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Axovant looks to Oxford Biomedica for gene therapy resurrection

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Axovant transforms itself into a new gene therapy company with an old Oxford Biomedica product.

Axovant transformed itself into a gene therapy player today with the licensing of a Parkinson's disease project for $30m up front. The asset has been in development for almost 20 years – Oxford Biomedica put the first incarnation, Prosavin, into the clinic in 2007.

Of course gene therapy is not exactly fringe science now – see Novartis's $8.7bn acquisition of Avexis – but much remains to be proven. Oxford Biomedica shareholders finally have something to celebrate at least; news of the deal sent the company's shares to a 10-year high, possibly on relief that project is finally somebody else's problem.

Oxford's failure to turn its Lentivector manufacturing platform into therapeutic products consigned it to the investment wilderness for years, but the UK company's capacity to supply the now burgeoning gene and cell therapy field is now standing it in good stead. Most notably, it is the manufacturer of the lentivirus used for transfecting the CTL019 CAR-T cells that Novartis uses in Kymriah.

The search for partners for Oxford Biomedica's R&D projects has been going on for some years, its shareholders having run out of patience. This is not something that troubled the investors that piled into Axovant this morning – its shares more than doubled in early trade, though admittedly from a very low base.

New payload

Pavan Cheruvu, who has been chief executive of Axovant since only February, justified the move by saying that the Parkinson's candidate comes with a decade of clinical safety data and a team of experts that have been working on the project for years.

Code-named OXB-102 and renamed AXO-Lenti-PD by Axovant, the product delivers three genes involved in dopamine production. Oxford Biomedica has described it as being tenfold more potent than Prosavin, which disappointed in the clinic.

Although encouraging signals were seen, dopamine production levels in patients treated with Prosavin only rose marginally, and it was concluded that a higher volume of gene therapy would be needed. The new project has a different payload configuration, which the companies believe will boost effectiveness.

AXO-Lenti-PD is intended as a one-time injection into the brain, with the ultimate goal being a more stable disease state for sufferers, who should also be able to reduce the amount of L-dopa they need to take.

Bearing fruit?

Mr Cheruvu said on a conference call this morning that Axovant aims to dose the first patient in a phase I/II study by the end of the year. The trial will be in two stages, firstly testing three doses, then progressing to a sham-controlled section; patients will be followed for up to 10 years.

He also pledged to bring in more “assets of this quality” by the end of the year. Going by some of the hires he has made this shift into more cutting-edge technologies looks serious. Today Axovant also announced the appointment of Fraser Wright, a co-founder of Spark Therapeutics, to head its gene therapies programmes. Michael Hayden, who was involved in the discovery and development of the ultimately ill-fated gene therapy Glybera and who was most recently Teva’s R&D boss, was recently appointed to head Axovant’s scientific advisory board.

In the coming weeks these executives will be forced to defend the decision to put money into an elderly project that is some way behind the leading Parkinson’s gene therapy, from Voyager Therapeutics. The two assets are different - Voyager is using an AAV vector to deliver the AADC gene, while AXO-Lenti-PD uses a lentivirus to deliver three genes, one of which is AADC.

Mr Wright, who was on the call, said lentiviral vectors have a good safety record and a higher packaging capacity than AAV, allowing the delivery of more genes. Voyager’s results have been far from emphatic, so presumably Axovant sees room for improvement (*Voyager still has a long way to go with Parkinson’s gene therapy, March 9, 2018*)

The catastrophic failure of intepirdine, Axovant’s much-hyped Alzheimer’s disease project, means that many investors will be hard to convince. But the firm is in dire need of new direction and with the seeming unfailing financial support of Vivek Ramaswamy – who injected another $20m into Axovant today via his Roivant vehicle – perhaps long term bets like gene therapy are a plausible way to go.

The shameless use of “bio-dollars” in the press release aside, the deal does not look too costly for now, particularly for a company with deep pockets. As well as $30m up front for worldwide rights, further milestones could reach $812m, with undisclosed royalties on any sales.

For Oxford Biomedica, its decades-long focus on gene therapy is finally bearing fruit. Axovant will be hoping to deliver progress much more quickly.

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