Insight brief

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U.S. Market Access:
Key learnings from a product launch success story

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Executive summary

The U.S. has been among the most sought-after pharmaceutical markets in the world for decades. But accessing this enormous market is not easy. Not only must entrants gain marketing approval from the Food and Drug Administration, they must also ensure access to patients by gaining prescriber and payer acceptance. Meeting the combined challenges of marketing approval, payer acceptance, assured reimbursement and access to patients is the result of a studied and deliberate approach to product development and launch. This studied approach is particularly important in rare disease product categories with low numbers of patients, high development costs and managed care plans focused on controlling spend.

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Introduction

The United States (U.S.) has been among the largest pharmaceutical markets in the world for decades. The Affordable Care Act (Obamacare) added significant new opportunities by expanding healthcare coverage to millions of individuals who could previously not obtain or could not afford health insurance. The dramatic jump in the number of covered lives increases the potential benefits for companies launching new therapies in the U.S.

Even as the U.S. expands its pharma market, it continues to maintain some of the highest barriers to entry, particularly for smaller biopharma companies. A few years ago, product marketing approval from the Food and Drug Administration (FDA) was the final step in an arduous biopharma research and development program. Today, FDA marketing approval is only another mandatory milestone in a thoroughly researched and well-designed product launch. A thoughtful and deliberate approach to a product launch is particularly important in the rare disease and orphan drug area in which patient numbers are low, development costs are high and managed care plans are focused on spend.

The ultimate goal is commercial acceptance in a market that is directed largely by payers and benefit managers who ultimately control patient access to new therapies. Meeting the challenges of payer acceptance, assured reimbursement and reasonable patient access requires a studied and considered product launch strategy that begins long before a new product is approved and continues long after distribution has begun. This insight brief will explore the structured product launch of Hemangeol®, a novel, orphan treatment for infantile hemangioma. The 120-day integrated launch program that we will discuss was designed and implemented in partnership with Pierre Fabre Pharmaceuticals, Inc, a European biopharma with limited experience in the U.S. market, and industry services provider Quintiles to successfully introduce a new agent into this complex, dynamic and vital marketplace.

A key global market

The United States is among the largest therapeutic marketplaces in the world, a commercial target that no serious biopharma firm can afford to ignore. Overcoming significant barriers to entry opens way to a regulatory and reimbursement system that can provide unmatched access to treatment and commercial rewards. As of June 2015, the U.S. spent $339 billion yearly in moving annual dollars with 11.8 percent annual growth in sales according to IMS Health and Citi. IMS Health reported that new brand spending grew by $19.8 billion between June 2014 and June 2015. No other healthcare market on earth offers greater rewards.

At the same time, a strictly codified and transparent regulatory regime allows manufacturers to provide patients with safe and effective products in an open marketplace that protects intellectual property. Both the private and public sectors have demonstrated decades of support for the research and development communities and a motivated workforce that allows companies to better leverage investments for future growth and continuing R&D. And while FDA regulators generally require products that are approved to be tested in U.S. populations, they welcome new and innovative therapeutics that have been developed elsewhere to support the goal of providing the best healthcare in the world.

Reaping those rewards is neither easy nor assured. The most obvious challenge is obtaining FDA marketing approval, a process that is clearly laid out in rules, regulations, procedures, and scientific advices to industry. But FDA approval does not ensure commercial success. Payers largely determine patient access to new agents, increasing attention to outcomes, not simply to treatment. This shift is particularly clear in the rare disease and orphan drug market, an area of growing interest for biopharma.

Clinicians may see the therapeutic value of a new product and prescribe it eagerly. But whether that prescription is approved and reimbursed is an independent decision made by more than 3,000 governmental and commercial sectors, payers and pharmacy benefit managers (PBMs). Payers want to see evidence that a new agent offers a measurable improvement in clinical efficacy, cost effectiveness, quality of life or defined outcomes compared to existing therapies. If there are no existing approved therapies, as was the case with infantile hemangioma before Pierre Fabre developed Hemangeol, payers expect to see improvements compared to the current standard of care, including surgery or off-label use of agents approved for other indications.

Breaking into the U.S. market

Why do companies want to market their product in the U.S.?

