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

In this edition

# LCT living insights

▶ Clinical update

## Message from the CEO



*I'm delighted to introduce the first edition of **Living Insights** – our new quarterly newsletter through which we will provide regular updates on our pipeline therapies and our business.*

*In our hot topics section, we review key issues in the therapy areas of type 1 diabetes and Parkinson's disease, as well as recent news from the sector. Of particular note in this edition, we highlight the March 2013 report from the Juvenile Diabetes Cure Alliance (JDCA), entitled 'What are characteristics of Practical Cure Research Projects?'. This is a brilliant piece that strips away the hype and looks at what a type 1 diabetes 'cure' would mean from a patient's point of view. It's a philosophy LCT has always held and followed in our research and development, and DIABECCELL is featured top of the table of projects in human clinical trials that have the potential to deliver a practical cure for type 1 diabetes.*

*I hope you find this and the other articles in the first edition of **Living Insights** interesting and informative and I welcome your feedback and questions.*

**Dr Andrea Grant**

MD and CEO, Living Cell Technologies

Email: [info@lctglobal.com](mailto:info@lctglobal.com)

## Clinical update

### DIABECCELL® for type 1 diabetes

- The clinical development for DIABECCELL as a treatment for people with type 1 diabetes is on track and, provided the registration study is successful, the target is still to launch a commercial product by 2016.
- A 30 patient registration study with a combined endpoint of reduction in unaware hypoglycaemia with no increase in HbA1c is predicted to be sufficient to secure registration in New Zealand by 2016.
- 20 patients for this registration data package will be provided from the study currently ongoing in Argentina (announced as a Phase IIb on 22 November 2012). All 20 patients have been recruited, and at 30 June, 16 implants have been performed.

- The remaining 10 patients will be recruited in New Zealand once the application for the registration study is accepted by the New Zealand regulator Medsafe, which is expected to occur before year end.
- Once the Medsafe application is approved and the New Zealand patients are recruited, then the 30 patient registration study is established.

### NTCELL® for Parkinson's disease

- The first patient is being consented into the Phase I clinical trial.
- Following consent, the patient will be monitored to establish baseline data, and the implant is expected to occur during Q3.
- The first patient will be followed for two months at which time the data safety monitoring board will determine if the subsequent three patients can be implanted.

*In a May Open Briefing interview, Dr Andrea Grant provides more detailed updates on the status of DIABECCELL and NTCELL clinical trials.*

▶ [Read a full transcript here...](#)

▶ [Listen to audio of part of the briefing here...](#)

## Return of the Jedi



*In June 2012, we undertook a small restructure of our R&D teams. One of the main drivers was to enable an orderly succession plan for our founder Professor Bob Elliott, who has made the leading of our company's R&D his life's work. Finding a successor to Bob was always going to be an impossible task.*

*LCT needed someone who had experience in the clinical development of an encapsulated, xeno-cell product through regulatory approval, as well as the development of new, exciting products from our cell therapy platform.*

*As well as that, our ideal person would also be qualified as a medical doctor, and have knowledge of both the diabetes and neurological disease areas. I can count the number of people in the world with this experience on one hand – one, Paul Tan.*

*So, you can imagine my pleasure when he accepted my invitation to return to LCT in the role of Head of R&D. With him and the rest of the LCT executive beside me, including Bob as our Director of Clinical Research and Innovation, I believe the future is very bright for our ongoing success.*

**Dr Andrea Grant**

## Business update

### Communications drive

In order to ensure that our exciting clinical and business news is reaching our audiences in a timely fashion, we are currently upscaling our public relations and communications.

As the first and critical step we have updated our website with the latest facts, figures and information from our joint ventures, our markets and our clinical trials. Take a look at: [www.lctglobal.com](http://www.lctglobal.com).



The homepage features a new video in which Dr Andrea Grant gives a brief overview of our business and our therapeutic portfolio.

