Reneuron eyes a partner with new stem cell data

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Promising retinitis pigmentosa results have given Reneuron a boost, but the company still faces a pivotal trial.

Last year Reneuron teased investors with the prospect of a partner for its retinal stem cell programme, only for this to come to nothing.

Now the UK company has fresh bait to help it hook a partner, with promising but early data with the project in retinitis pigmentosa. The group's stock climbed as much as 20% today – albeit from a very low base – after it reported improvements in visual acuity in three patients in a phase I/II trial.

Reneuron's chief executive, Olav Hellebø, told Vantage that the company hoped to partner its human retinal progenitor cells (hRPCs) outside Europe, but that it was prepared to carry out a registrational trial and launch the project itself if necessary.

Going it alone might not be easy. The company’s cash pile should last just over 12 months, Mr Hellebø said – but he conceded that the current burn rate did not include the money needed for a pivotal study.

Reneuron is enrolling patients into a much-delayed phase IIb trial of its lead project, ReN001, in stroke. Doubts about this candidate were raised last month after the failure of another stem cell stroke asset, Sanbio’s SB623 (Sanbio brings its Japanese retail fans down to earth, January 29, 2019).

Better than expected

If ReN001 turns out to be a dead end Reneuron now at least looks to have a backup in the shape of the hRPC project.

In the phase I/II trial retinitis pigmentosa patients received a single injection of the cells in the worst of their eyes, while their other eye remained untreated. In the treated eyes there was a mean 16-letter improvement on the ETDRS chart, versus a mean one-letter worsening in the untreated eyes.

“This is much stronger than we thought we would see so early on,” Mr Hellebø said. Stifel analysts noted that products in other eye disorders have got the go-ahead based on five to 15-letter improvements.

Still, Reneuron will have to show a similar improvement in more patients. The company plans to enrol another three patients into the phase II part of the trial in March; these will have better baseline vision than the first three.
The chief exec said it was still unclear what the optimal baseline vision level was, explaining: “We thought we’d have to push into better seeing eyes.”

Reneuron has the option to add another six patients in phase II, but Mr Hellebø said it was unlikely to do so. The trial had already enrolled 12 patients in its phase I section, primarily to assess safety. More data are due in mid-2019.

**One more trial?**

Reneuron now plans to speak to regulators about the path forward. This is likely to include at least one more trial, Mr Hellebø said, but the lack of approved therapies could help Reneuron. Spark's gene therapy Luxturna, which targets retinal diseases associated with RPE65 mutations, “treats a tiny sliver of the population”, according to the chief exec.

He contended that retinitis pigmentosa was probably not an ideal candidate for gene therapy, as it was "caused by 100 different genes or more". This has not stopped companies like Meiragtx, which recently signed a deal with Johnson & Johnson *(Johnson & Johnson dips a toe into gene therapy, February 1, 2019)*.

Mr Hellebø said Reneuron had no plans to raise money off the back of the recent data, and was only looking at non-dilutive forms of funding.

The group already tried – and failed – to find a partner for the hRPC project. Last July Reneuron said it was in exclusive talks with an unnamed US speciality pharma company, which had paid $2.5m for the privilege, but the potential partner later pulled out.

As well as looking to partner the hRPC projects in the US, Reneuron is also open to deals in Asia for both the hRPC and stroke programmes. And the group could also strike more agreements for its exosome technology, which recently tempted an anonymous biopharma company.

The retinitis pigmentosa data represent the best news Reneuron has had for a while, but the group will need to replicate these results in more patients if its resurgence is to continue.