

**ReNeuron Group plc**  
 (“ReNeuron” or the “Company”)

**Clinical update and conference presentation**

*Latest results show sustained and further improvement in vision at 60 and 120 days in first patient cohort of Phase 2a study of hRPC cell therapy in retinitis pigmentosa*

ReNeuron Group plc (AIM: RENE), a global leader in the development of cell-based therapeutics, is pleased to announce updated positive preliminary data in the Company’s ongoing Phase 1/2a clinical trial of its human retinal progenitor cell (hRPC) therapy candidate in the blindness-causing disease, retinitis pigmentosa (RP). All three subjects in the first cohort of the Phase 2a element of the study have demonstrated a sustained and further improvement in vision compared with their pre-treatment baseline.

These latest results are being presented today by Jason Comander MD, PhD, Associate Director, Inherited Retinal Disorders Service, Massachusetts Eye and Ear, and Assistant Professor, Harvard Medical School, at the sixth annual Retinal Cell and Gene Therapy Innovation Summit in Vancouver, Canada, which precedes the 2019 annual meeting of the Association for Research in Vision and Ophthalmology (ARVO) taking place on 28 April – 2 May.

**Summary of the preliminary efficacy data** (visual acuity measured using the standardised ETDRS chart, five letters per line):

Subject	Visual Acuity at Baseline	Improvement vs Baseline	
		Initial <sup>1</sup> (time point)	Further <sup>2</sup> (time point)
First subject	9 letters	+ 20 letters (60 day follow-up )	+ 21 letters (120 day follow-up)
Second subject	9 letters	+ 15 letters (18 day follow-up)	+ 25 letters (60 day follow-up)
Third subject	32 letters	+ 14 letters (18 day follow-up)	+ 23 letters (60 day follow-up)

<sup>1</sup> 20 February 2019 update

<sup>2</sup> 26 April 2019 update

At most recent follow-up, subjects in the study showed a mean improvement from baseline in visual acuity of + 23 letters in the treated eye. The untreated control eyes did not show meaningful improvement (mean change from baseline of + 5 letters, range - 2 to + 12 letters).

An improvement of + 23 letters is equivalent to reading an additional four lines of letters on the ETDRS eye chart, the standardised eye chart used to measure visual acuity in clinical trials. An improvement of at least + 15 letters from baseline on the ETDRS chart is considered to be clinically meaningful by the US Food and Drug Administration (FDA), as stated in their recent guidance on gene therapy for retinal disorders. As a comparator, the difference between a patient with 20/20 vision and 20/200 vision (the latter being the legal definition for blindness in terms of central visual acuity) would be the equivalent of being able to read an extra ten lines on the ETDRS chart.

In addition to these objective measurements, all three subjects have also noted a subjective improvement in vision in their treated eye.

**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, and study investigator, commented:**

“I am excited that the rapid and remarkable visual improvement in these RP patients has been sustained and even improved upon at 60 and 120 days. It is especially gratifying to feel the excitement and joy in my patients where this objective and subjective improvement in their vision has been a source of hope following years of slow but steady progression towards blindness.”

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. The Company’s RP clinical programme has been granted Orphan Drug Designation in both Europe and the US, as well as Fast Track designation from the FDA.

The Phase 1/2a clinical trial is an open-label study to evaluate the safety, tolerability and preliminary efficacy of ReNeuron’s hRPC stem cell therapy candidate in patients with advanced RP. The Phase 2a element of the study, which uses a cryopreserved hRPC formulation, enrolls subjects with some remaining retinal function and is being conducted at two clinical sites in the U.S. – Massachusetts Eye and Ear in Boston and Retinal Research Institute in Phoenix, Arizona.

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

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

“The extent of vision improvement observed in this patient cohort demonstrates the potential for our hRPC cell therapy candidate to make an enormous difference in the lives of patients with RP. Treatment has already begun in the next cohort of patients, who have a greater baseline level of visual acuity than those treated so far. The results from this cohort will be presented in due course.”

**ENDS**

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## About ReNeuron

ReNeuron is a global leader in cell-based therapeutics, harnessing its unique stem cell technologies to develop 'off-the-shelf' stem cell treatments, without the need for immunosuppressive drugs. The Company's lead clinical-stage candidates are in 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 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.*