

## Orphan Drugs

*(Source: Edited excerpts from a report by Andreas Hadjivasilou, an Analyst at EvaluatePharma, edited by Lisa Urquhart, Editor at EP Vantage and published by EvaluatePharma®)*

An orphan drug is a pharmaceutical product aimed at rare diseases or disorders. The development of orphan drugs has been financially incentivised through US law via the Orphan Drug Act of 1983. The National Organization for Rare Disorders (NORD), which was instrumental in establishing the Act, currently estimates 30 million Americans suffer from 7,000 rare diseases. Prior to the 1983 Act, 38 orphan drugs were approved. The success of the original Orphan Drug Act in the US led to it being adopted in other key markets, most notably in Japan in 1993 and in the European Union in 2000. Rare Disease Patient Populations are Defined in Law as: • USA: <200,000 patients (<6.37 in 10,000, based on US population of 314m) • EU: <5 in 10,000 (<250,000 patients, based on EU population of 514m) • Japan: <50,000 patients (<4 in 10,000 based on Japan population of 128m).

The steady and inexorable growth of the orphan drug market remains one of the prominent themes in the fourth edition of EvaluatePharma's *Orphan Drug Report*. What has changed in the last 12 months is the increased scrutiny of the price of these lifesaving products. There is little doubt that insurers will continue to cover orphan drugs, due to the fact they are frequently the only option for patients and, for now, remain a relatively small part of overall budgets. But according to the findings in this year's report the market will continue to expand rapidly, with sales growth forecasted at 11% per year, more than twice the rate predicted for conventional drugs. In fact, sales of orphan drug are expected to almost double between 2016 and 2022, to reach US\$209 billion. This rapid growth and current willingness of payers to pay the huge price tags are two of the main reasons why the sector has become more and more attractive to some of the industry's biggest players.

The Report finds that the market for orphan drugs, based on the consensus forecast for the leading 500 pharmaceutical and biotechnology companies, will grow by 11.1% per year (CAGR) between 2017 and 2022 to US\$209 billion. The growth of the orphan drug market is more than double that of the overall prescription drug market, which is set to grow by 5.3% over the period 2017-2022. Orphan drugs are set to account for 21.4% of global prescription sales in 2022, excluding generics, up from 6% in 2000. In 2016 orphan drug sales increased 12.2% to US\$114 billion vs. 2015, while non-orphan drug sales increased by 2.4% to US\$578 billion.

EvaluatePharma® estimates that the average cost per patient per year in 2016 for an orphan drug was US\$140,443 versus US\$27,756 for a non-orphan drug. The average drug price has increased year on year for both orphan and non-orphan drugs since 2012. The median price differential between an orphan and non-orphan drug in 2016 was 5.5 compared to 9.8 in 2012. The median

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## In Brief..

- ♦ New Zealand wholesaler and distributor **EBOS Group** reported strong first-half earnings, including double-digit revenue and profit growth. The company announced an unaudited net profit of NZD\$68.8 million (US\$48.4 million) for the six months ending December 31, 2016. This follows significant growth in its Healthcare and Animal care divisions in both Australia and New Zealand. Group revenue was up 17% to NZD\$4 billion (US\$28.1 billion).

- ♦ More than 50 of **Walgreen's** local specialty pharmacies have been designated as cancer-specialized locations. Each location will be led by pharmacists and pharmacy technicians with advanced cancer training and expertise. Pharmacy staff has completed a cancer-focused curriculum of advanced education courses focusing on colorectal, lung, prostate and breast cancer, as well as various blood cancers. The goal is to be sure patients get the medications and information they need to stay informed about their disease.

- ♦ South African logistics group **Imperial Holdings** will buy a 70% stake in Kenyan pharmaceutical distributor **Surgipharm** for R456 million (US\$35 million). This is in line with the company's African growth strategy. Surgipharm, which is headquartered in Nairobi, is a leading distributor of pharmaceutical, medical, surgical and allied supplies in Kenya and has an annual turnover of approximately US\$70 million. Imperial has also made acquisitions in the pharmaceutical sector in Nigeria and the Netherlands.

- ♦ **Allergan** CEO *Brent Saunders* is calling for President Trump to lead the pharmaceutical industry in negotiations pertaining to drug pricing. Saunders emphasized that voluntary agreements in the restraint of drug prices are "a very practical solution that doesn't, in my view, do anything to impede innovation," he said in an interview with Bloomberg. He did, however note that convincing fellow drug makers to agree with him could be a challenge. Companies such as Novartis AG, Takeda Pharmaceutical Co. and AbbVie have already taken voluntary action to cap drug price increases.

- ♦ The **U.S. Food and Drug Administration** approved a record-setting 800 generic drugs in 2016, including both full and tentative approvals. Last year's performance surpassed 2015's previous record of 726. Many of these approvals "first-time generics" which includes introduction of generic counterparts for brand-name drugs already on the market, which ultimately leads to lower drug prices. Often, there are multiple generic versions which drive price competition.

- ♦ **AmerisourceBergen** Executive Vice President *James Frary* has been named Chairman of the North Texas chapter of CEO's Against Cancer. In this volunteer position, he will focus on a variety of issues facing working professionals across the northern half of the states, including improving employee health, workplace productivity and health care costs.

## Orphan Drugs (cont.)...

price of orphan drugs and non-orphan drugs has increased by a factor of 1.3 and 2.3 since 2012 respectively.