We also have a newly developed fact sheet downloadable from the website homepage, which sets out in jargon-free language in a reader-friendly format, the key information on LCT for investors and the media.

**Please email: [info@lctglobal.com](mailto:info@lctglobal.com) if you have any feedback on the updated website or the new fact sheet and stay tuned for more updates.**

## Financial update

- As of 31 March 2013, LCT has \$4.91m in cash reserves to support approximately \$1.2m per annum of R&D and other activities that are not otherwise funded by the DOL or OPF agreements.
- Under the agreement signed with Otsuka Pharmaceutical Factory (OPF) for the co-development of NTCELL for Parkinson's disease, LCT expects to receive the second milestone payment of A\$2 million during late Q3/early Q4 of this year. This payment will occur two months after implant of the first patient, provided that the data safety monitoring board decides it is safe to proceed with the implants for the remaining three patients.
- The Diatranz Otsuka Limited (DOL) 50:50 partnership with OPF continues to provide the funding for the clinical trials of DIABECCELL.

## LCT in the media

*Interest.co.nz* – 14 May

**Business correspondent Andrew Patterson interviewed Dr Andrea Grant in May regarding LCT's successful history and ambitious plans to grow into a significant biopharmaceutical-player.**

### KEY HIGHLIGHTS INCLUDE:

#### Biotech innovation

*"One of the unique aspects of our business is that we have developed a system that allows us to put pig cells into a protective capsule. So when we put them into your stomach or into your brain they're not attacked by the immune system.... On that basis we really are right up there with the other innovative cell therapy companies in the world."*

#### Life-changing possibilities

*"The first group of people with type 1 diabetes that will benefit from our product DIABECCELL are patients who are typically called unstable... One of the features of this is they experience what are called unaware hypos... where suddenly their blood glucose drops, but... they have no awareness, and then they suddenly just lose consciousness... or become delirious. Of course if you're on your own and there's no one to help you... it can be very dangerous. And so people who are living with this stage of diabetes tend to be very limited in the job they can do and their quality of life is severely impacted."*

#### Special joint venture partnership with OPF

*"Because it's a 50/50 joint venture once the product goes to market and is creating revenue and greater value, then LCT receives 50% of the future profits."*

The joint venture arrangement also... means there's shared control over the development of the product... all of the intellectual property, the technology platform, and the know-how can be used and retained within LCT to apply it to other disease areas.

► [Read a summary and watch a video of the interview here...](#)

**Andrew Patterson talks Andrea Grant at Living Cell Technologies, a company expecting to grow to be a significant bio-pharmaceutical player**

Posted in Business May 14, 2013 - 08:04am, Andrew Patterson

**Double Shot Interview**

Dr Andrea Grant, MD Living Cell Technologies

By Andrew Patterson

Success doesn't come easily in the biotech sector.

With significant start-up costs, on-going research complications, the need for extensive clinical trials and the inevitable delays in getting products to market this is not a business for the faint hearted.

While New Zealand's track record in the sector has been mixed, one stand out survivor has been Auckland based biotech company Living Cell Technologies.

### In other news...

#### Idealog – 17 April

Inside: Living Cell Technologies  
► [Read the Idealog article here...](#)

#### Insulin Nation – 3 June

Four-Legged Islet Factories:  
The Pigs of Auckland Island  
► [Read the Insulin Nation article here...](#)

## Type 1 diabetes







### JDCA report on Practical Cure Research Projects

In March, the Juvenile Diabetes Cure Alliance (JDCA) published a report that aimed to define the characteristics of practical cure research projects for type 1 diabetes and identify work currently being undertaken that has the potential to deliver a practical cure.

According to the report, practical cure research is differentiated from other cure research by its defined outcomes and the potential to cure individuals now living with type 1 diabetes by 2025. Specifically, Practical Cure research endeavours to deliver a set of outcomes shown in the diagram above.







