**7 May 2014** **AIM: RENE**

# Further data from first clinical trial of ReNeuron's stem cell therapy for stroke to be presented at leading stroke conference

## Long term data continue to show good safety profile and evidence of sustained reductions in neurological impairment and spasticity

**Guildford, UK, 7 May 2014:** ReNeuron Group plc (the "Company") (AIM: RENE.L) today provides a further update on the PISCES Phase I clinical trial of its ReN001 stem cell therapy for disabled stroke patients ahead of the commencement of a Phase II efficacy study for which patient enrolment has now opened.

Long term follow-up data out to 12 months in all patients treated in the PISCES study are being presented in two platform presentations by the clinical team from Glasgow's Southern General Hospital at the 23rd European Stroke Conference, taking place in Nice, France this week.

There were no cell-related or immunological adverse events reported in any of the eleven patients treated in the study. Adverse events were related only to the implantation procedure or underlying co-morbidities. Sustained reductions in neurological impairment and spasticity were observed in most patients compared to their stable pre-treatment baseline performance, reflected in the summary evaluation scores below (n=11 patients):

- National Institutes of Health Stroke Scale (measure of neurological deficit):
  - Trial inclusion criteria require a score of 6 or more, representing stable moderate to severe disability (total of 2 or more for motor arm and leg scores)
  - Median pre-treatment score = 7
  - Post-treatment scores improved by median 2 points at 3 months and 3 points at 12 months

- Barthel Index (measure of independence in performing activities of daily living, rated 0-20):
  - Median pre-treatment score = 12
  - Post-treatment scores improved by median 1 point at 3 months and 3 points at 12 months

- Summated Ashworth Score (aggregated measure of spasticity in affected limbs, rated 0-40 arm, 0-25 leg):
  - Mean pre-treatment aggregate score = 29
  - Post-treatment scores improved by mean 5 points at 3 months and 7 points at 12 months

- Modified Rankin Score (overall measure of disability and handicap, rated 0-6):
  - Median pre-treatment score = 3
  - Score improved by 1 grade in n=4/11 patients at 12 months, with n=7/11 patients unchanged

- EuroQOL score (quality of life outcome measure, rated 0-100):

- Median pre-treatment score = 45
- Post-treatment scores improved by median 18 points at 12 months.

Preliminary functional MRI data in seven of the treated patients at resting state show, at a group level, evidence of increased short-term connectivity between the cell-implanted region of the brain (the putamen) and the other deep brain regions that are concerned with sensory motor control, although relevance to functional outcomes in the patients requires further evaluation.

The Company recently announced that it had received unconditional approval to conduct a UK multi-site Phase II clinical trial to examine the efficacy of ReN001 in patients disabled by an ischaemic stroke. This Phase II study is now open for patient enrolment at the Glasgow clinical site, with other UK centres expected to follow, as required, over the coming weeks and months. The study will involve the treatment of up to 41 patients between 8 and 12 weeks after their stroke. Patients will be monitored on a number of validated stroke efficacy measures up to six months post-treatment. The treatment window in the Phase II clinical trial is regarded as optimal in terms of the potential efficacy of the ReN001 therapy and differs from the treatment window in the PISCES Phase I clinical trial where patients were treated at least 6 months after their stroke.

Michael Hunt, Chief Executive Officer of ReNeuron, said:

"The PISCES study has continued to yield encouraging results in longer term follow up of the patients treated with ReN001, providing further evidence of the safety of the treatment and some evidence of the potential positive effects of the treatment against baseline measures of disability. This is gratifying and bodes well for our recently approved Phase II clinical trial with ReN001."

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

"We are pleased and encouraged by the data from the PISCES study. The long term follow-up data continue to demonstrate the safety and tolerability of the ReN001 treatment. The evidence of functional improvement and early imaging data warrant further investigation and, in this regard, we look forward to being a principal participating centre in the recently approved Phase II efficacy study."

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## About the PISCES clinical trial

The PISCES study is the world's first fully regulated clinical trial of a neural stem cell therapy for disabled stroke patients. Stroke is the third largest cause of death and the single largest cause of adult disability in the developed world. The trial is being conducted in Scotland at the Institute of Neurological Sciences, Southern General Hospital, Greater Glasgow and Clyde NHS Board.

The primary aim of the study is to test the safety and tolerability of the treatment in ascending doses of the ReN001 cells, in patients with moderate to severe functional neurological impairments resulting from their stroke. The secondary aim of the study is to evaluate efficacy measures for the design of future clinical trials with ReN001, including imaging measures as well as a number of tests of sensory, motor and cognitive functions.

## 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's ReN001 stem cell therapy for stroke

ReNeuron's ReN001 cell therapy for stroke consists of a neural stem cell line, designated *CTX*, 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, ReN001 is a standardised, clinical and commercial-grade cell therapy product capable of treating all eligible patients presenting.

ReN001 has been shown to reverse the functional deficits associated with stroke disability when administered several weeks after the stroke event in relevant pre-clinical models of the condition. Extensive pre-clinical testing also indicates that the therapy is safe, in both acute and long term safety studies.

If ultimately shown to be safe and effective clinically, ReN001 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 ReN001 cells that are being used in the PISCES clinical trial are 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 ReN001 cell lines for subsequent clinical trials or for the market – all such cells can simply be expanded from the existing banked and tested product. The Company recently gained UK regulatory approval to use a second-generation cryopreserved variant of its lead stem cell line, designated *CTXcryo*, in a Phase II study with ReN001.

**About the Institute of Neurological Sciences at the University of Glasgow**

The clinical Stroke Research Group of the Division of Clinical Neurosciences is based at the Institute of Neurological Sciences at the University of Glasgow and has major collaborations, internally with the Glasgow Experimental MRI Centre, with SINAPSE (Scottish Imaging Network: A Platform for Scientific Excellence), and with the Translational Medicine Research Initiative (TMRI). Around 900 patients per year are admitted through the Acute Stroke Unit, which provides stroke services to the population of south Glasgow and specialist stroke treatments for the West of Scotland.

The unit is the highest user of acute clot-busting (thrombolytic) treatment in the UK at present, and has been extensively involved in clinical trials in stroke. Major research interests include evaluation of advanced brain imaging techniques in acute stroke, development of novel brain imaging techniques, improving the use of clot-busting drug treatments in stroke, and developing trial methodology for evaluation of regenerative treatments. The group has support from the Stroke Association, the Medical Research Council, and the TMRI. Further work with regenerative strategies include collaborations with groups developing both drug-based and stem cell therapies across Europe.

**About ReNeuron**

ReNeuron is a leading, clinical-stage cell therapy development business. 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’s therapeutic candidates for stroke disability and critical limb ischaemia are in clinical development and its cell-based treatment for blindness-causing diseases of the retina is currently in pre-clinical development.

ReNeuron is also advancing a proprietary platform technology to exploit nanoparticles (exosomes) secreted by stem cells as potential new drug candidates targeting indications in tissue repair, fibrosis and cancer.

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.

*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.*