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AIM: RENE

**ReNeuron Group plc**  
("ReNeuron" or the "Company")

### **Reports positive results in Phase II stroke trial**

ReNeuron Group plc (AIM: RENE), a UK-based global leader in the development of cell-based therapeutics, is pleased to announce positive data from the Company's Phase II clinical trial (PISCES II) of its CTX cell therapy candidate for stroke disability.

PISCES II is a single arm, open-label study in patients living with disability resulting from ischaemic stroke. The study's primary endpoint was for two patients to reach a minimum two-point improvement in the grasping and lifting test, sub-test number 2, of the Action Research Arm Test ("ARAT"), at three months post-treatment. Three of the 21 patients achieved this at three, six or twelve months respectively after treatment and were within a group of four responders who also showed clinically relevant improvements on the total ARAT score of arm motor performance. Although the ARAT sub-test number 2 study endpoint was not met as some responses came later than the three-month target, the result is nonetheless highly encouraging.

Strongly positive results were also seen in the other endpoints of the study, with seven patients (33%) showing a clinically relevant improvement on the Modified Rankin Scale (a measure of disability and dependence) and eight patients (38%) showing a clinically relevant improvement on the Barthel Index (a measure of performance in activities of daily living). In total, 15 out of 21 patients had a clinically significant response on at least one efficacy measure. Improvements in the ARAT scores, Modified Rankin Scale and Barthel Index were all sustained throughout the follow up period.

As a result of the positive data from the PISCES II study, the Company intends to apply to the US and European regulatory authorities to commence a randomised, placebo-controlled, pivotal clinical trial in patients who are living with disability post-stroke.

Patients in the PISCES II study were monitored prior to treatment to ensure that their disability was stable and showing no spontaneous improvement. A dose of 20 million CTX cells was administered to the patients between two and thirteen months (median seven months) after the stroke via direct injection adjacent to the area of the brain with the stroke damage. Patients were monitored for changes in arm function as well as a range of measures to assess disability and activities of daily living. All 21 patients in the study have completed three-month

follow-up, with ten patients followed for six months and three for twelve months. Further data will continue to be collected until all patients have reached 12 months post treatment.

The PISCES II study also demonstrated that the CTX treatment was well tolerated. The most common adverse events were transitory and related to the surgical procedure, such as headache and nausea. Safety and efficacy data from the study will be presented at forthcoming stroke and rehabilitation medical conferences.

**Professor Keith Muir, SINAPSE Professor of Clinical Imaging, Division of Clinical Neurosciences at the University of Glasgow, and Principal Investigator of the PISCES II study, said:**

“The findings of the PISCES II study are encouraging in that the CTX treatment shows improvements both in specific neurological problems, such as arm function, and also in more general disability and independence. These improvements occurred in sufficient numbers of patients to warrant further investigation in a larger, controlled clinical study. Further, the CTX treatment was well tolerated in this Phase II study, which confirms and adds to the results of the earlier Phase I clinical trial. We look forward to the opportunity to further test the efficacy of this potential new treatment for disabled stroke survivors.”

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

“We are delighted that the PISCES II clinical trial has shown our CTX cell therapy candidate has the potential to become a treatment option for patients living with chronic consequences following stroke. We are particularly excited by the response rate seen on the measures relating to disability and activities of daily living, given that these are the most important for patients and their carers. These measures are also the ones viewed by regulatory authorities as most relevant for late-stage clinical development.

“We are extremely grateful to the patients who volunteered for the PISCES II study and to the stroke research teams across the UK who have been involved in this ground-breaking clinical trial.

“Based on these positive results we will move into a controlled clinical trial to further assess the efficacy of CTX treatment in a significantly larger cohort of patients. We have a strong balance sheet to fund this pivotal study as well as reaching other important milestones across our development pipeline.”

**Analyst meeting and webcast:**

A meeting for analysts will be held at 9.00am today at the offices of Buchanan, 107 Cheapside, London, EC2V 6DN. For a webcast of the analyst presentation,

please log on to the following web address approximately 10 minutes before 9.00am:

<http://vm.buchanan.uk.com/2016/reuron051216/registration.htm>

For further details please contact Buchanan on 020 7466 5000.

A recording of the webcast will be made available on ReNeuron's and Buchanan's websites, [www.reuron.com](http://www.reuron.com) and [www.buchanan.uk.com](http://www.buchanan.uk.com).

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#### **About the PISCES II clinical trial**

The PISCES II clinical trial is a UK study of patients with motor disability as a result of ischaemic stroke. Eight centres across the UK's NHS hospital service were involved in recruiting and treating patients. A total of 21 patients were treated between two and thirteen months post-stroke and have been followed-up for at least 3 months. The patients were dosed with 20 million CTX cells which were injected by way of a routine surgical procedure into the putamen, the region of the brain involved in learning and coordinating movement. Patients were typically discharged home following a day of recovery in hospital. Patients in the study also received physiotherapy following their surgery. Arm and leg motor performance was tested in the study using Action Research Arm Test and Fugl-Meyer Assessment. Stroke severity and ability to carry out routine daily tasks were also measured, using the National Institutes of Health Stroke Scale, Modified Rankin Scale and Barthel Index. The PISCES II study was part-funded by a regenerative medicine and cell therapy development grant from Innovate UK.

#### **About ReNeuron's CTX stem cell therapy candidate for stroke disability**

ReNeuron's CTX stem cell therapy candidate for stroke disability consists of a neural stem cell line which has been generated using the Company's proprietary cell expansion and cell selection technologies and then taken through a full

manufacturing scale-up and quality-testing process. As such, CTX is a cryopreserved, clinical and commercial-grade cell therapy product capable of treating all eligible patients presenting.

CTX has been shown to be safe and well-tolerated in an initial UK clinical trial (PISCES I) in eleven disabled stroke patients who were followed up for at least two years post-treatment. The data from this study were recently published in *The Lancet*. If ultimately shown to be safe and effective in larger, controlled clinical studies, CTX would therefore offer a ground-breaking new treatment option for stroke survivors. The therapy offers the potential for a degree of recovery of function in disabled stroke patients, resulting in greater independence and quality of life for these patients and reduced reliance on health and social care systems.

The CTX cells that were used in the both the PISCES I and PISCES II clinical trials were taken from the existing manufactured cell banks that will form the basis of the eventual marketed product. There will therefore be no need to re-derive and test new CTX cell lines for subsequent clinical trials or for the market – all such cells can simply be expanded from the existing banked and tested product.

#### **About stroke**

Approximately 150,000 people suffer a stroke in the UK each year and approximately 800,000 in the US. The vast majority of these strokes are ischaemic in nature, caused by a blockage of blood flow in the brain (as opposed to a haemorrhagic or bleeding stroke).

Approximately one half of all stroke survivors are left with permanent disabilities as a result of the damage caused to brain tissue arising from the stroke. The annual health and social costs of caring for these patients is estimated to be in excess of £5 billion in the UK and over \$70 billion in the US, with stroke patients estimated to be occupying at least 25 per cent of long term hospital beds.

The only current treatment for ischaemic stroke patients occurs in the acute phase of the condition (within several hours of the stroke), when anti-clotting agents are administered to dissolve the clot causing the blockage in blood flow to the brain. Only a small proportion of patients get to the hospital in time to be treated in this way.

Beyond the acute phase, there are no existing treatments, other than preventative or rehabilitation measures, to alleviate the disabilities suffered by stroke patients who have survived their stroke.

*Source: UK Stroke Association; American Stroke Association*

#### **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.*