

EvaluatePharma®

Orphan Drug Report 2020

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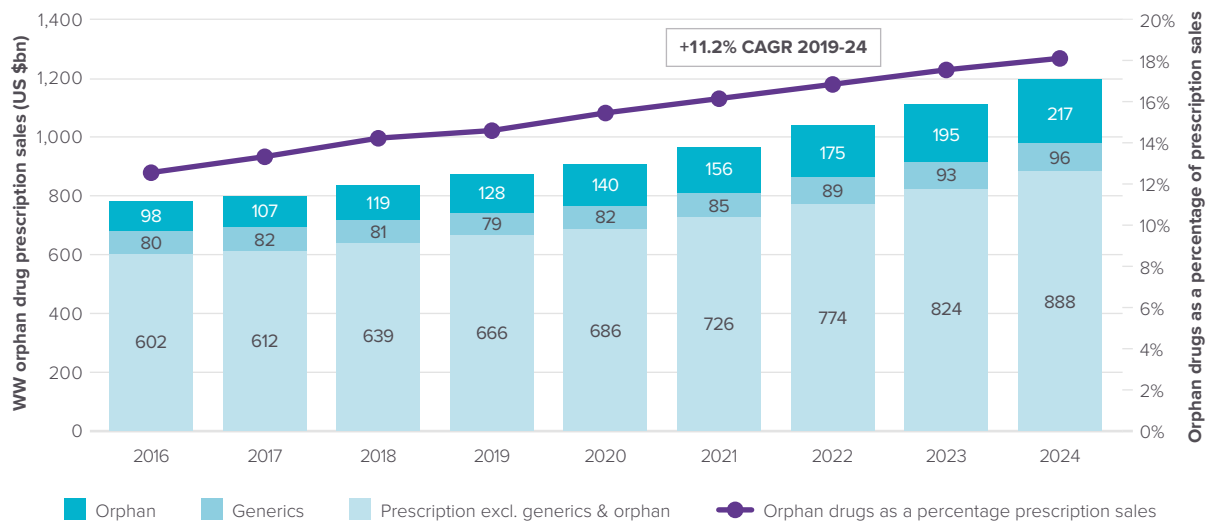
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Four decades ago, patients with rare diseases had very few treatment options. The 1983 Orphan Drug Act changed everything by providing substantial tax incentives for pharmaceutical companies to invest in rare conditions affecting fewer than 200,000 Americans. With an estimated 7,000 orphan diseases, 1 out of every 10 Americans lives with a rare condition.

Worldwide orphan drug sales and share of prescription drug market (2016-2024)

Source: EvaluatePharma® February 2020



After nearly four decades of relatively consistent growth, orphan drugs now comprise a cornerstone of the pharmaceutical market, with this category predicted to rake in \$217bn in 2024. That is over 18% of overall prescription sales in 2024. Remarkably, the market is so robust that rare diseases now take home a large portion of FDA drug approvals. In 2019, just under half (44%) of new FDA approvals went to orphan drugs. And the value created by orphan R&D drugs, based on consensus forecasts, is 20 percentage points higher than non-orphan drugs.

