and early development plan that advances the product concept to a clinical proof of concept, with clear timelines and investment required to achieve key milestones in the drug discovery and development process. In general, the product concept requires 2-3 years investment in discovery research and preclinical development to prepare a novel product candidate for human clinical trials, and 2-3 years of human clinical testing to achieve the key clinical proof-of-concept that would demonstrate the therapeutic potential and commercial value of the product opportunity. Detailed plans of the drug discovery, preclinical development, drug manufacturing and clinical trial stages are included, with a detailed budget and key decision milestones. Each plan is similar in content and detail to that which would be presented for significant venture capital investment. Essentially the company is operating as a private investor for each program.

The investment in training and networking within the IBE has been well worth it, with the quantity and quality of novel product concepts improving significantly over the past two years. The enhanced understanding of product commercialization principles has also resulted in a more highly engaged research team, and an organization better equipped to compete in the complex and fast-paced biotechnology ecosystem. This will ensure MNTA has a continuous flow of innovative and valuable product opportunities to fuel the future pipeline and growth of the company.