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Review Article

Recent trends in specialty pharma business model

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ABSTRACT

The recent rise of specialty pharma is attributed to its flexible, versatile, and open business model while the traditional big pharma is facing a challenging time with patent cliff, generic threat, and low research and development (R&D) productivity. These multinational pharmaceutical companies, facing a difficult time, have been systematically externalizing R&D and some even establish their own corporate venture capital so as to diversify with more shots on goal, with the hope of achieving a higher success rate in their compound pipeline. Biologics and clinical Phase II proof-of-concept (POC) compounds are the preferred licensing and collaboration targets. Biologics enjoys a high success rate with a low generic biosimilar threat, while the need is high for clinical Phase II POC compounds, due to its high attrition/low success rate. Repurposing of big pharma leftover compounds is a popular strategy but with limitations. Most old compounds come with baggage either in lackluster clinical performance or short in patent life. Orphan drugs is another area which has gained popularity in recent years. The shorter and less costly regulatory pathway provides incentives, especially for smaller specialty pharma. However, clinical studies on orphan drugs require a large network of clinical operations in many countries in order to recruit enough patients. Big pharma is also working on orphan drugs starting with a small indication, with the hope of expanding the indication into a blockbuster status. Specialty medicine, including orphan drugs, has become the growth engine in the pharmaceutical industry worldwide. Big pharma is also keen on in-licensing technology or projects from specialty pharma to extend product life cycles, in order to protect their blockbuster drug franchises. Ample opportunities exist for smaller players, even in the emerging countries, to collaborate with multinational pharmaceutical companies provided that the technology platforms or specialty medicinal products are what the big pharma wants. The understanding of intellectual properties and international drug regulations are the key for specialty pharma to have a workable strategy for product registration worldwide.

1. Defining specialty pharma

"What is specialty pharma?" many people question me. Is it in-licensing specialists? Niche marketers? Drug delivery firms? Will generic drug manufacturers be included? How about biotech companies that move into drug development? Well, depending on whom you ask, they are all of the above [1]. Wall Street's definition is a catch-all, and includes drug delivery, biotech, and generic firms. For instance, Morgan

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Stanley coverage of specialty pharma includes: generic companies like Teva, Mylan, and Actavis; over the counter companies like Perrigo and Warner Chilcott; development centric companies like Allergan, Forest, and Valeant (previously Biovail); drug delivery companies like Alkermes; and animal healthcare company like Zoetis (formerly Pfizer animal healthcare division) [2]. As the popularity of the specialty pharma business model has expanded, so has its scope. Today, many use the term "specialty pharma" interchangeably with development-centric pharmaceutical or biopharmaceutical companies. Others apply it to companies developing generics, reformulating existing drugs, or targeting niche markets. Some others more often use the term to identify companies that are "not biotech not big pharma", where big pharma is defined as large-cap pharmaceutical companies. In other words, "specialty pharma" has become such a broad term that it covers just about everything except the big pharmaceutical companies and medical device and diagnostic makers.

2. Specialty pharma business model

After defining specialty pharma is inclusive of all healthcare-related firms that are neither big pharma houses nor medical device and diagnostic makers, the next question is "What is specialty pharma's business model and why it gains so much popularity nowadays?" In order to answer these questions, it is necessary to compare and contrast big pharma with specialty pharma. Big pharma typically follows a vertically integrated business model. It means that big pharma carries out the work from the beginning to the end on a worldwide scale including discovery research, drug synthesis, preclinical research, clinical development, regulatory work, scale up and manufacturing, and worldwide distribution, sales, and marketing. Moreover, big pharma has more breadth by working in four to six therapeutic areas. These may include cardiovascular, antimetabolite (such as antidiabetics), central nervous system (CNS), oncology, and infectious diseases. Specialty pharma, by contrast, acquires drugs from academia, research institutions, or other companies, and seeks to commercialize them in new markets. It selects a core of activities while relying on a network of contract research organizations (CRO), contract manufacturing organizations (CMO), and other preferred pharma partners to accomplish its commercial goal. Specialty pharma focuses most of its efforts on one or two therapeutic areas with specified physician populations. These specialized non-primary care physicians can be managed with a smaller sales force. Specialty pharma often has a small research and development (R&D) organization and contracts out animal and human tastings to CRO and its manufacturing to CMO. It is a business model that has been prevalent in the last years as venture investors seek to find a way around the long, expensive, and risky drug discovery process. The attributes of specialty pharma are "small", "niche", "agile", and "focused" that are popular with Wall Street. The specialty pharma business model is compared with that of traditional big pharma in Table 1.

3. Four categories of specialty pharma

The business model of specialty pharma can be divided into four categories (Fig. 1). Some companies are experts in the search of compounds for in-licensing; some focus on marketing specialty medicines to a limited number of clients; some started as a generic company; and some with a specific delivery technology knowhow. The world largest generic company, Teva ("Nature" in Hebrew) is on the list of specialty pharma. In fact, the largest product of Teva is a specialty brand medicine, glatiramer (Copaxone), which constitutes nearly 50% of profit and 20% of revenue which is $20.3 billion in 2012 [3]. Glatiramer, the most popular multiple sclerosis drug, was originally discovered by three professors at the Weizmann Institute of Science in Israel. It is a random polymer (6.4 kD) composed of four amino acids (namely glutamic acid, lysine, alanine, and tyrosine) that are found in myelin basic protein [4]. Administration of glatiramer shifts the population of T cells from pro-inflammatory Th1 cells to regulatory Th2 cells that suppress the inflammatory response. Given its resemblance to myelin basic protein, glatiramer may have acted as a decoy, diverting the autoimmune responses against myelin. Glatiramer was approved in 1996 in the US and in 2000 in the EU. It is currently marketed in 49 countries.

4. Story of Teva and generic glatiramer

Teva's glatiramer patent expires in 2014 and 2015 in the US and Europe, respectively. Two generic glatiramer products from Sandoz/Momenta Pharmaceuticals and Mylan/Natco Pharma partnerships that Teva assumes in a news release for the 2015 budget, will launch in September 2015. Mylan, the first filer of generic glatiramer, is in litigation with Teva which has filed two citizen petitions trying to stop the launch of generic glatiramer. In the meantime, Teva is developing a sustained injection of glatiramer reducing the dosing frequency from 20 mg/day to 40 mg three times/week, which was approved by the Food and Drug Administration (FDA) in January 2014 [5]. The new formulation with a different strength and dose regimen would not be subject to generic competition. Once patients convert, it would be hard for insurers to force them to use a generic that would require them to go back to daily injections. However, if Teva's own history is of any lesson, Teva is unlikely to prevail, albeit with a changed role from a generic aggressor to a brand defender. While the competition in specialty pharma may be fierce, Teva will still be working hard in specialty drugs. It anticipates four specialty product approvals and five submissions next year "which we believe will improve treatment options for patients and add value for all of our stakeholders," Teva CEO Vigodman said [6].

5. Defining specialty medicine

The reader now may understand what specialty pharma is but wonder what specialty medicine is. Specialty medicines are those indicated for rare conditions that affect a small number