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**The “one and done” era for orphan drugs: Evaluate predicts 12% growth, driven by new technologies, but benefits go to big pharma**

*Orphan drug sales reach \$242bn by 2024, according to EvaluatePharma® Orphan Drug Report 2019*

**LONDON, BOSTON, TOKYO (April 25, 2019)** – Scientific advancements will help propel the orphan drug market to a 12% CAGR to 2024, double the 6% rate expected for non-orphan products. In addition to recent breakthrough cell and gene therapy products like Yescarta and Luxturna, a number of “one and done” treatments for orphan diseases are approaching the market, including Novartis’s Zolgensma and bluebird bio’s Lentiglobin.

The explosion of new technologies brings their high price points into greater focus — for example, the \$425,000 per eye list price for Spark’s Luxturna is the value-based price agreed on with Spark and payers. In 2018, the mean cost for an orphan drug was 4.5 times that of a non-orphan product.

Despite the clear advancements in treatment, however, those calling for reform of the Orphan Drug Act may gain further ammunition. Big pharma continues to entrench their position in the orphan market, as evidenced by Bristol-Myers Squibb’s bid for Celgene, Roche’s pending acquisition of Spark, and Novartis’s \$8.7bn payment for Zolgensma.

“Seven of the top 20 R&D orphan products by net present value are either a cell or a gene therapy,” said report author Karen Pomeranz, PhD. “However, we may not see the full market impact of these drugs by 2024, which means that the long-term growth and patient benefits could be even more pronounced.”

**Additional report highlights include:**

- The mean cost per patient per year of the top 100 orphan products was \$150,854 in 2018, compared to \$33,654 for a non-orphan drugs.
- Blood, CNS and respiratory are the leading orphan drug therapeutic areas, accounting for more than 50% of the non-oncology orphan drug market.
- In 2024, Celgene, Abbvie and Johnson & Johnson will lead the oncology orphan drug market, accounting for approximately 30% of worldwide sales.
- Vertex’s triple combination for cystic fibrosis is the most valuable R&D orphan product, with an estimated NPV of \$24bn.
- The orphan drug pipeline is anticipated to contribute \$187.7bn in sales by 2024, 35% of the combined forecast for all pipeline products.

Download your complimentary copy of the “EvaluatePharma® Orphan Drug Report 2019” at <http://www.evaluate.com/OrphanDrug2019>.

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