Companies Healthcare & Fitness Biotech

The drug developer bucking the biotech sell-off

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Orphan drug developer Neuren Pharmaceuticals is bucking the biotech sell-off. Its share price has almost doubled in the past six months thanks to positive phase three trial results.

The Auckland-born company revealed positive results from its first commercial drug candidate, trofinetide, [https://www.afr.com/markets/equity-markets/neuren-s-shares-double-on-stunning-drug-trial-results-20211208-p59frx]in December.

Neuren Pharmaceuticals CEO Jon Pilcher first joined the business as CFO in 2013. **Eamon Gallagher**

It is aimed at children and adults with a rare genetic neurological and developmental condition known as Rett syndrome, which harms the brain causing a progressive loss of motor skills and speech from as young as six months old.

The syndrome presents differently in each person, but the phase three study of 187 girls and young women aged five to 20 indicated significant improvement in a range of symptoms.

Most participants were non-verbal, but by the end of the 12-week study, many could speak a few words. Another who struggled with motor skills could open a paint pot, hold a brush and paint for the first time.
Despite its share price surge, investors believe the business – which also has a range of phase two trials poised to commence in other rare neurodevelopmental disorders – remains undervalued thanks to the weak sentiment surrounding the risky sector.

Rising value

Hashan De Silva, head of healthcare research at Karst Peak Capital, one of the stock's largest institutional investors, said the company was one of very few local biotech companies to take a drug through to a positive phase three trial result.

"We are very excited for the near future as we believe most of the upside is still to come," he said. "In fact, in our view the risk/reward of the stock is more attractive today than at any point in the past few years, and we added to our position following the phase three readout."

Karst Peak bought a 14 per cent stake in Neuren when it was valued at $1 a share in March 2020. Last Friday, it closed at $3.94, giving it a market capitalisation of almost $500 million.

Neuren's phase three trial was done with US partner Acadia, whom it sold its US licensing rights to.

Under the terms of the deal, Acadia paid for the trial and Neuren will receive $111 million automatically if trofinetide is approved by the US Food and Drug Administration, and an additional $US350 million or so in payments for hitting various sales targets.

It will also receive double-digit royalties on sales in the US, which Karst Peak estimated could be about $85 million to $100 million a year.

The company, which is led by chief executive Jon Pilcher, was created in the early 2000s from research and technology at Auckland University.

Early work

Neuren started out developing therapies for traumatic brain injury sufferers in conjunction with the US Army. The business made a critical pivot in 2012, switching its focus to the treatment of neurodevelopmental diseases and relocating its headquarters to Melbourne.

Neuren has developed synthetic analogues of two fragments of a critical protein in the brain called human insulin-like growth factor 1 (IGF-1), which ongoing research is showing helps the brain build and maintain connections as its develops.

"What we're trying to do is kick-start the IGF-1 to work properly. We're almost trying to get the body to fix itself," Mr Pilcher said. "It's a patient-friendly drug which is important to this population. It's a liquid dose, but it's not like a syrup, it's like a cordial, and they take it twice a day.

"Any improvement is a big deal for these families."

Although the US licensing rights have been sold to Acadia, Neuren retains the rights in the rest of the world.

Success is close

Trofinetide will probably be Neuren's first commercial success. However, Mr Pilcher said he considered its second candidate, NNZ-2591, the jewel in Neuren's crown.

NNZ-2591 is still focused on getting IGF-1 to function, but does so through a different molecule.

Neuren already has approval for phase two trials of NNZ-2591 to take place in a range of conditions, including Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. The Angelman trial will take place in Australia at hospitals in Brisbane, Sydney and Melbourne.

Other investors in Neuren include Milford Asset Management [https://www.afr.com/markets/equity-markets/meet-milford-a-new-type-of-fund-manager-20190625-p520zt] and Antares.

Milford portfolio manager Will Curtayne said the fund had held a small number of shares since 2007, but it increased its position materially when the company raised capital in 2020, and then it went substantial after the phase three results.

"We put the likelihood of FDA approval at 90 per cent to 95 per cent," he said.

"When it's trading at $4, we think it's worth much more than that from this one drug alone.

"With NNZ-2591, it's a new and improved way of tackling the same problem as trofinetide, and given trofinetide works, that gives Neuren confidence it will too.

"We can get the company to $10 per share on trofinetide alone, but if the rest work, it's worth significantly more than that."

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Mr Curtayne said he expected Neuren to partner with a pharmaceutical company, possibly Acadia again, to help commercialise trofinetide across the rest of the world.

Already being close to FDA approval, he said, put Neuren in the box seat to negotiate more favourable terms.

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