Out of 332 projects in human clinical trials, only 6 were identified by JDCA to target a Practical Cure for type 1 diabetes. DIABECELL is listed at the top:

### PRACTICAL CURE DEFINITION

	<b>MINIMAL MONITORING</b> ✓ Does not require blood glucose monitoring beyond once a week ✓ Keeps A1C levels between 5 and 7.		<b>SLEEP WORRY FREE</b> ✓ Allows patients to sleep care free
	<b>FREE DIET</b> ✓ Does not restrict a patient's diet ✓ Does not require carb counting		<b>MINIMAL SIDE EFFECTS</b> ✓ Best case: Zero side effects ✓ Acceptable: Insignificant / non-invasive side effects
	<b>REASONABLE MEDS</b> ✓ If pharmacological, an easily managed regime		<b>FAST RECOVERY (IF SURGICAL)</b> ✓ If surgical, less than 72 hours recovery

***"We believe that islet transplantation used in conjunction with cell protection... could potentially result in a Practical Cure."***

*JDCA REPORT, MARCH 2013*

Project Name	Description	Location
DIABECELL	Transplanted porcine islets that are micro-encapsulated	
Monolayer Cellular Device	A beta cell encapsulation approach that uses human islets	
ATG/GCSF	Drug combination aimed at stopping both the autoimmune attack and stimulating growth of beta cells	
Sitagliptin/Lansoprazole	Drug combination aimed at stopping both the autoimmune attack and stimulating growth of beta cells	
BCG	Drug that kills disease-causing autoimmune cells and restores pancreatic beta-cell function through regeneration	
Stem Cell Educator	An individual's blood is treated with stem cells which has the effect of reversing autoimmunity and stimulating beta cell growth	

Source: JDCA Research

The report show that that out of all cure research funded by non-profits, only 2% goes towards Practical Cure research projects. The author concludes that shifting more cure research funding to Practical Cure projects, such as DIABECELL, would materially increase the likelihood of curing individuals who are living with type 1 diabetes. Donors can have a more positive impact on type 1 diabetes research by stipulating that their contribution fund only Practical Cure research projects.

► [Read the full JDCA report here](#)

## Parkinson's disease

### Burden, current treatment options and the potential of NTCELL

- Parkinson's disease affects approximately 4 million people worldwide, including an estimated 64,000 Australians and 10,000 New Zealanders.
- It is a progressive neurological condition which is related to a deficit of dopamine as a result of degeneration of dopamine-producing brain cells.
- Most pharmaceutical treatment options focus on restoring the balance of dopamine and other neurotransmitters. For many patients, drugs become ineffective as the severity of the symptoms increases over time
- More recently, high-frequency deep brain stimulation (DBS) of regions of the brain involved in the control of movement has become a widely practised and accepted form of management for complex Parkinson's disease. DBS does not impact on disease progression, is not curative or neuroprotective and does not improve major non-motor symptoms such as cognition, poor balance or autonomic dysfunction.
- Unlike current therapy options for Parkinson's disease, NTCELL is neuroprotective and offers people living with the disease the hope of being able to halt disease progression and restore quality of life.
- The exciting results of preclinical studies on NTCELL were presented at the International Congress of Parkinson's Disease and Movement Disorders in Sydney in June, with NTCELL showing a significant benefit in animal models of Parkinson's disease:
  - ~ **Recovery from movement abnormalities**
  - ~ **Improvements in neurological defects**
  - ~ **Demonstrative increase in neural connections and number of dopamine-producing cells in the affected area of the brain**
  - ~ **Well tolerated with no evidence of inflammation or other adverse reaction**
  - ~ **Improvements were seen within two weeks and lasted for at least six months, the trial endpoint**
- NTCELL is currently in Phase I clinical trials in humans.

## Biotechnology &amp; Industry

### Highlights from the 19th Annual International Society for Cellular Therapy (ISCT) Meeting, in Auckland, April 22-25, 2013



For the first time this year, New Zealand hosted an ISCT annual meeting following the highly successful Sydney meeting in 2007. This reflects ISCT's interest in fostering the significant developments in cell therapy in the Australasian sector.

