



AIM: RENE

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**ReNeuron Group plc**

**ReNeuron successfully develops cryopreserved formulation of its retinal stem cell therapy candidate and expands ophthalmology programmes**

ReNeuron Group plc (the "Company") (AIM: RENE), a UK-based global leader in the development of cell-based therapeutics, provides an update on its cell therapy development programmes targeting degenerative diseases of the retina.

We are pleased to report that we have successfully developed a cryopreserved formulation of our human retinal progenitor cell (hRPC) therapeutic candidate. The ability to cryopreserve our retinal cell therapy candidate at drug product level represents a major step forward for our retinal disease programme and mirrors the earlier breakthrough we achieved with the cryopreservation of our CTX cell therapy candidate. This new hRPC formulation enables the cells to be frozen for shipping and storage and easily thawed at the point of clinical use. This freeze-thaw modality enables a greatly enhanced shelf life for the product, lower prospective cost of goods and the capability to ship the cells for clinical and commercial application anywhere on the globe.

As a result of the above development, we will shortly file an application to the FDA seeking approval to switch from the fresh hRPC formulation to the new cryopreserved formulation for dosing of the third and final Phase I dose cohort of our ongoing US Phase I/II clinical trial in retinitis pigmentosa (RP) patients. This study, which is being conducted at Massachusetts Eye and Ear Infirmary in Boston, is an open-label, dose escalation study to evaluate the safety, tolerability and preliminary efficacy of our hRPC stem cell therapy candidate in patients with advanced RP.

The new hRPC formulation has also allowed an expansion of ReNeuron's clinical programmes in ophthalmology. Firstly, subject to regulatory approval, we intend to expand the Phase II element of the ongoing Phase I/II clinical trial in RP from six to 20 patients in order to provide a richer data set from which to embark on a subsequent Phase II/III pivotal study. In order to maintain patient recruitment pace and reduce reliance on a single clinical site, we also intend to open up further US clinical sites to this study. As a consequence of these changes, we expect safety and tolerability data from the Phase I part of the RP study in the first nine patients later this year, with longer term safety data as well as efficacy read-outs from the enlarged Phase II part of the study in the second half of 2018.

Secondly, we intend to expand our hRPC retinal disease programmes into a further disease indication, cone-rod dystrophy (CRD). In contrast to RP, where the initial impact is a loss of rods leading to a deterioration in peripheral vision and night vision, CRD is a group of rare eye disorders associated with a loss of cone cells in the retina that initially results in deterioration of central visual acuity and colour vision. CRD frequently affects patients in childhood and has no cure. It is an inherited orphan disease that affects roughly 1 in 40,000 people.

The expansion of our ophthalmology programmes into CRD is part of a broader strategy to evaluate the efficacy of our hRPC therapeutic candidate across a range of genetic diseases of the eye. We intend to file an application to commence a Phase II clinical trial later this year in patients with CRD, to be run alongside the Phase II part of the ongoing RP clinical trial.

**Olav Hellebø, Chief Executive Officer of ReNeuron, said:**

“We are delighted to be able to report these very significant positive developments with our retinal disease programme. The successful development of a cryopreserved formulation of our hRPC retinal cell therapy candidate enables an expansion of our clinical programmes in ophthalmology and also gives ReNeuron a significant commercial advantage in terms of prospective cost of goods and ease of use of a retinal disease therapy.

“The expansion of our retinal disease programmes into cone-rod dystrophy and the enlargement of the Phase II part of the ongoing clinical trial in retinitis pigmentosa reflect our strong belief in the potential of our hRPC platform to benefit patients severely affected by retinal diseases where no effective treatments are currently available.”

**ENDS**

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## **About ReNeuron**

ReNeuron is a leading, clinical-stage cell therapy development company. Based in the UK, its primary objective is the development of novel cell-based therapies targeting areas of significant unmet or poorly met medical need.

ReNeuron has used its unique stem cell technologies to develop cell-based therapies for significant disease conditions where the cells can be readily administered “off-the-shelf” to any eligible patient without the need for additional immunosuppressive drug treatments. The Company has therapeutic candidates in clinical development for motor disability as a result of stroke, for critical limb ischaemia and for the blindness-causing disease, retinitis pigmentosa.

ReNeuron is also advancing its proprietary exosome technology platform as a potential new nanomedicine targeting cancer and as a potential delivery system for gene therapy treatments.

ReNeuron’s shares are traded on the London AIM market under the symbol RENE.L. Further information on ReNeuron and its products can be found at [www.reneuron.com](http://www.reneuron.com).

*This announcement contains forward-looking statements with respect to the financial condition, results of operations and business achievements/performance of ReNeuron and certain of the plans and objectives of management of ReNeuron with respect thereto. These statements may generally, but not always, be identified by the use of words such as "should", "expects", "estimates", "believes" or similar expressions. This announcement also contains forward-looking statements attributed to certain third parties relating to their estimates regarding the growth of markets and demand for products. By their nature, forward-looking statements involve risk and uncertainty because they reflect ReNeuron's current expectations and assumptions as to future events and circumstances that may not prove accurate. A number of factors could cause ReNeuron's actual financial condition, results of operations and business achievements/performance to differ materially from the estimates made or implied in such forward-looking statements and, accordingly, reliance should not be placed on such statements.*