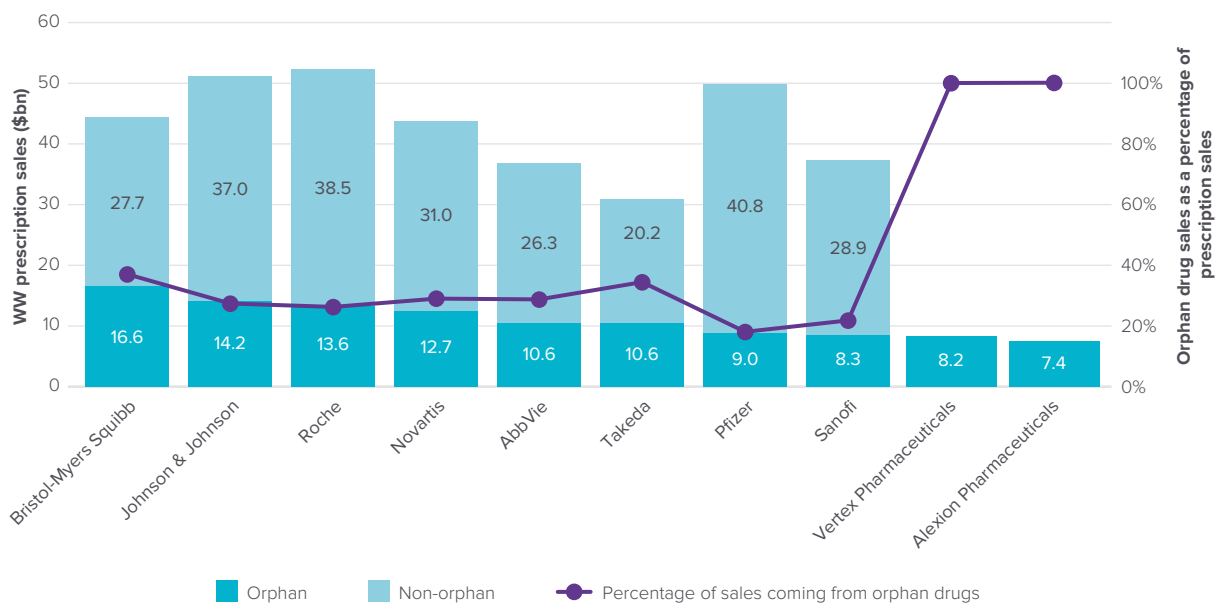


The Orphan Drug Landscape in 2024

This rising market growth continues to fuel calls to reform the Orphan Drug Act in the US from those who argue that big pharma should not be benefiting from the regulatory and tax benefits meant for orphan drug developers. Big pharma, meanwhile, is forecast to make up 8 of the top 10 orphan drug companies in 2024.

Worldwide orphan drug prescription sales in 2024 for the top 10 companies

Source: EvaluatePharma® February 2020



Of the top 10, Bristol-Myers Squibb and Takeda have the highest forecast CAGR between 2018 and 2024 — 28% and 20% respectively. This follows their acquisitions of companies that had previously been significant orphan players. It certainly looks like the orphan market's current exclusivity rights and financial incentives still make it worth investing.

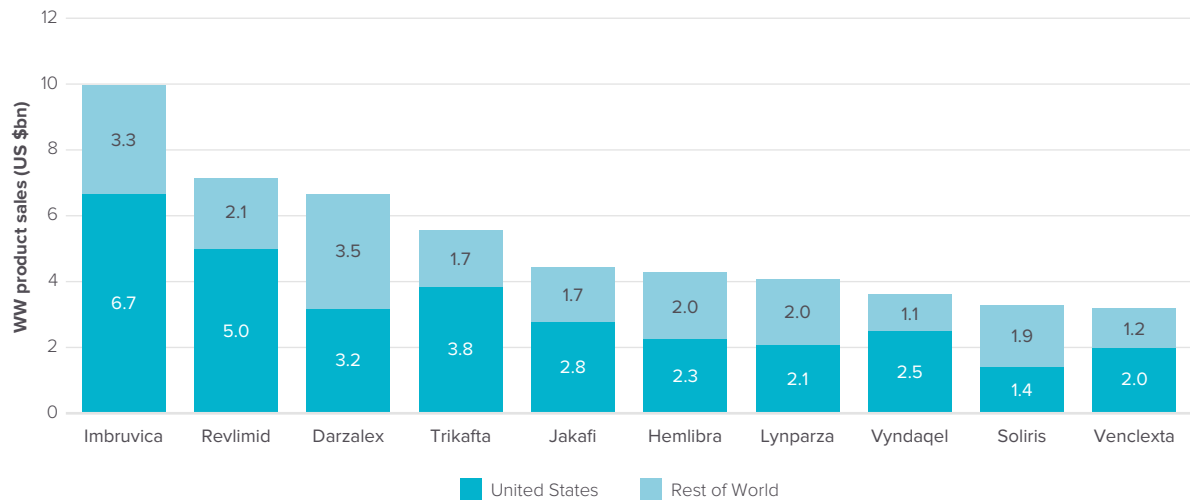
For example, \$5.5bn of Vertex Pharmaceuticals' orphan sales forecast in 2024 come from Trikafta alone, the FDA's fastest novel drug approval of 2019. Historically, top-performing orphan drugs have hailed from oncology categories. While Rituxan was Biogen/Roche's golden goose early in the decade, sustained revenue from Bristol-Myers Squibb's Revlimid forecasts it to be the second highest selling drug in 2024. This is surprising, considering that generic competitors are expected to hit the market in 2022.



