

Alexion turns to Europe for its next rare disease bet



[Jacob Plieth](#)

Lingering doubts about Alexion's ability to switch patients from its Soliris cash cow to the follow-on asset ALXN1210 mean that the company must keep its foot on the takeover pedal - hence today's acquisition of Sweden's Wilson Therapeutics.

By Alexion's standards the move by no means threatens to break the bank, but it does give it a shot at a new rare disease. For Wilson's investors this is a fantastic result, equating to a near-fivefold return less than two years after IPO, and without the group even having to shell out for pivotal trials.

Wilson is a one-asset company focused on WTX101 for Wilson's disease. Phase II data at East a year ago, followed by an agreement with the US FDA and EMA on phase III design in October, was enough for Alexion to strike with [today's \\$855m takeout](#), a 70% premium to Wilson's previous closing price.

That said, Wilson's investors might wonder what might have been had their company been listed in the US, where biotech valuations are far higher than on the other side of the Atlantic. That Alexion could pick up a phase III rare disease asset for under \$1bn was likely an attraction.

Wilson trails by some distance Alexion's biggest recent takeout, that of Synageva for \$8.4bn. That 2015 move now looks like one of the most overpriced deals in biotech history, and Alexion will have been keen to avoid making a similar mistake.

Wilson's disease

Its investors should find Wilson easily digestible, and could in WTX101 see a novel way to tackle Wilson's disease.

This is an autosomal recessive metabolic disease in which copper builds up in the liver and brain. Wilson's approach is a shot across the bows for Valeant, which controls the treatment market via the first and second-line copper-chelating agents, Cuprimine and Syprine respectively.

WTX101 (bis-choline-tetrathiomolybdate) is a superoxide dismutase inhibitor that on today's analyst call Alexion said had the potential to become the standard of care. Preclinical studies have shown the project to clear copper in the liver - a key differentiating factor versus current chelating agents.

Alexion said this property meant WTX101 addressed the underlying physiology of Wilson's disease. It must be stressed, however, that it does not address the condition's underlying cause, a mutation in the *ATP7B* gene - something that can only a gene therapy could treat.

In phase II four weeks of WTX101 treatment lowered free serum copper by 72% from baseline after 24 weeks, and this effect was sustained at 48 weeks. Liver status was said to have stabilised or improved in most subjects.

After extensive regulatory discussions a phase III study began in February, testing as primary endpoint copper control, measured by the same NCC-corrected score used in phase II. In what Alexion said was the first ever randomised, controlled trial in Wilson's disease, second-line and treatment-naive cohorts will receive standard of care or WTX101 for 48 weeks.

Superiority?

The key unknown here is whether this design will, as Alexion desperately wants, differentiate WTX101 from Valeant's standard of care. At present the phase III trial aims to show non-inferiority (within a 15-point margin), but Alexion today strongly hinted that it could amend the design to show superiority.

It might well need this; not only did Cuprimine and Syprine together sell \$169m last year, but [as of February both are subject to generic competition](#). Interestingly, Valeant acquired both drugs through its 2010 purchase of Aton Pharma, after which it hiked their prices.

Of course Alexion itself is no stranger to orphan drug pricing, though right now its main focus remains on switching Soliris patients to ALXN1210 before the former's patent expires in 2027 ([Alexion and the cash cow that never runs out of milk](#), March 15, 2018).

Jitters about ALXN1210's inability to show superiority to Soliris have weighed on Alexion's stock. A market the size of Cuprimine and Syprine might not move the needle too much, but superiority for WTX101, combined with premium pricing, could give Alexion a useful diversification plan.

To contact the writer of this story email Jacob Plieth in London at jacobp@epvantage.com or follow [@JacobPlieth](#) on Twitter

© Copyright 2020 Evaluate Ltd.