The Report finds that revenue per patient for the Top 20 USA selling orphan drugs is correlated ( $R^2 = 0.77$ ) to the number of patients treated in 2016. A similar analysis of the Top 10 selling orphan drugs that treated fewer than 10,000 patients confirms a closer correlation ( $R^2 = 0.94$ ). This analysis confirms industry perceptions that smaller patient groups allow a pricing premium to be achieved versus non-orphans. Products such as *Gleevec* support the notion of an innovation premium for drugs that create a step change in treatment options and therapy outcomes. *Soliris* confirms the pricing power resulting from indications with the fewest number of patients.

Celgene will surpass Novartis, according to EvaluatePharma®, as the world's number one orphan drug company in 2022, climbing two places, and pushing Novartis down to number three. One product contributes the majority of orphan sales for two of the top four; Celgene (*Revlimid*, 80% of sales) and BMS (*Opdivo*, 68% of sales). Seven of the Top 10 companies by orphan drug sales in 2022 are forecast to be Global Majors. The Top 5 companies in 2022 are forecast to account for almost one third (30.6%) of the orphan drug market.

EvaluatePharma® conducted a secondary analysis of the companies active in the Orphan drug space excluding orphan products in the oncology therapy area. Shire is forecasted to remain the top selling company in this space in 2022 with US\$7.9 billion in sales. This space is growing at a CAGR of 7.7% with sales forecasted at US\$94.6 billion in 2022. The top 20 companies will represent 66% of the non-oncology orphan space by 2022. The non-oncology space represents 53% of the orphan market in 2016 and is forecasted to decrease to 45% by 2022.

*Revlimid* is the world's largest orphan drug in 2022, with sales of US\$13.6 billion for all indications. *Revlimid* from Celgene was first approved in December 2005 for the orphan treatment of myelodysplastic syndrome. *Revlimid* is also approved for the orphan indications Non-Hodgkin's lymphoma and multiple myeloma and remains in development for a number of other orphan conditions. Bristol-Myers Squibb's *Opdivo* approved for multiple myeloma & Hodgkin's lymphoma, and designated for hepatoma, glioblastoma, small cell lung cancer and oesophageal cancer is set to be a distant second with US\$9.1 billion in worldwide sales.

*Opdivo* is Europe's largest orphan drug in 2022, with sales of US\$2.4 billion for all indications. All of the orphan drugs in the top 10 are already marketed, with all but one forecast to continue to grow through to 2022. Orphan drugs are set to account for 55% of the cumulative value of the European pipeline through to 2022. Orphan products forecasted to launch in the coming years are expected to grow at a CAGR of 116% vs. non-orphan pipeline drugs which are forecasted to grow at 95% CAGR.

EvaluatePharma® found that the number of US orphan drug designations granted decreased 6% in 2016 to 333. Despite this, the number of applications to the FDA reached a new high of 582, a 23% growth on 2015. At a 57% acceptance rate this resulted in the lowest acceptance level since 2002. European orphan designations grew by 9% in 2016 reversing a 5% fall in 2015.

## WBA Discloses Its 2016 CSR Program Results

Walgreens Boots Alliance (WBA), which includes their international pharmaceutical distribution arm, released its annual Corporate Social Responsibility Report for fiscal 2016, highlighting the company's ongoing support for people in its communities and workplaces, and overall progress toward its environmental and social goals.

"Our company is full of inspiring stories of social and environmental responsibility," stated Ornella Barra, WBA's COO and leader of Corporate Social Responsibility (CSR) (who is also an IFPW Director). "I feel immensely proud of these initiatives, which show how much our people care and which are embedded in the work we do every day. We have set ourselves 12 challenging goals connected with our business activities and are excited to report on our progress."

The report, covering the fiscal year that ended Aug. 31, 2016, was recently launched at the Knight Conference Center in the Newseum in Washington, D.C.. WBA made significant strides in CSR accountability in 2016, reporting metrics that will serve as the baseline for evaluating future progress.

For the first time since it was created in December 2014, the company collected data across all of its businesses, on charitable contributions, carbon emissions, energy usage, waste disposal, employee retention and employee gender. In the 2016 report, the company maps its CSR goals to the United Nations Sustainable Development Goals. WBA's commitment to those targets was recognized last year when the United Nations Foundation honored the company with its Global Leadership Award.

The award highlighted, in part, the accomplishments of Walgreens highly impactful "Get a Shot. Give a Shot" initiative, which has provided as many as 15 million life-saving vaccines to children in developing countries through the Foundation's Shot@Life campaign over the past three years. Other events of note include the 2016 Red Nose Day, which doubled the donations raised the year prior by raising more than US\$20 million.

WBA has built on the strong CSR tradition of its legacy companies, leveraging its global scale to make a real difference in programs to reduce environmental impact and to champion health and wellbeing around the world. The company has identified 12 strategic goals connected to its business and to its four key CSR areas: Community, Environment, Marketplace and Workplace.

"We truly embrace our corporate and social responsibility, and we embed it in how we operate our company as a sustainable and profitable enterprise," commented Stefano Pessina, executive vice chairman and CEO of WBA. "As a healthcare champion, for us there is a strong commercial as much as moral imperative to be a good corporate citizen. Assessment, measurement and monitoring of social and environmental impact are built into our operating initiatives, procedures and our consideration of new contracts or corporate opportunities. Delivery against these policies is implemented in our daily work, in our businesses around the globe."

*We would like to share your stories of Corporate Social Responsibility program. Please send details to Christina Tucker at [c.tucker@ifpw.com](mailto:c.tucker@ifpw.com).*