The scientific programme was diverse, covering the translational aspects of and issues involved with all types of cell and tissue-based research, as well as the scientific, clinical, laboratory, and regulatory issues related to each type of cell-based therapy.

LCT featured prominently on the agenda within a plenary session on 'Unique Approaches in Cell Therapy Commercialisation' and Paul Tan's keynote presentation on 'Taking the pig to market: LCT's regulatory and commercial journey so far.'

► Visit the congress website here for further information...



### LCT shortlisted for the 2013 New Zealand Hi-Tech Award for Emerging Company of the Year

The NZ Hi-Tech Awards celebrate the success of the New Zealand technology sector and its contribution to the New Zealand economy.

Dr Andrea Grant was delighted to be a 2013 finalist for the prestigious Emerging Company of the Year award "I'm incredibly proud of the efforts of our team over the past year. We've made tremendous advances in our business by forming an innovative joint venture with an international pharmaceutical company as well as the progress we've made in the clinical development of our lead products DIABECELL for type 1 diabetes and NTCELL for Parkinson's disease."

An equally pleased Roy Austin, Chairman LCT, said, "To be shortlisted in a year of record entry numbers against such tough competition is a great honour and testament to the quality of our people and their commitment to translating our unique science and intellectual property into breakthrough treatments that are of true value to patients."

By entering the awards, Living Cell Technologies' success gains the attention of some of the country's most successful companies and business leaders.

► Read more about the New Zealand Hi-Tech Awards here...



### Twitter trends ► Follow us @LCTglobal



**LCT Global** @LCTGlobal 2 May  
A wrenching story, but sadly all too real for many of our followers - The family living with type 1 diabetes [bbc.in/10hb6Nt](http://bbc.in/10hb6Nt)  
Expand



**JDCA-Diabetes** @JDCA2025 2 Jun  
Only 2% of the 2012 nonprofit research grants have the potential to deliver a #type1 #diabetes #PracticalCure [thejdca.org/wp-content/upl...](http://thejdca.org/wp-content/upl...)  
Expand



**JDRF Advocacy** @JDRFAdvocacy 19 Apr  
Insulin is not a cure for #type1 diabetes! #T1D [pic.twitter.com/S1SJDScAa](http://pic.twitter.com/S1SJDScAa)  
View photo



**Amer. Diabetes Assn.** @AmDiabetesAssn 17 Apr  
Our new book w/ @JDRF identifies what is known—and unknown—about diagnosing/treating #type1 #diabetes: [bit.ly/Z0N7Xi](http://bit.ly/Z0N7Xi)  
Expand

### Next quarter:

- First NTCELL implant in the Phase I clinical trial in New Zealand to take place
- Final results from the Phase IIa DIABECELL DIA-09 clinical trial in Argentina are expected to be released
- NZTE America's Cup Technology and Investment Showcase - read more...  
LCT has been invited to showcase to some of Silicon Valley's most active investors at this event, which will take place on Emirates Team New Zealand base during the America's Cup



**Winner of the 2013 NZBIO Janssen Industry Excellence Award for Company of the Year**



**Finalist for the 2013 NZ Hi-Tech Award for Emerging Company of the Year**

LCT is incorporated in Australia. Research and development, operations and manufacturing facilities are based in New Zealand.

#### Australia operations

Phone: +61 2 9239 0277

Email: [info@lctglobal.com](mailto:info@lctglobal.com)

#### New Zealand operations

Phone: +64 9 276 2690

Email: [info@lctglobal.com](mailto:info@lctglobal.com)

#### Media and investor enquiries

Rachael Joel, Botica Butler Raudon

Phone: +64 9 303 3862

Email: [rachaelj@botica.co.nz](mailto:rachaelj@botica.co.nz)

ASX: LCT OTCQX: LVCLY

For more information visit

[www.lctglobal.com](http://www.lctglobal.com)

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