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living insights

Published on an occasional basis, *Living Insights* is a source of up-to-date information for followers of the Australasian biotechnology company Living Cell Technologies (LCT)



Message from the CEO

Welcome to this issue of Living Insights.

This issue outlines the progress of our lead compound, NTCELL as well as the further projects in our pipeline. At the most recent board meeting, the Board approved pursuing the strategies outlined in this newsletter.

Ken Taylor
CEO

Half Year Report

LCT released its half year report to the ASX on 18 February.

As at 31 December 2018 net assets were \$5.8m compared to \$5.8m at 31 December 2017 and \$7.1m as at 30 June 2018. Cash and cash equivalents at 31 December 2018 were \$5.4m (30 June 2018 \$6.9m). This balance is projected to allow the planned level of operations to continue for approximately 16 months. Operating cash outflow is being managed by the directors to the extent of funding available.

[Click here for the full report on our website](#)

NTCELL

Phase IIb study

In May 2019, the 24-month efficacy data of the Phase IIb trial of NTCELL® in Parkinson's disease will be available. The 18-month data, released in November 2018, showed a significant benefit for two of the three dose groups, as did the 12-month data. The 6-month data did not show a significant benefit. At all times NTCELL has been shown to be safe for recipients.

Early in January, we met with our contacts at Medsafe, New Zealand's medicine regulatory authority to present this data and to get guidance on what options would be available to LCT as the next step towards commercialising NTCELL for Parkinson's disease, under the current regulations. The team at Medsafe has undertaken to respond in writing after it consults with overseas regulatory authorities and its legal advisors. We anticipate receiving this response before May.

Re-testing of our pig herd will soon be underway to test for its continued suitability as a source of tissue for manufacturing NTCELL.

I/IIa study

In January, LCT received the data from the 4 year follow up of the three remaining patients in the Phase I/IIa study of NTCELL for Parkinson's disease.

Initially four patients with advanced Parkinson's disease who were not responding to symptomatic treatment and were listed for DBS treatment, received implants of 40 NTCELL capsules unilaterally in the putamen.

Patient 1, four years after implantation, continues to show a clinically significant improvement in Parkinson's disease symptoms as measured by UPDRS and the motor subscale. Patient 3, after two years of follow-up, requested DBS treatment and withdrew from further evaluation.

Patients 2 and 4, four years after implantation, show no change from their study entry score.

This data should be regarded as individual case studies.

NTCELL Medical Advisory Board

At each data milestone we review the findings with our NTCELL medical advisory board to determine the next appropriate steps. The medical advisory board members are:



Professor Roger Barker (Chairman)

Professor of Clinical Neuroscience and Honorary Consultant in Neurology at the University of Cambridge and at Addenbrooke's Hospital.

Professor Barker's research looks at neurodegenerative disorders of the nervous system in particular Parkinson's disease and Huntington's disease. He combines basic research looking at novel therapies (including cell transplants and gene therapies) to treat these conditions with clinically-based work on defining the natural history and heterogeneity of both Huntington's disease and Parkinson's disease.



Dr Patrik Brundin

Professor and Director of the Center for Neurodegenerative Science, VARI Associate Director of Research and Jay Van Andel Endowed Chair in Parkinson's Research.

Dr Brundin's current research focuses on pathogenic mechanisms of Parkinson's disease as well as the development of new therapies that slow or stop disease progression or that repair damaged brain circuits. He was among the first to articulate the "prion-like" hypothesis of Parkinson's, which describes how abnormal proteins related to the disease spread from cell to cell in the brain, causing the cellular damage that perpetuates Parkinson's. He is heavily involved in the Parkinson's community and works closely with advocates to find ways to further research and to improve the quality of life for people with Parkinson's.



Professor Thomas Foltynie

Professor of Neurology in the Department of Clinical and Movement Neurosciences, UCL Institute of Neurology and Consultant Neurologist at the National Hospital for Neurology and Neurosurgery, Queen Square, London.

Alongside his general neurology practice, Professor Foltynie has a specific interest in patients with movement disorders.

He is now involved in a number of trials of new treatments for the disease and is responsible for patients undergoing Deep Brain Stimulation for Parkinson's and other conditions.



Professor Carolyn Sue

Director of Neurogenetics at Royal North Shore Hospital and the Director of the Kolling Institute, University of Sydney.

