BIOBUSINESS BRIEFS

MARKET WATCH

Are orphan drug companies the pick of the pharmaceutical industry?

There is a common perception that companies that focus on drugs for rare diseases — orphan drugs — offer opportunities for greater returns on investment compared to other companies, owing to factors such as the higher pricing, financial incentives, accelerated regulatory filings and smaller sales force requirements for orphan drugs. Here, we analyse whether the evidence supports this perception by comparing 5 years of historical financial data (from 2007 to 2011) from specialized orphan drug companies with data from other companies that focus most of their research and development (R&D) efforts on drugs for non-rare diseases. Our data set included 33 publicly listed companies, divided into five baskets of six to seven companies in the following groups: specialized orphan drug companies, large, medium-sized or small pharmaceutical companies, and biotechnology companies (FIG. 1; see Supplementary information S1 (box) for details of the data set and methodology).

The average gross margin of our sample of orphan drug companies was 11 percentage points higher than that of non-orphan drug companies across all categories considered (85.9% versus 74.8%; FIG. 1). This higher gross margin for orphan drug companies results from the relatively higher pricing and market exclusivity associated with their products. We believe that the perception of the financial success of orphan drug companies mainly derives from this financial indicator.

However, taking into account the commitment to R&D by these groups of companies reveals a contrasting perspective. R&D investment as a percentage of sales in our sample of orphan drug companies was on average twice as high as that of companies in the other groups, accounting for 33.9% versus 17.3% of sales, respectively (FIG. 1).

As a consequence of higher R&D costs, the operating profitability of orphan drug companies (as measured by the EBITDA margin: earnings before interest, tax, depreciation and amortization divided by total revenue) was half that of the other companies sampled (15.6% versus 32.3%; FIG. 1). Furthermore, when assessing return on equity (ROE) for investors, ROE among orphan drug companies was one-third lower than the average return observed in the other baskets (FIG. 1). Average return on accounting equity in our sample of orphan drug companies was 8.7% (versus 23.2% for other groups) and average ROE on market capitalization was 1.9% (versus 6.8%).

So, our analysis, using multiple financial indicators, suggests that orphan drug companies have not been performing as strongly relative to other companies in recent years, as commonly perceived. Limitations of the analysis include the generalizability of the sample of companies used and the study period, and the selection of financial indicators (discussed further in supplementary information S1 (box)). Nevertheless, the results support the need to retain existing regulatory and financial incentives for sponsors to promote the sustained development of orphan drugs, as the vast majority of rare diseases still lack therapeutic options.

However, as more orphan drugs become available, there is concern that current pricing practices for orphan drugs, which some have criticized as opaque and socially irresponsible, may become unsustainable. Although relatively higher prices for orphan drugs may be justified considering the level of R&D investment and the small sizes of the patient populations, there may be a case for a more transparent and value-based pricing framework, in which prices would be set in reference to the benefits brought by the drugs, rather than solely based on their orphan status.

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Competing interests statement
The authors declare competing interests: see Web version for details.

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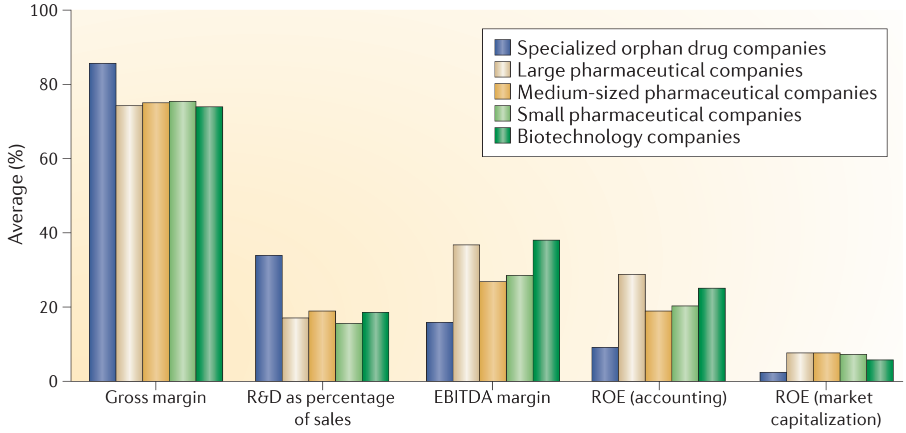

Figure 1 | **Comparison of the performance of specialized orphan drug companies with other companies.** The graphs show the mean results over the period 2007–2011 for five baskets of six to seven companies, drawn from 33 publicly traded companies. The following financial indicators for each basket were analysed: gross margin; spending on research and development (R&D) as a percentage of sales; EBITDA margin (a measurement of a company's operating profitability), equal to earnings before interest, tax, depreciation and amortisation (EBITDA) divided by total revenue; return on accounting equity; and return on equity (ROE) on market capitalization. For details of the data set and analysis methods, see Supplementary information S1 (box).