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# Bristol-Myers and Amicus affirm industry's love of orphans

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Two $1bn M&A deals disclosed yesterday highlight again – as if any confirmation were really needed – that obtaining orphan drug assets remains a key focus in the industry's effort to expand its R&D pipelines.

Although those acquisitions were made by two very different-sized companies, Bristol-Myers Squibb and Amicus, they have in common the fact that both were for private companies developing mid to late clinical-stage R&D programmes that are expected to enjoy the regulatory and commercial advantages of serving small patient populations.

**Focus on fibrosis**

Bristol-Myers' $1.25bn option to acquire Promedior provides it with a recombinant form of human pentraxin-2, PRM-151, which is in phase II trials for idiopathic pulmonary fibrosis (IPF) and myelofibrosis (MF). The asset has orphan designation in the US and EU for both indications, and holds US fast-track designation.

Bristol-Myers has put down $150m in an up-front that includes a payment for services in support of MF and IPF phase II trials that are expected to start in the coming weeks. The remaining $1.1bn will be payable once the option is exercised, which can occur on completion of either of these trials.

Currently, there is a 104-patient phase II study under way comparing PRM-151 with Jakafi in primary myelofibrosis, post-polycythemia vera MF, or post-essential thrombocythemia MF, according to clinicaltrials.gov; this is due to render data in October 2016.

Meanwhile, Amicus has agreed an outright acquisition of Scioderm, a private US company developing a treatment for epidermolysis bullosa (EB), a rare inherited condition characterised by severe blistering of the skin from even mild physical contact. The headline figure for the deal is $947m, with the bulk of the payments tied to downstream milestones.

Amicus will initially pay Scioderm shareholders $229m, of which $125m is in cash and $104m in shares, and has agreed to make additional further payments of up to $361m on clinical and regulatory milestones and $257m on sales milestones, both payable in cash or stock.

**Valuing the voucher**

Furthermore, Amicus has also agreed that if an FDA priority review voucher is obtained for Zorblisa and subsequently sold on, as is the current trend, Scioderm shareholders will receive 50% of the proceeds or $100m, whichever is the lesser.

AbbVie paid $350m for the last such priority review voucher to be sold, although it is hard to gauge the value of these. Only three have been sold to date, and it is probable that their value was tied more to the circumstances of the purchaser than their inherent value to the original owner. Nevertheless, the approach pioneered here could well become a trend.

Amicus gains Scioderm's lead project, Zorblisa/SD-101, which is in a 130-patient phase III study for EB that is due to read out in the first half of next year. The asset has US and EU orphan drug designations and, indeed, Sciopharm was the first small biotech company to gain a breakthrough therapy designation in 2013.

The deal could be controversial for two reasons. Firstly, Zorblisa is a topical, although proprietary, formulation of allantoin, a compound commercially available at lower doses, and so will have to rely on the orphan drug protection and formulation patents for commercial exclusivity. Secondly, Zorblisa has a somewhat chequered history, with a near miss in a prior phase II study.

This 48-patient trial completed in 2014 tested two different doses of Zorblisa against placebo and showed a 67% reduction in median time to complete wound closure, but this fell short of statistical significance. A post-hoc analysis showed that the higher dose was more effective in treating larger baseline wounds.

Both the Bristol-Myers and Amicus transactions have large contingent payments, so that a proportion of the development risk is retained by the investors of the selling companies. Amicus has one major advantage that is not the case for Bristol-Myers: there is no current treatment for EB and almost no others on the horizon, barring a couple of late preclinical candidates, whereas there are various approved agents for IPF and MF.

Given the multibillion-dollar valuations accorded to late-stage orphan assets, both deals could look smart next year.

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