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Amicus investors untroubled by SD-101 costs

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Amicus investors who shrugged off the failure of SD-101 in its phase III epidermolysis bullosa trial today seem to have forgotten the $229m the group paid to acquire the asset with its purchase of Scioderm.

Although SD-101 had been thrust into the uncomfortable position of being the company’s lead asset after US setbacks to the Fabry disease project Galafold, the small trial size and adjustments to SD-101’s pivotal programme raised questions over its likelihood of success. Nevertheless, discontinuation of SD-101 work was taken as good news, although surely the money used to buy and advance it could have been used for projects making use of Amicus's main enzyme-targeting technology for rare diseases.

Look over there!

SD-101, formerly known as Zorblisa, missed its two primary endpoints of time to wound closure and wound closure at three months, compared with placebo, in the 169-patient Essence trial. While Amicus spoke optimistically about trends in certain subgroups and discussing the results with regulators and physicians, it said it had no plans to initiate any new clinical studies – two extension studies will continue.

The message from the sellside was that this allows the company to focus on the more important US submission of Galafold in Fabry disease, as well as early data from the Pompe disease candidate ATB200/AT2221. Shares rose 5% in early trading today.

This is not necessarily an unwise conclusion – if the sellside is to be believed. Galafold has forecast sales of $306m in 2022, according to *EvaluatePharma* and $167m for SD-101 in the same year. ATB200, meanwhile, carries a $114m forecast, hefty for a phase II rare disease asset.

Management rewards

And yet the failure definitely should prompt some to question the wisdom of the Scioderm acquisition. Epidermolysis bullosa is without doubt a rare disease in dire need of treatments – it was one of the first treatments to receive the FDA’s breakthrough therapy designation – but as a topical therapy treating a skin disorder rather than a lysosomal storage disorder it seemed out of Amicus’s area of focus.

The acquisition was made at around the peak of Amicus’s valuation – and indeed, that of the entire biotech sector – and the group did put its shares to work. As well as $104m in new shares issued to Scioderm’s investors, the up-front consideration included $125m in cash – not a bad return for $36m in venture investment.

Still, $125m is nothing to sneeze at, and now this is dead money to Amicus. In frothier times such failure might have caused more blowback; perhaps shareholders have become more focused since the peak of the boom passed, but rewarding management missteps might not be a winning long-term investment strategy.

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