living insights

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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)

In this edition

Message from the CEO

Welcome to this issue of Living Insights.

Since the last issue, we are pleased to have completed all implants in our phase IIb clinical study of NTCELL® in Parkinson's disease. The strength of the study outcome depends on maintaining the double-blind status.
The final group of patients in the Phase IIb trial received NTCELL implantation at the end of April, so we, like all shareholders and prospective patients, await the results in November this year.

In recent months, we've also received an update on the progress of the first four patients in our initial Phase I/IIa trial of NTCELL for Parkinson's disease and announced the beginning of a research collaboration with the University of Auckland Centre for Brain Research.

Ken Taylor
CEO

Latest international research

At the International Congress of Movement Disorders and Parkinson's Disease held in Vancouver in June, there was a conspicuous lack of any emerging treatments with data that caused any excitement about their potential as a disease modifying treatment.

There was renewed interest in cell transplant programs, from stem cell to human fetal cell transplant.

Barry Snow, our Principal Investigator, was encouraged by the NTCELL trial progress being ahead of other trials in that we have completed recruitment and treatment and now await the trial result. Other implantation programs are still dealing with recruitment, production, QA and cost issues.

LCT received credit for maintaining the double-blind trial design which is necessary to evaluate any placebo effect. Moreover our trial endpoint, UPDRS motor function subscale, is confirmed as the correct efficacy measurement. In the June 2017 issue of the prestigious journal, *Movement Disorders*, Thibault et al review "New Perspectives on study design for evaluating neuroprotection in Parkinson's disease." They also recommend UPDRS part III motor function sub scale as the best primary endpoint for early phase clinical studies of Parkinson's disease.

Research collaboration with University of Auckland Centre for Brain Research

In May we initiated a research collaboration with the Centre for Brain Research (CBR) at the University of Auckland. The research collaboration aims to explore how LCT's products can reverse human brain neurodegenerative processes associated with pericytes (and other brain cells), which help sustain the blood-brain barrier and other homeostatic and haemostatic functions in the brain.

The research project has two primary goals:

1. To extend the pipeline for LCT's lead product NTCELL by examining the effects of NTCELL on cell cultures derived from human brains with Alzheimer's disease and Huntington's disease. It will also examine the effects of NTCELL on cell cultures from neurologically normal human brain tissue.

2. To identify other encapsulated cell therapies which may have potential to treat these neurodegenerative disorders, by examining whether the cell therapies can promote neuroprotective effects in the brain.

Ultimately we hope this research will contribute to LCT's product pipeline by demonstrating that NTCELL has the potential to treat neurodegenerative disorders other than Parkinson's disease. It may also show whether other encapsulated cell therapies developed by LCT have the potential to promote cytoprotective effects, which may indicate that the cells are generating a cocktail of molecules that promote capillary function in the brain (which is very likely to be beneficial to brain function).

The research is being conducted by Auckland UniServices Limited (UniServices), the commercial research company of the University of Auckland, using the breakthrough drug testing and drug target validation platform, Neurovalida, developed by Professor Mike Dragunow, Distinguished Professor Sir Richard Faull and Associate Professor Maurice Curtis from the CBR.

Increasingly researchers are finding animal models of limited use in discovering new treatments for these difficult and ever more prevalent neuro-degenerative diseases. Using human tissue cell cultures such as pericytes, and other human brain cells from the CBR's Human Brain Bank eliminates that problem.

The treatment-directed project brings together LCT's expertise in the clinical development of cell-based therapies and the CBR's expertise in identifying targets from its knowledge and availability of human brain tissue.

Director of the CBR, Distinguished Professor Sir Richard Faull, is enthusiastic about the project which enables LCT to benefit from the CBR's world-class capabilities.

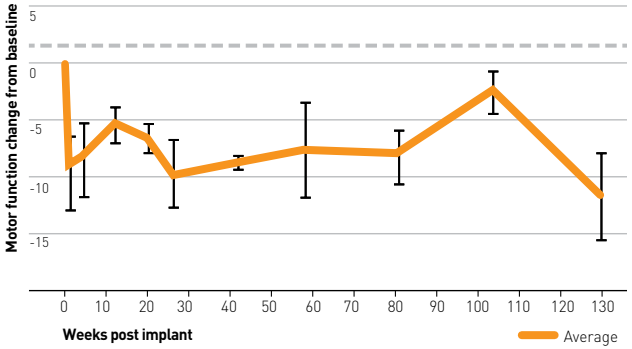
NTCELL continues to halt progression of Parkinson's disease

In June we reported that, 130 weeks after treatment, all four patients who took part the Phase I/IIa clinical study of NTCELL for Parkinson's disease remain well, there are no safety concerns, and they continue to improve.

All patients continue to show improvement over baseline, as measured by the Unified Parkinson's Disease Rating Scale (UPDRS). Efficacy is most evident in the measurement of motor function (UPDRS part III subscale – Figure 1).

Principal Investigator, Dr Barry Snow, Auckland City Hospital, says the sustained improvement is interesting and encouraging.

Figure 1: Unified Parkinson's Disease Rating Scale (UPDRS) Motor function with patients off medication



During follow up patients have the right to request implantation on the other side of the brain, continue as is, or elect to have deep brain stimulation (DBS). One patient has elected to be treated with DBS. At this time the other three patients have not requested other treatments.

CNZM for Chairman Roy Austin

LCT Chairman Roy Austin was appointed a Companion of the New Zealand Order of Merit for services to children's health and the community in the Queen's Birthday Honours in June.

Mr Roy Austin has been a Board member of Cure Kids since 1994 and Chairman since 1996. Under his leadership Cure Kids has become one of the largest funders of child health research in New Zealand, funding four professorial chairs in children's health at Auckland and Otago Universities.

Cure Kids has also seen a fourfold increase over 10 years of annual research funding grants to $2.5 million, established an $8 million government seed investment fund for child healthcare businesses, and held iconic national fundraising campaigns such as 'Red Nose Day', 'Ticket to Hope', 'The Great Adventure Race', and '$10 Queenstown Challenge'.

Through Cure Kids Mr Austin supported the joint venture with the Ministry of Foreign Affairs and Trade to assist in funding a Rheumatic Heart Disease Project in Fiji.

He played an active role in the formation of the Rotary Diabetes Prevention Trust. Mr Austin has been involved with a wide range of other community activities, including fundraising for the ASB Stadium, the Anglican Investment Trust Board, and school Boards of Trustees.

LCT in *New Scientist*

In May, Dr Ken Taylor spoke to a journalist at *New Scientist* about LCT's NTCELL trials and the potential for it to be the first disease-modifying treatment for Parkinson's disease.

▶ *Read the New Scientist article online here*

The article garnered a great deal of attention worldwideand spawned stories in numerous publications worldwide including:

- ▶ *Futurism*
- ▶ *The New York Post*
- ▶ *IFL Science*
- ▶ *Big Think*

Looking ahead

We are looking forward to November when we will be unblinded to the results of the Phase IIb study currently underway, which is designed to measure efficacy. This study will confirm the most effective dose of NTCELL, define any placebo component of the response and further identify the initial target Parkinson's disease patient sub-group.

We are maintaining a database of people who have expressed interest in NTCELL for Parkinson's disease so that we may provide them with relevant information if we are successful in obtaining provisional consent to treat paying patients.

Our goal, subject to continued satisfactory data, remains to obtain provisional consent and launch NTCELL as the first disease modifying treatment for Parkinson's disease in 2018.

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

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