

**ReNeuron Group plc**

(“ReNeuron” or the “Company”)

**Positive preliminary data in US retinal trial**

*Significant improvement in vision in first cohort of Phase II subjects in US trial of hRPC cell therapy in retinitis pigmentosa*

ReNeuron Group plc (AIM: RENE), a UK-based global leader in the development of cell-based therapeutics, is pleased to provide an update on progress with the ongoing Phase I/II clinical trial of its hRPC cell therapy candidate in the blindness-causing disease, retinitis pigmentosa (RP).

The Company is pleased to report that all three of the first cohort of subjects in the Phase II part of the trial have reported a significant improvement in vision, on average equivalent to reading an additional three lines of 5 letters on the ETDRS eye chart, the standardised eye chart used to measure visual acuity in clinical trials.

The Phase II part of the Phase I/II trial, which uses a cryopreserved, commercially ready hRPC formulation, enrolls subjects with some retinal functionality, in contrast to the very poor vision and lack of potential for improvement of the subjects in the trial’s Phase I component.

In the latest observations of the first Phase II cohort, at two months follow-up for one subject, and at 18 days for the other two, all three subjects have reported improved vision, with standardised eye chart testing showing objective improvement in visual acuity compared with their pre-treatment baseline vision and compared with the patients’ untreated control eye.

The trial’s principal investigator, Jason Comander MD, PhD, Associate Director, Inherited Retinal Disorders Service, Massachusetts Eye and Ear, and Assistant Professor, Harvard Medical School, said: *“It is exciting to move into a population of patients whose remaining vision and retinal architecture support the possibility of visual improvement and I am very much looking forward to obtaining additional results from this patient cohort and the next.”*

The first Phase II cohort’s other investigator, Pravin Dugel MD, Managing Partner, Retinal Consultants of Arizona, Phoenix, Arizona, and Clinical Professor, Roski Eye Institute, USC Keck School of Medicine, Los Angeles, California, said: *“When I heard about the rapidity and magnitude of the visual gain in the first patient that Jason treated in this cohort, I was hopeful, but sceptical. Now that I saw this repeated in my first two patients, I am very excited indeed.”*

The Company notes that these data are early and it will continue to generate further data, including regular ongoing monitoring of the three treated subjects, to assess durability of effect and efficacy over a longer period of time and in a larger number of patients.

**Summary of the preliminary efficacy data from the three Phase II subjects treated to date**  
(visual acuity measured using the standardized ETDRS chart)

Subject	Visual Acuity at Baseline	Visual Acuity at Latest Review	Latest Review – Post-surgery follow-up point
First subject	9 letters	29 letters	2 months
Second subject	9 letters	24 letters	18 days
Third subject	32 letters	46 letters	18 days

For the first subject in the cohort, visual acuity improved in the treated eye from 9 letters at baseline to 29 letters at two months follow-up; for the second subject, visual acuity improved from 9 letters at baseline to 24 letters at 18 days follow-up; for the third subject, visual acuity improved from 32 letters at baseline to 46 letters at 18 days follow-up. The untreated control eyes did not show significant improvement (mean change from baseline -1 letter, range -5 to + 5 letters). The mean change from baseline in visual acuity for these first three subjects early on in duration of follow up is thus plus 16 letters in the study eye, compared with a mean change from baseline of minus 1 letter in the untreated control eyes. All three subjects have noted a subjective improvement in vision in their treated eye.

The Phase I/II study, which is being undertaken at two clinical sites in the US – Massachusetts Eye and Ear in Boston and Retinal Research Institute in Phoenix – is an open-label study to evaluate the safety, tolerability and preliminary efficacy of the hRPC stem cell therapy candidate in patients with advanced RP. RP is a group of hereditary diseases of the eye that lead to progressive loss of sight due to cells in the retina becoming damaged and eventually dying.

Dosing in the Phase I part of the study was completed last year in twelve subjects with extensive retinal damage and very limited remaining vision to establish the safety of the treatment. The safety and visual stability data from this part of the study resulted in the Data Safety Monitoring Board (DSMB) approving the transition to a commercially ready hRPC drug product formulation to be administered to patients with less retinal damage and consequently better visual potential.

Dosing of the second cohort of three Phase II subjects is expected to commence in March 2019 following DSMB review of the clinical data from the first Phase II cohort. Based on the positive early data thus far from the Phase I/II study, the Company intends to seek regulatory advice regarding the optimal clinical development path for its hRPC candidate in RP.

The Company's RP clinical programme benefits from Orphan Drug Designation in both Europe and the US, as well as Fast Track designation from the FDA. As previously reported, the Company expects to report further short term read-outs in mid-2019 from the ongoing Phase I/II study.

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

“We are excited by both the speed and extent of improvement observed in this first patient cohort in the Phase II part of the ongoing Phase I/II study with our hRPC cell therapy candidate in retinitis pigmentosa. We will continue to generate further data to assess durability of effect

and efficacy in a larger number of patients in the months ahead and will also consult with the FDA and EMA in order to establish the optimal development pathway to market approval for this pioneering therapy.”

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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 disability as a result of stroke and for the blindness-causing disease, retinitis pigmentosa.

ReNeuron is also advancing its proprietary exosome technology platform as a potential delivery system for drugs that would otherwise be unable to reach their site of action.

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 inside information. The person responsible for arranging for the release of this announcement on behalf of the Company is Olav Hellebø, Chief Executive Officer.*

*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.*