Meanwhile, Johnson & Johnson's Imbruvica quickly eclipsed other top-performing orphans, and is now expected to generate \$10 billion by 2024.

Top 10 selling orphan drugs in 2024 by worldwide sales

Source: EvaluatePharma® February 2020



Outside of oncology, other top-performing orphan drugs tend to include those for rare genetic diseases, blood disorders and central nervous system diseases. Biogen's Spinraza and Alexion's Soliris were the first to market for their respective indications, spinal muscular atrophy and paroxysmal nocturnal hemoglobinuria. And more recently, strong sales for Roche's hemophilia A treatment Hemlibra and Pfizer's rare cardiomyopathy treatment Vyndaquel have helped solidify these two big pharma companies as leading players in the orphan drug market.

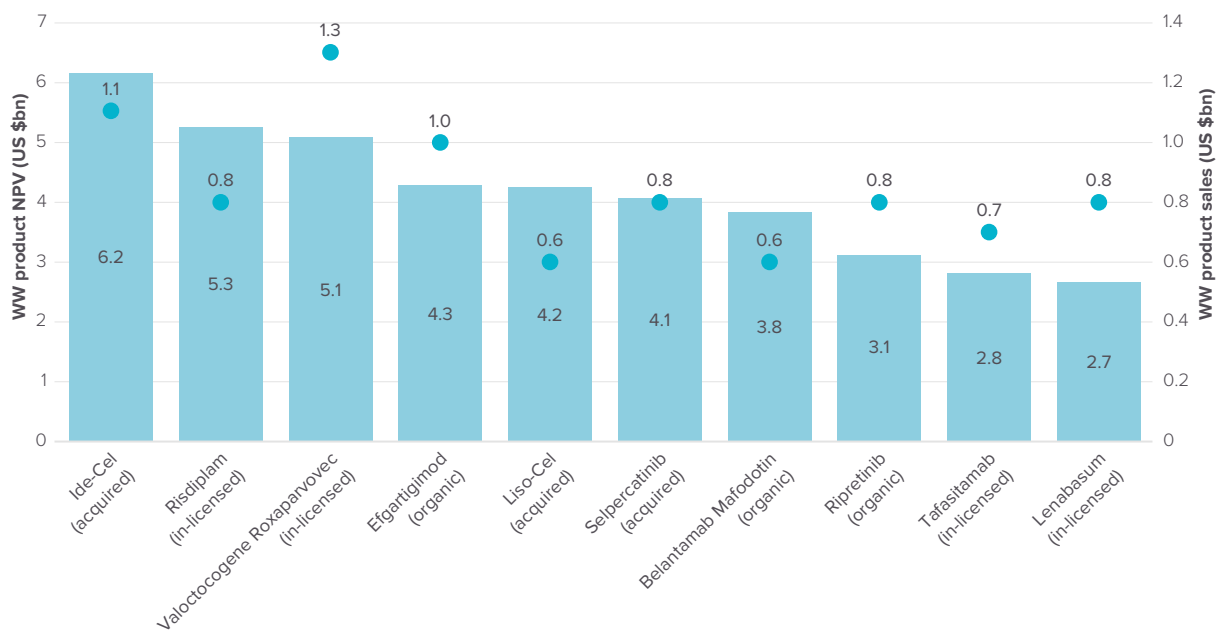


The Orphan Drug Pipeline

As science evolves, orphan drugs will become even more precise, targeting a wide range of genetic diseases. The most promising pipeline products in 2024 include two CAR-T therapies acquired by Bristol-Myers Squibb via its Celgene takeover: Ide-Cel and Liso-Cel. Both these CAR-T therapies are forecast to be in the top 10 selling R&D drugs – those currently in phase III or filed – in 2024.