Professor Sue is an international expert in mitochondrial disease and movement disorders. Her research focuses on understanding the role of mitochondrial function in neurodegeneration, especially the disease processes involved in mitochondrial disorders, Parkinson's disease and other movement disorders. She established the Centre of Excellence for Parkinson's disease and Movement Disorders at Royal North Shore Hospital in 2011.



Dr Barry Snow

Director of Adult Medicine and Consultant Neurologist at Auckland District Health Board.

In addition to his general neurology work, Dr Snow runs a Movement Disorder Clinic and research programme. He is the Principal Investigator in the clinical studies of NTCELL in Parkinson's disease.



Pipeline

Alongside our continuing NTCELL studies we are exploring other product opportunities. These all emanate from the University of Auckland where we are working with some of their best researchers.

Pericyte Protective Agent (PPA)

Pericytes are brain cells important for maintaining the integrity of the barrier that protects the brain from toxins and promotes elimination of metabolites from the brain to the blood supply. The blood brain barrier is weakened by aging, and accelerated by neuro-degenerative diseases such as Alzheimer's and Parkinson's, allowing neurotoxins to accumulate in the brain.

Our pilot study confirmed potent activity of NTCELL and its constituents on release to protect human brain pericytes in vitro from damage induced by a toxin. Initial studies to identify the active component were promising but fractionation attempts have not yet been able to identify a single active PPA. The pericyte protective activity of NTCELL may contribute to its efficacy seen in our two clinical studies to date. At the next meeting in May, the Board will decide the next steps for this project.

Long-acting Calcitonin Gene-related Peptide (CGRP)

Exploratory studies of a lead compound synthesised by Dame Margaret Brimble's using her patented powerful peptide technology showed promising activity in the laboratory and animal models of migraine.

As a result of the successful pilot study, LCT has signed milestone-driven



development and licensing agreements to undertake pre-clinical development and scale-up manufacturing studies with a target date of November 2019 to review pre-clinical data. If the results are promising, we will initiate a Phase I clinical study in 2020.

CGRP antibodies and CGRP receptor antagonists have shown positive clinical activity promising a new generation of therapeutics to treat migraine. Current product candidates in development show promising efficacy but are limited by short duration of action requiring multiple daily dosing or producing side effects. If the Phase I study shows the compound could be injected once daily, it would be an attractive out-licence compound. We have already had interest from a number of global pharmaceutical companies keen to discuss the potential of this project.

Long-acting Pramlintide

Weight loss is well demonstrated in patients who take Pramlintide to treat diabetes. The anti-obesity action is achieved by blocking amylin receptors in the brain which relates to control of food intake.

Our pilot study confirmed that the University of Auckland lead compound was as potent as Pramlintide in an animal obesity model.

We have agreed to undertake pre-clinical and scale up manufacturing studies to enable a Phase I clinical study in 2020. Patient compliance is a recognised problem in the continuity of treatment of morbidly obese patients. If this clinical study demonstrates an extended plasma half-life enabling single daily dosing it would be an attractive out-licence as above. Patient compliance will be greater with a single daily dose schedule particularly if administered with new electronic pen injectors that can monitor compliance. Our contacts in the global pharma industry have also indicated interest in this project.

Anti Cell Proliferative

Although we have a potent anti-glioma compound on offer from the Centre for Brain Research, our due diligence on the development challenges facing compounds that can block human glioma cell proliferation in vitro conclude that this is a difficult target. Consequently, we have decided not to invest further in this target.

Conferences/Events

We will present the most up-to-date data on all our current projects at the Phar-East conference in Singapore in March.

We also have meetings scheduled with a number of Asian companies and investors at this meeting.

BIO in Philadelphia in June is another prime opportunity to discuss out-licensing opportunities for the compounds we are developing.

PHAR-EAST ASIA'S PHARMA & BIOTECH FESTIVAL

18-20 March
Singapore



3-6 June
Philadelphia

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LCT is incorporated in Australia with its operations based in New Zealand.

Australia

Phone: +61 3 8689 9997
Email: info@lctglobal.com

New Zealand

Phone: +64 9 276 2690
Email: info@lctglobal.com

Media & investor enquiries

Rachael Joel
Botica Butler Raudon
Phone: +64 9 303 3862
Email: rachaelj@botica.co.nz

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