CPhI conferences

UBM

CPhI Orphan Drugs 2014

Driving the New Commercialisation and Sustainability Frontier of the Pharmaceutical Industry

24 - 26 March 2014 | Royal Plaza on Scotts, Singapore

Aiswariya Chidambaram
Senior Research Analyst - Life Sciences
Frost & Sullivan

UBM: How has the market changed over the last 10 years for orphan drugs both in Asia and globally?

AC: Orphan drugs has transitioned from relatively few companies pursuing treatments to a focus for most big pharma companies in addition to hundreds of smaller biotech firms. The high price commanded by orphan drugs (which payers generally have to accept because there are no other options) is attracting more companies particularly as the opportunities in more common, chronic diseases are starting to dry up. The US FDA has approved a total of 443 orphan drugs since 1983, with at least an additional 1,466 granted orphan drug designation. Japan ranks second with 293 approved orphan drugs, and Europe third with 65 approved orphan drugs.

Furthermore, treatments are advancing from palliative care to true disease modification; good examples are Vertex's drug for Cystic fibrosis and Alexion's drug for Paroxysmal Nocturnal Hemoglobinuria and other orphan diseases.

UBM: What challenges still remain and how do you think the orphan disease marketplace can be improved?

AC:

- *Huge unmet medical need, particularly for disease-modifying therapy*
- *Lack of experience and expertise in the development of orphan drugs*
- *Absence of adequate infrastructure and distribution channels for small patient populations, especially in rural areas.*
- *Limited understanding of the attributes of rare disease markets*
- *The inability to manage the sensitivities based on the high prices of orphan drugs. Often, treatment costs for rare diseases are high, ranging from $200,000 to $600,000 per patient per year.*

Incentivising orphan drug manufacturers in the form of Grants and Tax credits to offset R&D costs, fee waivers and more structured reimbursement policies are most likely to help combat these challenges. Additionally, efforts to improve access to orphan drugs across all groups of patient population worldwide are crucial.

UBM: What can governments do to drive the manufacturing of orphan drugs into the region?

AC:The Government has been very supportive of orphan drug development for rare disease indications over the last five years. Some of the key existing incentive highlights include:

Seven-year Market Exclusivity: Orphan drug manufacturers are given the opportunity to enjoy seven years of marketing exclusivity. On approval of an orphan drug, the FDA will not allow other manufacturers to market a drug that treats the same rare disease for a period of seven years. However, marketing exclusivity may be withheld if the manufacturer does not meet the demands of patients.

Access to Grants and Tax Credits to Offset R&D Costs: The Internal Revenue Service United States department offers tax credits equivalent to 50 per cent of the cost of clinical trials. The Orphan Products Grants Program provides funds for conducting clinical trials. All applications for the grants are reviewed by the expert committee of OOPD. Decisions are made on a competitive basis, and sponsors may avail up to $200,000-$300,000 per year for three years.

Fee Waivers: Since 1992, the FDA has made various changes to the fee exemption criteria. In general, drug sponsors have to pay application, establishment and product fees during the drug registration process. In 1997, the FDA waived the application fee for manufacturers of orphan drugs through the Food and Drug Administration Modernisation Act (FDAMA).

Increasing support for reimbursement and improving patient access to specialists are some of the ways by which the Governments can drive orphan drugs into the regions.

CPhI conferences

UBM

CPhI Orphan Drugs 2014

Driving the New Commercialisation and Sustainability Frontier of the Pharmaceutical Industry

24 - 26 March 2014 | Royal Plaza on Scotts, Singapore

Aiswariya Chidambaram
Senior Research Analyst - Life Sciences
Frost & Sullivan

UBM: What do you think the future holds for tackling orphan diseases?

AC: Knowledge of the underlying pathology of diseases and more druggable targets will continue to be advanced, leading to more effective drugs that can slow or stop disease progression rather than just treating symptoms. However, many predict that, with more and more expensive orphan drugs entering the market, the healthcare system will start to have a lot of trouble supporting them, and prices will be forced to drop, which of course will start to significantly eat into the ROI, since orphan drugs are not much less costly to develop in comparison to conventional drug development (although trials are of course smaller since there are just not many patients)

UBM: What is in store for regional/local manufacturers in the orphan market?

The pipeline is generating hope for some of the most serious and debilitating or life-threatening rare diseases in need of better therapies such as pancreatic cancer, Pompe disease and Amyotrophic Lateral Sclerosis. Pipeline drugs targeting orphan diseases utilize diverse approaches such as small molecules, antisense, gene therapy, monoclonal antibodies, bi-specific antibodies, peptide therapies, stem cell and other cell therapies to correct the underlying pathology of disease. Many are targeted therapies designed to address a specific genetic defect for a personalized medicine approach, and many have strong potential to slow or stop disease progression rather than simply manage the disease symptoms.

As a vast majority of the orphan drugs in development are biologics, adequate biomanufacturing capabilities would be required.