

## **Living Cell Technologies Limited**

ACN: 104 028 042 ASX: LCT OTCQX: LVCLY

### ASX ANNOUNCEMENT

# NTCELL® Clinical Study in Parkinson's Meets Endpoints

Living Cell Technologies Advancing Regenerative Cell Therapy into Larger Phase IIb Study to Evaluate Its Potential as a Disease-modifying Treatment

Company to Host Conference Call on Thursday 18 June at 8:30 a.m. AEST / 10:30 a.m. NZT

**15** June 2015 – Sydney, Australia, and Auckland, New Zealand – Living Cell Technologies Limited (LCT) today announced results from a Phase I/IIa clinical study of NTCELL<sup>®</sup>, an experimental regenerative cell therapy being studied as a disease-modifying agent in Parkinson's disease. The study, conducted in four patients in New Zealand, met its primary endpoint of safety, showing NTCELL implantation was well tolerated, with no adverse events considered to be related to NTCELL. NTCELL implantation also improved clinical features of Parkinson's disease in the four patients studied, as measured by validated neurological rating scales and questionnaires, with the improvement sustained at 26 weeks post-implant.

The study results will be presented at the 19<sup>th</sup> International Congress of Parkinson's Disease and Movement Disorders in San Diego on Wednesday 17 June, by principal investigator Barry J. Snow, MBChB, FRACP, in a Guided Poster Tour presentation. The poster will be available for viewing starting Monday 15 June at 9:00 a.m. Pacific Daylight Time.

"Currently, clinicians are able to manage only symptoms in patients with Parkinson's disease as there are no disease-modifying treatments available that can reverse the underlying progressive degeneration of neurons in the brain," said Dr. Snow, a neurologist in the Department of Neurology at Auckland City Hospital and medical director of Adult Medical Services at the Auckland District Health Board. "The positive clinical response observed in this small study of four patients is encouraging and I look forward to evaluating efficacy in a larger study."

Ken Taylor, Ph.D., chief executive officer of LCT, said, "NTCELL is the most advanced and only cellbased therapy currently in a clinical trial to target regeneration of brain cells in patients whose symptoms cannot be controlled by current therapies for Parkinson's disease. While this regulatoryenabling study is small in scale, the secondary endpoint efficacy results are sufficiently encouraging to warrant further studies. We are advancing the clinical development of NTCELL and will use the results of this study to design a larger registration-enabling Phase IIb study to evaluate its potential as a disease-modifying treatment for patients with Parkinson's disease."

LCT plans to initiate the confirmatory Phase IIb study in the fourth quarter of 2015. The study will be led by Dr. Snow at Auckland City Hospital. The efficacy and safety endpoints will be the same as those evaluated in the Phase I/IIa study.

#### Phase I/IIa study design and results

The open-label Phase I/IIa clinical study evaluated the safety and clinical effects of implantation of NTCELL, which contains specialised brain cells that produce cerebrospinal fluid (CSF) and neuroactive growth factors, into patients who had been diagnosed with Parkinson's disease for at least five years and who no longer responded to current therapy. The study was conducted at Auckland City Hospital in four patients aged 59 to 68 years at the time of consent. NTCELL was injected under guidance by neuroimaging into the affected area of the brain where neural activity was substantially diminished or degenerated. No immunosuppressive drugs were used.

The primary endpoint of the study was the safety of NTCELL implantation, which was assessed by the occurrence of adverse events and serious adverse events, as well as clinical and laboratory evidence of porcine endogenous retrovirus (PERV) in study participants and their partners. The secondary endpoint was efficacy, which was measured by validated neurological rating scales and questionnaires, including the Unified Parkinson's Disease Rating Scale (UPDRS), the Unified Dyskinesia Rating Scale (UDysRS) and the Parkinson's Disease Quality of Life Questionnaire (PDQ-39) score. These scales assessed improvements in patients' movement abnormalities, other physical symptoms, well-being and ability to perform everyday tasks. PET scans were conducted to measure the effects of NTCELL on dopamine brain metabolism. The results at week 26 following implantation were compared with those at baseline.

In the study, NTCELL was well tolerated. There were no adverse events or serious adverse events related to NTCELL in any of the four patients. Eight adverse events occurred, all of which were considered to be related to the implant procedure and none to NTCELL. There was no clinical or laboratory evidence of PERV transmission in patients or their partners. MRIs showed no evidence of inflammation.

All four patients experienced sustained improvement in clinical features as seen in UPDRS, UDysRs and PDQ-39 scores at week 26 post-implant. The first patient treated continued to show improvement in neurological scores at 74 weeks post-implantation.

In this study, PET scan results did not show any consistent changes in the uptake of fluorodopa and tetrabenazine in the four patients, suggesting that the mechanism of NTCELL is not likely due to a direct change in dopaminergic neurons.

#### Poster presentation details

The abstract is available at <u>www.mdsabstracts.com</u>. The Guided Poster Tour presentation includes additional data not currently available in the abstract.

Title: Safety and clinical effects of NTCELL<sup>®</sup> [immunoprotected (alginate-encapsulated) porcine choroid plexus cells for xenotransplantation] in patients with Parkinson's disease (PD): 26 weeks follow-up (abstract #550114, poster #321)

Presenter: Dr. Barry Snow

Session Name: Parkinson's disease: Clinical trials

Session Date and Time: Wednesday 17 June 12-1:30 p.m. Pacific Daylight Time

Session Location: Seaport F, Manchester Grand Hyatt San Diego

#### **Investor Conference Call**

Living Cell Technologies will host a conference call on Thursday 18 June 2015, at 8:30 a.m. NZT / 10:30 a.m. AEST. Telephone numbers for the conference call, Conference ID 6398 0894, are: Australia 1800 123 296, New Zealand 0800 452 782 and +61 2 8038 5221 (international).

