IDENTIFYING PRODUCTS FOR DRUG DEVELOPMENT PROGRAMS

THE GLOBAL 505(B)(2) EXPERTS
Camargo
PHARMACEUTICAL SERVICES

Knowing which products have verified profit potential before development begins helps defend their value to investors and reduces the risk of costly marketing errors.

Introduction

Assume a generic drug company, let's call them AB Generics, Inc., always targets the introduction of its generic drugs when the reference product comes off patent. AB's executive team has looked ahead and seen the generic cliff — few products coming off patent after 2016 — and is looking for alternative revenue sources. They know that the 505(b)(2) NDA pathway can be used to make changes to reference products, but a major question is which products offer the greatest profit potential and least risk.

For innovative pharmaceutical companies, opportunities for new pharmaceutical products exist within the products, patents, technologies and intellectual properties they already own or control. But for generic companies, there is usually no basis other than their manufacturing capabilities upon which to select a 505(b)(2) candidate.

AB Generics, Inc. has turned to the 505(b)(2) development leader, Camargo, to identify and assist in the development of its 505(b)(2) candidate. Camargo Pharmaceutical Services offers a clear process to identify products that have documented market differentiation.

Selection Criteria

Our first step in choosing a product to develop is an assessment of the client's business profile — what the client brings to the table as a starting point charted against its specific goals and business needs. The criteria that are germane to this are many and may include everything from existing products or APIs the client holds, to market areas it wishes to enter or targeted populations it wishes to address.

This narrowing of focus is essential to getting beyond broad and unmanageable marketing segments, such as indications or diseases, and down to highly specific definitions of unique products and discrete market segments. The research effort spans the scientific, medical, manufacturing and commercial space because all must work in tandem to create a product that can be defined, differentiated from what else is available and effectively marketed at a profit.

How the Process Works

Once the market segment has been narrowed, the next step is to identify, assess and rank each unmet medical need in terms of product viability. Considerations to establish product viability cover a lot of ground and include a broad range of potential research and reporting activities, including:

Market segmentation

- Disease overview
- Epidemiology
- Current standards of care
- Needs assessment

Product analysis

- SWOT analysis
- Critical success factors
- Strategic objectives
- HEOR
- Drug development programs
- Positioning and target product profile (TPP) development
- Non-clinical programs

Production analysis

- Regulatory considerations
- CMC considerations
- 505(b)(2) candidate assessment

Marketing analysis

- Market and competitor assessment
- Detailed commercialization plan
- Distribution assessment and plan
- Key messaging

Investor analysis

- Financial analysis
- Partner targets
- Pipeline analysis
- Compendium assessment and plan

Profit potential

- Payer reimbursement plan
- Pricing development

Case History

Pentamidine

Although Pentamidine had been approved for sleeping sickness, research uncovered a new indication as a treatment and prophylaxis for AIDS-related Pneumocystis pneumonia (PCP). Because Pentamidine addressed an orphan indication and was reformulated through 505(b)(2) in an aerosolized dosage form that reduced side effects, patents granting market exclusivity were issued. The company was sold for more than $1 billion.

As an example, a European pharmaceutical company sought Camargo's assistance with entering the U.S. market via a 505(b)(2) NDA filing for its drug candidates. The products had been developed with proprietary drug delivery technology and its plan included transfer of both manufacturing and clinical studies to U.S. service providers.

Before this company made decisions about manufacture or clinical development, however, Camargo conducted a comprehensive feasibility assessment to achieve in-depth understanding of critical factors affecting the U.S. market potential, including need in the therapeutic area, the competitive environment, payer acceptance in the drug treatment category and regulatory considerations.

Armed with this knowledge, the client was able to plan its market-entry strategy and prioritize the products with the highest potential market value. Camargo's detailed timeline and cost analyses, both of which were key elements of the assessment, identified drug development time frames and costs before embarking on the projects.

Defending the Value

If the object is to maximize investment from outside entities, research studies serve the purpose of defending the value of the product to investors by proving its validity.

The 505(b)(2) development pathway provides a lower risk pathway to obtaining marketing approval in the United States. Because drugs approved under 505(b)(2) can rely in part on data from existing reference drugs, they have known safety profiles and efficacy endpoints and can achieve FDA approval with only a fraction of the number of required clinical trials and at a much lower cost.

