Creating Profitability and Expanded Value as an Orphan Drug Manufacturer - BLINCYTO's Unique, Initial Market Offerings Explored

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Amgen's bispecific antibody, BLINCYTO, was catapulted into the mainstream in 2014 after receiving breakthrough therapy designation from the FDA. BLINCYTO was born out of the company's BiTE® (Bispecific T cell engager) program and was the first of its kind.

According to their press release announcing the news, BLINCYTO hailed the honor of being both the first approved "bispecific CD19-directed CD3 T-cell engager antibody construct product" and "single-agent immunotherapy" on the market to treat patients with refractory B-cell precursor acute lymphoblastic leukemia (ALL).

BLINCYTO is considered an orphan drug, critical to the market, accelerating their FDA approval early on.

BLINCYTO's Orphan Drug Status - What the Designation Means for Pharma Developers

Orphan drugs are therapeutics that treat conditions so rare that they will likely not generate a high level of profitability. ALL was, and still is, one of the rarest forms of cancer in the United States.

It accounts for less than 1% of all cancer types diagnosed annually (5,690 patients on average). Mortality rates are high for specific age groups, with only 38% of patients age 20+ expected to live five years past initial diagnosis. Children have better odds, with 89% survival rates over the same period.

Despite being labeled as potentially unprofitable, the orphan drug category provides many strategic benefits, one being expedited FDA clearance in Amgen's case.

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During the study stages, orphan medicines traditionally have lower clinical trial costs in the US market. Once designated as an orphan medicine, pharma companies will unlock many incentives, including tax credits, waivers for prescription user fees (measured at $2.9M in 2021), and seven years of marketing exclusivity, among other industry advantages.

To ultimately reap the benefits of their orphan drug status within the market, Amgen also had to focus intensely on developing resources for their target patient community.

Patient Support Methods Offered Immediately Upon Launch

While telehealth services are offered by most hospital systems today, back in the pre-pandemic days of 2014, they were still considered unique offerings, especially for companies not directly within the hospital sphere.

To successfully commercialize BLINCYTO within the US market, Amgen developed a patient and caregiver-centric platform to provide around-the-clock support for early adopters. Onyx 360 was created in collaboration with the subsidiary Onyx Pharmaceuticals to provide all information needed to make receiving and prescribing treatments as simple as possible.

Beyond existing treatment resources, Onyx 360 also featured payment support and referrals for partnering organizations. Partners did not only include medical professionals trained to support patients with everyday activities. They also had workers providing additional value levels for patients, including emotional support.

The program was a strategic commercialization choice as it packaged BLINCYTO as more than another prescription drug for consideration. Prescribers could see the level of support their patients would be provided outside of their services.

In the US market, doctors have minimal time with patients as is. Amgen effectively communicated that prescribing BLINCYTO would not take away from practitioners' time and could provide expanded levels of support to patients (beyond medical offerings).

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Doctors are ultimately focused on the well-being of their patients, making Amgen's initial value proposition of 360 degrees of support undeniable in the US medical industry.

Considerations for the Pharmaceutical Industry in 2022

At the end of 2021, Amgen reported that BLINCYTO sales had increased 29% from the previous year, resulting in $103M in revenue. The orphan drug's initial solid foundation within the market can be accredited as one of the principal drivers of this level of success.

Pharma companies seeking to achieve similar results should consider the ultimate drivers of US medical professionals and their target patient segments. Identify multiple avenues of the unmet needs they may be dealing with (beyond the traditionally highlighted areas).

What programs can you implement to go the extra mile? Who will you partner with to create truly differentiated value on the market?

The answers to these questions will guide effective implementation strategies for new drug candidates.

- Content by the BDMT Global Team

If you are interested in potential partnerships and long-term relationships with the companies mentioned in this article or others in the U.S. and/or S. Korea, contact Suzy at sim@bdmtglobal.com.

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