#### **About NTCELL**

NTCELL, a unique cell therapy, is an alginate coated capsule containing clusters of neonatal porcine choroid plexus cells that are sourced from a unique herd of designated pathogen-free pigs bred from stock originally discovered in the remote sub-Antarctic Auckland Islands. Choroid plexus cells are naturally occurring "support" cells for the brain and secrete CSF, which contains a range of factors that support nerve cell functions and protective enzymes that are crucial for nerve growth and healthy functioning. In NTCELL, the porcine choroid plexus cells are coated with LCT's propriety technology IMMUPEL<sup>™</sup> to protect them from attack by the immune system. Therefore, no immunosuppressive regimen is required for treatment.

Following implantation into a damaged site within the brain, NTCELL functions as a neurochemical factory producing CSF and secreting multiple nerve growth factors that promote new central nervous system (CNS) growth and repair disease-induced nerve degeneration while potentially removing waste products such as amyloids and proteins.

NTCELL has the potential to treat neurodegenerative diseases because choroid plexus cells help produce CSF as well as a range of neurotrophins (nerve growth factors) that have been shown to protect against neuron (nerve) cell death in animal models of disease. NTCELL has been shown in preclinical studies to regenerate damaged tissue and restore function in animal models of Parkinson's disease, stroke, Huntington's disease, hearing loss and other non-neurological conditions, such as wound healing. In addition to Parkinson's disease, NTCELL has the potential to be used in a number of other CNS indications, including Huntington's, Alzheimer's and motor neurone diseases including amyotrophic lateral sclerosis (ALS).

#### About Parkinson's disease

Parkinson's disease is a progressive neurological condition characterised by a loss of brain cells that produce dopamine (a neurotransmitter that conveys messages between brain cells to ensure effective movement and planning of movement) and many other types of neurons. People with Parkinson's disease experience reduced and slow movement (hypokinesia and bradykinesia), rigidity and tremors.

Parkinson's disease is the second most common neurodegenerative disorder after Alzheimer's disease, affecting approximately 4 million people worldwide. The average age of onset is 60 years, and the incidence increases with age. Men are one and a half times more likely to have Parkinson's disease than women.

Current treatments for Parkinson's disease are symptomatic and do not reverse or slow the degeneration of neurons in the brain. Most existing pharmaceutical treatment options focus on restoring the balance of dopamine and other neurotransmitters. The effectiveness of dopamine replacement therapy declines as the disease progresses. When dopamine treatments are no longer useful, some patients are treated with Deep Brain Stimulation (DBS), in which a medical device is surgically implanted in the brain in order to send electrical impulses to regions of the brain involved in the control of movement. While DBS leads to short-term symptomatic improvement, it does not impact disease progression and is not curative or neuroprotective.

#### **About Living Cell Technologies**

Living Cell Technologies (LCT) is an Australasian biotechnology company improving the wellbeing of people with serious diseases worldwide by discovering, developing and commercialising regenerative treatments that restore function using naturally occurring cells. LCT's unique proprietary encapsulation delivery technology, IMMUPEL<sup>™</sup>, coats cells with protective capsules that prevent them from attack by the immune system. This allows cell therapies to be used without the need for immunosuppressive drugs. LCT's lead product, NTCELL<sup>®</sup>, is being developed for neurodegenerative diseases. LCT recently completed a Phase I/IIa clinical trial of NTCELL in New Zealand for the

treatment of Parkinson's disease and plans to initiate a Phase IIb study in the fourth quarter of 2015. LCT holds a 50 percent interest in Diatranz Otsuka Limited, which is developing DIABECELL<sup>®</sup>, a cell therapy in late-stage clinical trials for the treatment of type 1 diabetes. LCT is listed on the Australian (ASX: LCT) and United States (OTCQX: LVCLY) stock exchanges. The company is incorporated in Australia, with research and development and operations based in New Zealand. For more information, visit <u>www.lctglobal.com</u> or follow @lctglobal on Twitter.

#### Forward-looking statement

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This document contains certain forward-looking statements, relating to LCT's business, which can be identified by the use of forward-looking terminology such as "promising," "plans," "anticipated," "will," "project," "believe," "forecast," "expected," "estimated," "targeting," "aiming," "set to," "potential," "seeking to," "goal," "could provide," "intends," "is being developed," "could be," "on track," or similar expressions, or by express or implied discussions regarding potential filings or marketing approvals, or potential future sales of product candidates. Such forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause actual results to be materially different from any future results, performance or achievements expressed or implied by such statements. There can be no assurance that any existing or future regulatory filings will satisfy the FDA's and other health authorities' requirements regarding any one or more product candidates nor can there be any assurance that such product candidates will be approved by any health authorities for sale in any market or that they will reach any particular level of sales. In particular, management's expectations regarding the approval and commercialisation of the product candidates could be affected by, among other things, unexpected clinical trial results, including additional analysis of existing clinical data, and new clinical data; unexpected regulatory actions or delays, or government regulation generally; our ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; government, industry, and general public pricing pressures; and additional factors that involve significant risks and uncertainties about our products, product candidates, financial results and business prospects. Should one or more of these risks or uncertainties materialise, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected. LCT is providing this information and does not assume any obligation to update any forward-looking statements contained in this document as a result of new information, future events or developments or otherwise.

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