Top 10 R&D orphan drugs (phase III-filed) in 2024, based on NPV

Source: EvaluatePharma® February 2020



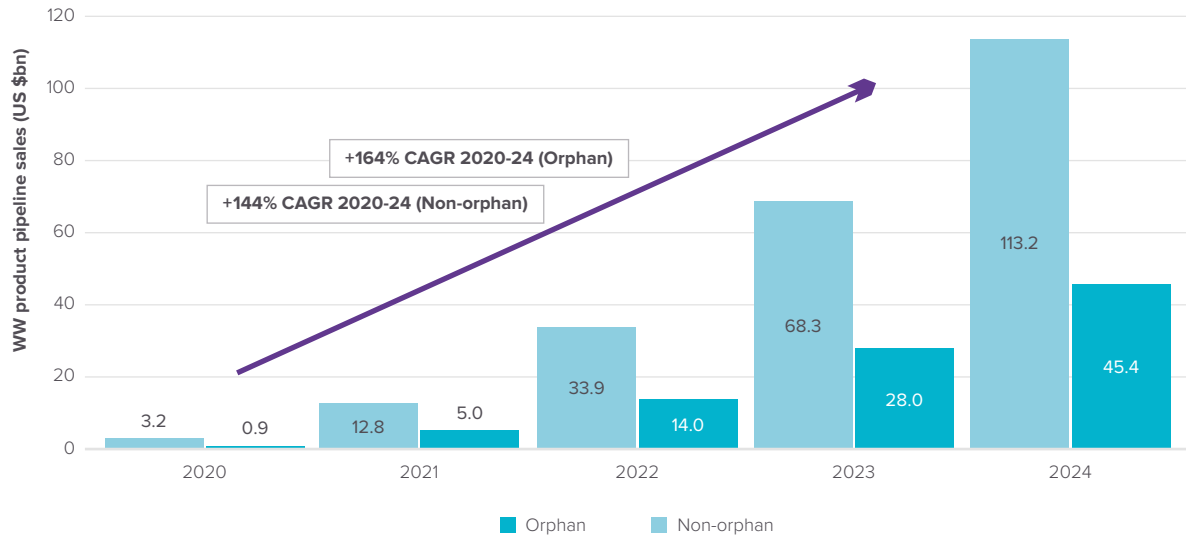
Also in the top 10 is BioMarin Pharmaceutical’s Valrox, which is filed for hemophilia A. Its 2024 forecast sales of \$1.3bn are still no match for that of blockbuster Hemlibra’s \$4.3bn, though. Perhaps potential payers are put off by the \$2-3 million price tag BioMarin expects Valrox to fetch, particularly given the concerns over the product’s durability in clinical trials.

And as awareness of rare diseases continues to grow, more and more patients will be diagnosed, broadening the potential patient population and driving even greater sales projections.



Worldwide sales of pipeline orphan and non-orphan products (2020-2024)

Source: EvaluatePharma[®] February 2020



Potential for Legislative Reform

Over the past several years, a cloud of legislative reform has gathered over the orphan drug sector. Sky-high pricing of approved gene therapies has fueled bi-partisan consensus against price gouging. Meanwhile, unprecedented jockeying for orphan drug designation has prompted calls for reform of the Orphan Drug Act.

Despite the public bedlam, meaningful change hasn't materialized – though these issues will be in the spotlight throughout the 2020 presidential election. Although legislative uncertainty remains, the current orphan development model continues to encourage drug makers to address rare diseases. We predict that orphan drugs will continue to generate strong prescription sales, growing at twice the rate as non-orphan drugs in the years to come.



Discover more about the orphan drug market with Evaluate


- Find out where the commercial opportunities lie by learning the Net Present Value of orphan products.
- Identify the drivers of risk for orphan products in development, as well as their probability of coming to market.
- Judge the cost of orphan R&D clinical trials against indication and phase benchmarks so you can realign investment accordingly.
- Contextualize potential risk and return by learning about your competitors – and their forecasts – in the orphan market.
- Learn which drugs have the potential to be first in class for treating an orphan disease.


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