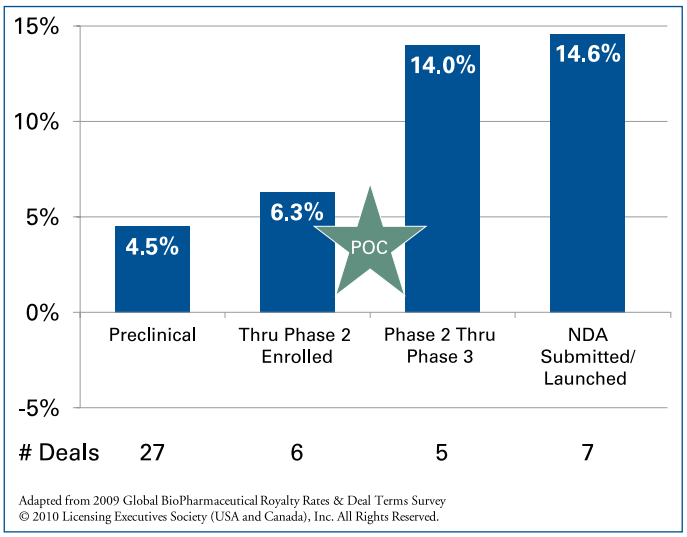
The pathway also yields products that will be of interest to different types of partners and investors, depending on when they are approached. There are four distinct "inflection points" that impact the value of a 505(b)(2) drug candidate in clinical development:

- Completion of the pre-IND meeting with FDA
- Proof of concept (PoC) achieved in man
- Filing of the New Drug Application (NDA)
- FDA approval of the NDA

As you can see from the chart below, the best time to approach investors in terms of the royalty rate is as soon as the proof of concept has been established in man, when the average royalty rate more than doubles. This is the moment when evidence in hand is most valuable.

Elements of Deal Value – Royalty Rate

Average Royalty at State of Development



Adapted from 2009 Global BioPharmaceutical Royalty Rates & Deal Terms Survey
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Case History

Amphotericin B

Amphotericin B is a polyene antifungal drug, often used intravenously for systemic fungal infections. Administered orally or intravenously, it is well known for its severe and potentially lethal side effects, including high fever, shaking chills, hypotension, vomiting, headache, dyspnea and tachypnea. Reformulated as a liposome through 505(b)(2), the new product is difficult to genericise and dramatically reduces side effects and improves efficacy.

Maximizing Investment

Investors lend most credence to those propositions where it is obvious that due diligence has been performed. To maximize investment, it's important to nail down your defense of the value in each area before you approach investors.

Clinical development

- Project management by established experts in:
  - Technical assessment and product formulation
  - Clinical trial management
  - Regulatory strategy and interaction with FDA

Intellectual property

- Types and sufficiency of product patents
- U.S. marketing exclusivity
- Life cycle management strategy

Product revenue forecasts

- Product differentiation and market positioning
- Pricing and discounting strategies by payer class
  - Managed care
  - CMS programs
- Compendia positioning
- Distribution strategies
  - Third-party logistics, wholesalers and retail chains
- Sales and marketing strategies
  - Targeted physician audience (breadth and depth)
  - Direct to consumer promotion
  - Social media, other non-traditional promotion

Product and promotional cost forecasts

- Manufacturing strategy
  - Product unit cost expectations
  - Licensed technology royalties
  - Redundancy
- Product liability/insurance strategy
  - Assessment of insurance industry capacity
  - Insurance costs and structures
- Sales and marketing infrastructure
  - Size and cost of sales force
  - Cost of A&P programs
  - Effective tax rate optimization

Narrowing a Universe of Possibilities Down to One

Many of us are in the pharmaceutical business because we have a drive to help alleviate the world's suffering. But given all the world's diseases and medical conditions and their stages, all the organs and systems of the human body, all the different human population groups, all the possible drug delivery platforms, and all the compounds known to have an effect in humans, the concept of choosing a single pathway to address is almost inconceivable.

Case History

Glycopyrrolate

Originally approved for reducing gastric and other secretions intravenously before surgery as well as for use during anesthesia and intubation, a Glycopyrrolate tablet formulation is approved for peptic ulcers. Research into new indications and formulations led to the development under 505(b)(2) of a liquid formulation for cerebral palsy patients to reduce drooling. This new formulation was granted orphan drug status for new indications. It is currently being developed as a long-acting muscarinic antagonist (LAMA) for COPD patients in MDI, DPI and nebulized dosage form. The estimated market potential for these new indications exceeds $1 billion.

When this decision is additionally complicated by factors that affect viability in the marketplace, including the competitive position; costs of manufacturing, marketing and distribution; and available patent protection, among others, only a deliberative, step-by-step approach by a team of experts can narrow the universe of possibilities down to a manageable number of viable options. And only expert analysis can choose the best option from an array of possibilities.

Many companies perform studies; few have the market expertise to synthesize the results into a viable 505(b)(2) development plan. Camargo, the leader in 505(b)(2) drug development, has developed a detailed process for choosing appropriate development candidates. We are eager to put this knowledge to work for you.

About Camargo Pharmaceutical Services

Camargo Pharmaceutical Services is the most experienced global strategist providing comprehensive drug development services specialized for the 505(b)(2) approval pathway and global equivalent processes. By assessing the scientific, medical, regulatory and commercial viability of product development opportunities, Camargo systematically builds and executes robust development plans that align with business strategies and ensure FDA buy-in every step of the way. Routinely holding three to six pre-IND meetings a month, Camargo works with product developers across more than 25 countries.

Worldwide Reach

In more than 25 countries across North America and Europe, to India, Israel and China, Camargo's regulatory know-how and customized clinical solutions are as varied as our clients' global drug development needs.

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