

## Jazz plays the M&A song again



[Jonathan Gardner](#)

Jazz Pharmaceuticals' most expensive transaction yet, the \$1bn buyout of the Italian rare disease specialist Gentium, gives the group access to one of the most valuable orphan drugs awaiting launch, the veno-occlusive disease treatment Defetelio.

From a strategic perspective the acquisition makes sense, expanding Jazz's blood cancer portfolio as well as its European footprint. Financially Jazz might rely on some positive developments – geographic expansion or approval in new indications – to extract a value from the transaction that merits the price. Still, it was the sort of deal that the company had been trailing, and it evidently pleased investors as shares rose 8% to \$124.08 in early trading.

### On the launch pad

Defetelio recently received European Union approval to treat veno-occlusive disease in patients receiving hematopoietic stem cell transplantation therapy; the chemotherapeutic conditioning necessary to prevent immune system rejection of transplanted tissues can damage hepatic blood vessels and result in organ damage.

As an orphan drug it commands significant leverage with payers and, consequently, higher prices: analysts from Wedbush estimated a price of €48,000 for an average-sized adult, an amount that can vary because of weight-based dosing. Jazz executives estimate 3,600 cases of veno-occlusive disease each year.

With sales forecast at \$262m in 2017, according to Wedbush, this is one of the more valuable orphan drugs in late-stage development or awaiting launch; certainly, it has the uncommon distinction of having yet to fall into the hands of big pharma developers. The relatively low cost of marketing and selling orphan drugs, along with their potential return on investment, made it an attractive target ([Orphan diseases' appeal lies in return on investment](#), April 23, 2013).

For Jazz, the opportunity to pair marketing of the Gentium drug with the leukaemia chemotherapy Erwinaze made the asset that much more attractive; the haematological cancer specialists now using Erwinaze will be logical targets for Defetelio. Chief executive Bruce Cozad said the incorporation of Gentium staff would expand Jazz's European commercial presence, adding that he did not expect to cut staff after the deal.

Yet the Jazz executives described the deal as “modestly accretive” in 2014. A European launch for Defetelio in the first quarter of 2014 should drive new revenues; the drug has been generating sales based on named-patient prescriptions since 2009.

The \$1bn price tag, or \$57 a share cash, comes at a 26% premium. Jazz is borrowing \$500m and suspending share buybacks.

### Expand the market

The current market in Europe, which represents half of all stem cell transplants, could be sufficient to make Gentium worth \$1bn. Certainly, the orphan designation gives it exclusivity through 2023, and its manufacturing processes probably provide additional intellectual property protection. As analysts from UBS put it, the deal “isn't inexpensive and the payback will be long”.

However, it would help justify the price if Defetelio were able to expand its market. Gentium withdrew its FDA filing in 2011 because of data issues – regulators challenged how the company had used historical controls. Sigma Tau Group has US commercialisation rights, but milestones and royalties will be due after approval.

In addition there is a potential for development in prevention of veno-occlusive disorder and in graft vs host disease, indications that would not require much additional commercial effort but would expand the population significantly – 32,500 patients undergo haematopoietic stem cell transplantations each year in Europe, and 6,300 are considered at high risk of veno-occlusive disorder.

It could very well be that this deal will be a long-run winner for Jazz. However, expanding the target population for Defetelio would increase confidence.

To contact the writer of this story email Jonathan Gardner in London at [jonathang@epvantage.com](mailto:jonathang@epvantage.com) or follow [@JonEPVantage](https://twitter.com/JonEPVantage) on Twitter

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