$399bn

moving annual total (MAT) dollars spent

as of June 2015[1]

Double-digit sales growth of

11.8%

at August 2015 compared to 2014 figures[2]

New brand spending increased by

$19.8bn

between June 2014 – June 2015[1]

1. IMS Health, August 2015, Pharmaceutical trends, issues and outlook
2. Citi, September 2015, IMS Monthly Prescription Trends & Charts

The challenge in the U.S. is to demonstrate that a new agent, which is almost certainly more expensive than the current standard of care, represents a significant improvement in outcomes, overall costs for treatment, or other factors that justify the additional expenditures for a new product. In essence, sponsors must demonstrate that an agent is safe and effective to obtain FDA approval, then demonstrate that it is more effective or more cost-effective than the current standard of care to gain payer approval.

Product launch planning

Pierre Fabre sources noted that infantile hemangioma is a vascular neoplasm, often called a strawberry birthmark, that affects about 30,000 to 40,000 children yearly. And while most hemangiomas are benign, 10 to 12 percent of affected children require systemic therapy due to the location and severity of their hemangiomas. Hemangiomas that are rapidly proliferating can cause functional impairment, especially when the lesions are located in areas that affect hearing, vision or breathing. Rapid proliferation can be especially threatening for infants in the first few months of life.

The conventional treatment for infantile hemangioma is watch-and-wait with surgery as needed. Pediatric surgery can affect quality of life for both infant and parents. Hemangeol was the first agent to be approved by the FDA to treat infantile hemangioma, although laser surgery, interlesional steroids, propranolol and topical solutions have been used off-label for many years. Hemangiomas usually disappear after several years, but even lesions that did not cause functional impairment can leave permanent tissue damage leading to physical and psychosocial scarring.

U.S. payers traditionally see drugs and biologic agents as cost drivers. The reality is that new agents can be cost savers compared to the existing standard of care. The challenge is to help payers recognize and understand that investing in a new therapeutic agent today can save significantly more on longer term therapy with less effective agents, surgery or other medical intervention and improved quality of life for patients and their families.

Commercial success in the U.S. market depends on three key factors that are controlled largely by payers. The new agent must:

- find a place on payer and PBM formularies.
- avoid or minimize utilization controls such as prior approval or step therapy requirements that discourage prescribing.
- be placed on payment tier that ensures patients can afford the product and are willing to accept the required copay.

Building access for patient coverage in managed care is a structured, four step process. The first step is market research to understand the payer environment prior to FDA approval. The second step is establishing an advantageous formulary position that allows easy and affordable access to the drug after approval.

The third step is to analyze initial business to ensure that coverage is maximized to provide access to a sufficient number of patients to ensure commercial success. The final step is to develop patient programs, medication guides and caregiver education materials to enhance adherence and proper use of the medication to maximize outcomes. Each of these four steps must be tweaked and adjusted to reflect the disease state, the patient population, the existing standard of care, awareness of the disease, attitudes toward the disease and commercial factors such as potential competition, pricing, distribution and formulary placement.

Pierre Fabre and Quintiles invested more than two years, starting well before FDA approval of Hemangeol, in analyzing the potential market and creating a detailed 120-day product launch plan. The initial steps included surveys designed to gauge how managed care organizations, prescribers, payers, parents and regulators would likely view this novel product, assess the general level of knowledge about the condition and identify appropriate business partners to help build out the infrastructure needed to launch a new product.

Gaining access in the U.S. Key factors:

- *Knowledge, communication and collaboration are necessary ingredients*
- *Survey market landscape and prepare the market*
- *Identify appropriate business partners to help build out the infrastructure and fill the gaps*
- *Conduct additional market research to adapt the business model to the U.S. market dynamics*
- *Identify the business model to meet distribution needs*
- *Brand the corporate name with payers and healthcare providers*
- *Build KOL advocacy to establish disease and clinical profile*
- *Participate in pre-launch congresses and society meetings*
- *Develop patient support groups and foundations*

One of the most important considerations was the size of Pierre Fabre itself. An international biopharma based in France, Pierre Fabre launched the U.S. campaign for Hemangeol with a single marketing employee. Establishing the corporate brand identity with payers and healthcare providers was an important early step. Creating a unique identity in the marketplace also enabled the company to identify the key opinion leaders (KOLs) in infantile hemangioma and work with them to establish an appropriate disease and clinical profile. The primary thought leader for Hemangeol is in Bordeaux, France, and helped establish connections with U.S. based thought leaders, who helped establish clinical trial centers.

As clinical trials progressed, these same KOLs presented results at clinical congresses and medical society events. This active participation in prelaunch scientific events helped to build awareness of both infantile hemangioma and emerging clinical data supporting the drug candidate that became known as Hemangeol. The company also pursued opportunities to engage patient advocacy groups, helping them to understand the disease, its potential implications for infants and parents and the potential improvements to treatment and quality of life than an approved drug could bring.

