

Pharmaceuticals: big firms' healthy outlook underpinned by orphan drug growth



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Big pharma is facing down critics worried that expiring patents, a lack of new blockbuster treatments, constrained public healthcare budgets and squeezed drug prices would up-end its business model. The success of orphan drugs is one reason why.

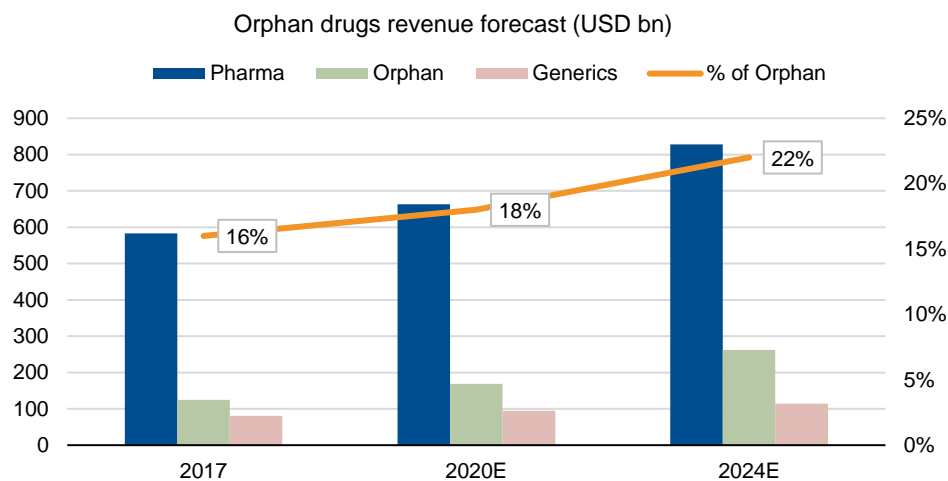
We see sales of treatments for rare diseases - known as orphan drugs – continuing to grow fast. Revenue will grow by around 12% a year between 2019 and 2024, outpacing the industry's otherwise robust overall growth of around 5% a year, according to data from EvaluatePharma. Orphan drugs will account for an estimated 22% of pharmaceutical revenue in 2024 – equivalent to more than double the contribution from generic drugs - an increase from an estimated 18% in 2020 and 16% in 2017.

To be sure, developing orphan drugs is a high-cost, low-volume business. But with that come some advantages for deep-pocketed pharmaceutical companies: barriers to entry are high for smaller competitors; and profit margins are healthy. Not only are there a large number of rare diseases, but demand is strong from health authorities, doctors and patients for more investment in effective treatments.

Elevated costs, high prices

Bringing a new orphan drug to market costs as much as five times as more run-of-the-mill drugs which is why the pharmaceutical companies have been able to justify prices running into hundreds of thousands of dollars per patient or more.

Figure 1: Orphan drugs become fast-growing pharma segment



Source: Scope, EvaluatePharma

Switzerland's Novartis makes Zolgensma, a \$2 million gene therapy so far approved for small children suffering from spinal muscular atrophy, a rare but fatal muscle-wasting disease. The company recorded revenue of USD 175 million since the drug's launch early this year.

Around 7,000 rare diseases affect an estimated 350 million people worldwide, according to Global Genes, but there might be only a few hundred or thousand sufferers in any one country, with, until recently, few treatment options. The approach of regulators and pharmaceutical companies has changed, leading to growing investment in the sector. The US Food and Drug Administration now gives special "orphan drug" status to potential treatments for rare diseases.

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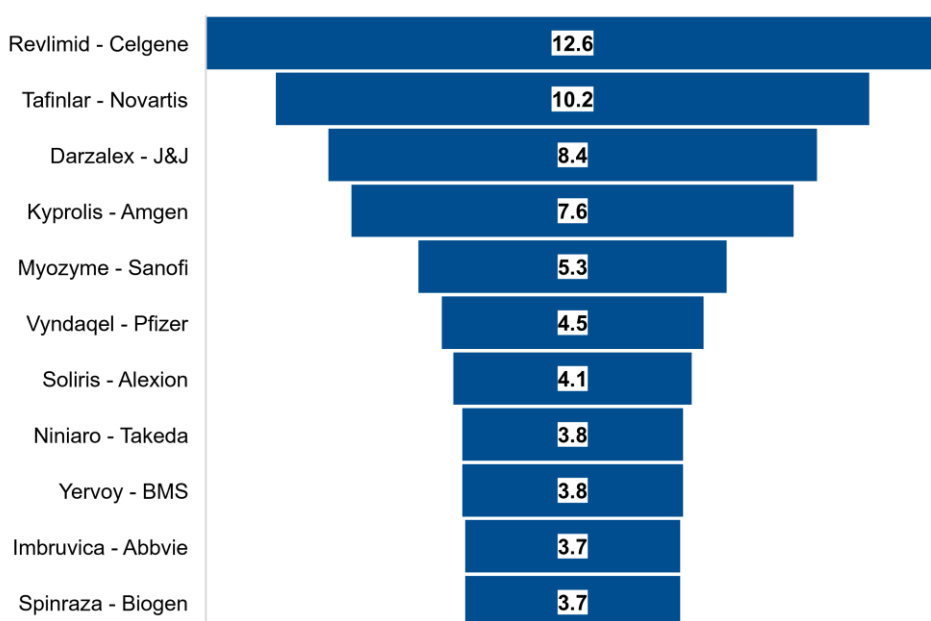
Rare diseases proving lucrative new market

The shift is proving lucrative for the healthcare sector. Profit margins for the world's biggest pharma companies have proved resilient in recent years despite concerns about top-selling drugs losing patent protection, falling prices and rising costs, among other issues.

Earnings before interest, taxes, depreciation and amortisation at the biggest drug makers by revenue - Novartis, Pfizer, Roche and Sanofi – held steady at 25% to 40% of revenue between 2014 and 2018.

Helped by orphan drug sales, the big pharmaceutical firms can be confident of similar stability in the years ahead despite a gloomier global economic context.

Figure 2: Top 10 orphan drugs by sales, 2018 (USD bn)



Source: Scope, EvaluatePharma



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