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Orphan drug lead cements Novartis ascendency

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With its stable of rare-disease treatments such as Tasigna, Afinitor, Gleevec and Sandostatin LAR, the Swiss big pharma Novartis tops the table as the biggest orphan drug maker in the world – a lead it will retain through 2018 when it will have sales of $11.8bn, according to *EvaluatePharma's* first ever analysis of the sector.

Roche's cancer antibody Rituxan will retain the crown of biggest-selling orphan drug with sales of $6.95bn, but could be challenged in 2018 by Celgene's immunomodulator Revlimid; Soliris, the world's most expensive drug, will round out the podium, according to the 2013 Orphan Drug Report. One dollar in every six spent on drugs in 2018 will go for a rare-disease treatment, demonstrating the growing importance of orphan drugs to the future of both big pharma and small biotechs.

Not a hard-knock life

Much has been made of the importance of orphan drugs to the industry's outlook, as a streamlined regulatory process, smaller trial sizes and favourable pricing mean a better return on investment on R&D in rare diseases – although they might not always be able to command high prices (*Orphan drugs can be a route to success, but pricing is a future problem, October 16, 2012*).

The report, to be released tomorrow at the Biotechnology Industry Organization international convention, quantifies the magnitude of the industry's shift; detailed tables and charts will be contained in the report.

Orphan drugs are forecast to grow at 7.4% per annum between 2012 and 2018, and by that year will amount to 15.9% of all non-generic prescription drug sales, from 12.9% in 2012. As Novartis is forecast to remain the biggest drugmaker in the world in 2018, perhaps it should not be too surprising that it will also be the biggest producer of orphan drugs (*Novartis set to remain top spender as R&D investment dips, June 18, 2012*).

Indeed, the fact that many oncology drugs qualify for orphan drug designation, and that many of those end up in the hands of big pharma before launch, should tend to mean many familiar names are in the league table of top rare-disease companies.

On the other hand, Celgene, with eight marketed drugs, is number three on the orphan list but is forecast to come 22nd in overall sales in 2018. Other companies outside the top 20 in pharma sales that rank higher in the orphan sector are Baxter International, which benefits from its presence in blood products, particularly haemophilia, and Biogen Idec, forecast to be pulling in more than a half-billion dollars from orphan haemophilia products in 2018 in addition to $2.1bn from its multiple sclerosis treatment Avonex.

Alexion Pharmaceuticals is the hedgehog that knows one good thing – as it is the owner of the $400,000-a-year Soliris, it makes it onto the list as the ninth biggest orphan drug manufacturer despite having only one launched product. The UK group Shire, which has branched out from its CNS franchise into rare lysosomal storage disorders, also makes the top 20 in orphan diseases, along with non-giants Vertex Pharmaceuticals, Merck KGaA and Actelion.

Also of interest is which players are moving up and down in the rankings of orphan disease market share: Pfizer loses third place to Celgene. Sanofi, helped along by its acquisition of Genzyme, moves up four places – three of Genzyme's top products are forecast to account for nearly a quarter of the French group's sales growth between now and 2018.

Vertex moves an incredible 28 places up the orphan disease rankings, helped along by cystic fibrosis treatments Kalydeco and VX-809, giving it an estimated $2.2bn in orphan sales in 2018.

Growth the rule

While Rituxan will retain the crown of biggest-selling orphan drug, it has a flat sales outlook for the coming years – it falls back from $7.16bn to $6.95bn in 2018, according to the report. On the other hand, sales of Onyx Pharmaceuticals' multiple myeloma drug Kyprolis are forecast to grow 70% following a 2012 approval.

The Incyte and Novartis myelofibrosis drug Jakafi will grow 48%, while Kalydeco, also launched in 2012, is expected to grow 47%, underlining Vertex's rise as an orphan drug play. Sixteen of the top 30 orphan drugs in

2018 will be cancer treatments, explaining big pharma's continuing fascination with oncology (*Therapy area licensing in 2012 – Rags and riches, February 19, 2013*).

Orphan drugs in the top 30 that are forecast to fall back include Gleevec, the first tyrosine kinase inhibitor, which loses patent protection in 2015 and as a small-molecule drug is thought to be vulnerable to generic competition, and Teva's Copaxone, which could be under threat as early as next year.

These two are the only drugs on the list subject to double-digit losses between now and 2018. Double-digit growth is the rule, demonstrating why interest in orphan drugs remains so intense.

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