The company's small U.S. footprint also helped to guide decisions on distribution and sales. A limited distribution network using specialty pharmacies made sense, given the small patient numbers and the scattered geography of the market. The small, geographically scattered patient population suggested a syndicated sales force as the most effective method to introduce the product to the payer market. Syndicated sales allowed a new-to-market company to leverage the established reputation of Quintiles' national sales team.

Focus on payers

The key consideration in developing KOLs, working with advocacy groups and creating the sales and distribution strategy was maximizing access to payers. Like many rare diseases, infantile hemangioma was not on their institutional or personal radar.

Early payer contacts, before Hemangeol was approved, focused on disease awareness. Market research showed that 90 percent of pediatricians took a watch-and-wait approach to the condition. Neither clinicians, parents nor payers recognized that if a proliferating hemangioma goes untreated, seven out of ten children will have permanent scarring or tissue distortion that can affect their quality of life, their physical and psychosocial functioning and their utilization of healthcare services.

The key to getting appropriate treatment is to get the product on formulary so providers can write prescriptions without hindrance and patients or caregivers can get access. Providers can write a prescription for any product that is approved by the FDA, but getting a prescription for a non-formulary product reimbursed by health plans can be a discouraging, bureaucratic process.

KOLs can help identify the kind of information health plan Pharmacy and Therapeutics (P&T) Committees are likely to request in their consideration of a new drug. P&T decisions are crucial in whether a new agent is placed on formulary and what restrictions may be placed on its use. Utilization management tools such as prior authorization, step therapy or requirements that patients first fail another treatment can discourage many physicians from writing a prescription in the first place.

Pricing and tier placement are equally important. Managed care is fast becoming the dominant payer structure for both governmental payers (Medicare, Medicaid, Veterans Administration, and Department of Defense) and commercial payers. Payers must pay attention to price as well as to clinical and quality of life improvements a new drug can bring. Only when payers understand the value that a new product can bring will they ease or eliminate prescribing barriers and place it on a copay tier that is acceptable and affordable for patients and caregivers.

Ready, set, approved

Another key part of the 120-day launch plan is ensuring that launch materials are ready the day the new product is approved. In addition to pre-launch work, making the case for early and accessible formulary placement, the manufacturer must ensure the new product has its unique ten-digit NDC (National Drug

Building access for patient coverage in managed care

Commercial managed markets

- Conduct market research to understand payer environment prior to FDA approval
- Establish advantageous formulary position allowing patient access to Hemangeol
- Analyze business to ensure maximized coverage and access to patient lives for commercial success
- Develop patient programs, medication guide, and caregiver education to enhance patient adherence, compliance and proper use of medication

Code) number loaded in their systems. All relevant materials are loaded by First DataBank and other compendia that provide drug information to prescribers and payers. If the compendia do not have complete and accurate information on a new therapeutic, it cannot be adjudicated and reimbursed properly.

Once compendium files are loaded, we must check with payers immediately after launch to insure information is not just loaded, but loaded correctly. Any compendium errors or data transmission problems will slow initial uptake.

It may also be helpful to launch a patient assistance program when the product is launched. A high copay could block access for many parents while an easy-to-navigate patient assistance program can boost utilization and create good will among advocacy groups and prescribers.

Treatment guidelines are another important issue. Treatment recommendations at the time of approval are expert recommendations. And it can be easy for payers to seek out the opinion of another, less well-informed expert to justify not putting a new agent on formulary or placing the product in an upper tier with a high copay to discourage utilization. Helping KOLs coalesce around a new drug, encouraging them to amass clinical data on usage and outcomes, can encourage the development of clinical guidelines to replace expert opinion. Once treatment guidelines are in place, KOLs, advocacy groups, prescribers and the manufacturer are in a stronger position to push for more favorable formulary and tier placement to increase patient access and utilization.

About the authors

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Randolph has more than 25 years in the pharma industry and currently manages a team of reimbursement, access and distribution specialists in managed care and trade. He is formerly a Principle of VCG & Associates, which was acquired by Quintiles in 2011. His team's reach has access to greater than 85% of managed lives in the United States. Randolph has participated in launching more than 150 products in his career, covering product marketing, 3PL, sales management, IDN, trade and account management.

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Joe has 30 years' experience and is currently head of the Pharmaceutical division at Pierre Fabre U.S.A. Joe has led market entry for Pierre Fabre into the U.S. by creating Pierre Fabre Pharmaceuticals Inc., which includes Dermo-Pediatric and Oncology business units.

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