Evaluate Vantage

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Amylyx aces launch as tofersen's big date approaches

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Breakeven beckons for Amylyx.

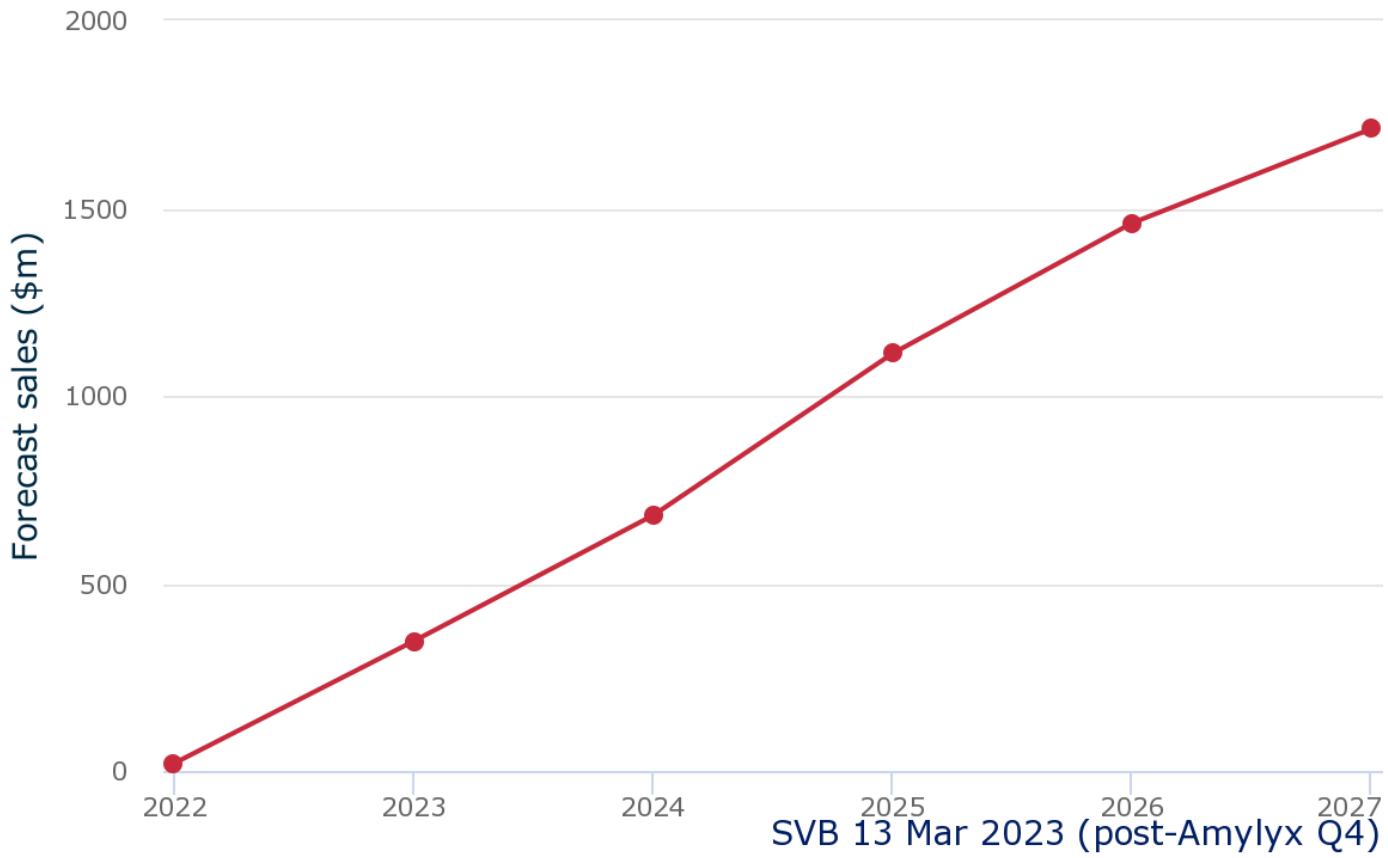
Small companies tend to run into problems when launching new drugs, so it is no wonder that investors were pleasantly surprised by strong early numbers for Amylyx's amyotrophic lateral sclerosis therapy Relyvrio.

The group's stock climbed 16% this morning after fourth-quarter sales of the product, which was launched in the US in October, totalled $22m. This was well ahead of expectations that had already been boosted by a promise of higher-than-expected demand last month – setting Relyvrio up for blockbuster revenues, if Amylyx can keep it on the market.

1,300 patients were on Relyvrio by the end of last year, and the group expects to double this in the first quarter – an estimate that Evercore ISI's Umer Raffat believes could be conservative. He expects Amylyx to come close to breakeven next quarter, which would be an impressive feat so soon after launch.

Will the good times continue? Amylyx execs famously promised to withdraw Relyvrio if the ongoing confirmatory Phoenix trial fails. The group has plenty of breathing space: initial results are due mid-2024 but the focus then will be on functional data. A survival analysis, which will likely be used as the barometer for success, is not due for a further year.

Still, it is hard to see Relyvrio being pulled, particularly if demand stays strong in a disease with so few options. In the long term, Amylyx expects around 10,000 patients to be on Relyvrio at any given time.



Another ALS contender, Biogen and Ionis's tofersen, is aimed at a much smaller niche: disease caused by Sod1 mutations, which affect around 300 patients in the US.

That project is approaching judgement day, with an FDA adcom scheduled for 22 March.

The verdict, and the agency's eventual decision on approvability, will not be needle-moving for the companies: SVB Securities' analysts expect peak sales of just $300m. However, the panel could help gauge sentiment at the FDA's neuroscience division following the departure of its erstwhile chief Billy Dunn, who had been a champion of controversial drugs including Relyvrio.

Tofersen's fate should have been sealed after it flunked the Valor study. However, Biogen and Ionis pressed on with a filing for accelerated approval based on reductions in neurofilament light chain. Bulls will also point to open-label extension data suggesting stabilisation of disease.

Given the fast-moving nature of ALS, and Sod1 ALS in particular, it is understandable that patients want new drugs fast. However, regulators need to balance this desire against potential harms, and tofersen is not without risks: 7% of patients on the drug had serious neurologic adverse events.

This story has been updated to clarify Relyvrio's approval status.