



## NEWSLETTER AUGUST 2011

### Living Cell Technologies breaking new ground in cell therapies

With the commencement of our Phase 2 patient trials in Argentina adding a third jurisdiction to the company's international late-stage clinical trial program, LCT is now the clear and recognised pioneer of cell therapies globally. As the only company in clinical trials using xenotransplantation technology, LCT investors stand to benefit from the company's unique position in the final stages of commercialisation.

The body of evidence to support the safety and efficacy of LCT's treatment for type 1 diabetes is growing with the completion of implants in the New Zealand trial adding to the results from the Russian trial. The Argentina trial will further validate the exciting premise that [DIABECCELL](#)® will improve the quality of life of people with unstable type 1 diabetes through the normalisation of blood sugar levels, a significant reduction in sometimes fatal episodes of unaware low blood glucose, as well as potentially allowing significant reduction of insulin dependency.

Safety and preliminary efficacy data generated to date show promise that DIABECCELL will change the paradigm for treating diabetes and potentially lead the way for other indications when it becomes the world's first approved porcine cell implant.

*"We were pioneers in this space and we are now also in the enviable position as the only company in the world to be in clinical trials. Many other companies and collaborations have either failed in early stage trials or they are still in early development. We are very proud to be the most advanced*

*in this space and for helping the many patients who have been involved in our clinical trials.” Prof. Bob Elliott, founder and acting CEO.*

LCT is gaining international scientific attention for its leadership role in this area, including recognition from organisations such as the International Xenotransplantation Association. However, Elliott says the company needs to now remind the investment community of the strength of LCT’s technology. “It really is incredible when you think about how far we’ve come on much lower levels of funding, around \$75m so far, when compared to drug therapies,” says Elliott.

In fact, recent statistics released by the Australian Medicines Industry association (<http://www.ausmedindustry.com.au/>) show the typical drug requires upwards of \$900 million dollars and 15-20 years to reach a similar stage to LCT’s current treatment development.

“We have advanced this treatment with significantly less funding and shown great results in a much shorter time period. We are very proud of what we have achieved and hope it will bring significant benefit to our loyal investors,” concludes Elliott.

#### ***How does DIABECCELL work?***

Tiny DIABECCELL microspheres containing live islet cells are implanted into a patient's abdomen using a simple laparoscopic procedure. Once implanted, DIABECCELL works by self-regulating and efficiently secreting insulin and glucagon in response to the patient's changing glucose levels. DIABECCELL islets are protected from the body's immune response by LCT's breakthrough proprietary encapsulation technology, so DIABECCELL patients do not require any immunosuppression.

## **Enhancing the Board of Directors and Executive Management**

There have been some changes at executive and Board level at LCT over the last few months, we as a Board consider that we now have the directors and senior management team who are well equipped to steer the business through the final stages of clinical trials and the market launch of DIABECCELL. Prof. Bob Elliott has taken on the role of interim CEO while the company plans an international search for a replacement with excellent team building skills and deep commercialisation experience.



## Roy Austin

Investment banker and Director Roy Austin was elected Chairman of the Board. Mr Austin advised LCT on company financing during its formative years and is very familiar with the science and the company's financial background.

Mr Austin also has first-hand knowledge in biotechnology; he is the chair of Cure Kids, a large and highly successful New Zealand charity, seeking better treatments and preventive measures for sick children.

He directly influenced the formation of Cure Kids Ventures, the commercial arm of the charity, aimed at bringing academic research into viable commercial use.

"I am privileged to chair such a well balanced and experienced board and I have been impressed with the capability and depth of the management team," says Mr Austin. "This is an exciting time for LCT. We are perfecting our treatments in Phase 2b clinical trials to ensure they have optimal benefits for patients when we bring them to market. As a Board our major focus going forward will be commercialisation of this technology and the related funding. DIABECCELL is already showing good results in patients but we want to ensure we are bringing the absolute best product to market when we plan to begin commercialisation in 2013."



## Dr Bernie Tuch

The company also welcomed internationally lauded cell transplant leader Dr Bernie Tuch. Dr Tuch is currently a senior scientist with CSIRO on a cell transplantation project. He possesses first class xenotransplantation knowledge and experience.

Dr Tuch directed the Diabetes Transplant Unit at Sydney's Prince of Wales Hospital from 1991 to 2009 and worked steadily on finding a cell-based treatment for diabetes using a number of methods.

Here are Dr Tuch's view on current therapies for type 1 diabetes and the future of cell transplantation in improving treatment options:

### ***What are the limitations of treatments for people with type 1 diabetes?***

Whilst insulin administration beneath the skin keeps people with type 1 diabetes alive, the dynamics of its release do not simulate the fine degree of control achieved in non-diabetic people by the cells in their pancreas which secrete insulin every minute as required to keep blood sugar levels normal.

Replacing the pancreatic insulin-producing cells which have been destroyed with similar cells from other sources, such as from the pig, does allow tight glucose control to be achieved. In this way, it is anticipated that insulin administration will eventually no longer be needed by people with type 1 diabetes. Their quality of life will improve, and long term diabetic complications such as damage to the eye and kidney will be prevented.

### ***You have dedicated your life to working on cell transplant technologies for the treatment of the disease, can you share your observations?***

Cell transplant technology as a therapy for diabetes began in the early 1970's but it was not until 1989 that the first patient was able to cease insulin administration after having received insulin-producing cells from a deceased human. That person together with most others who receive such cells required anti-rejection therapy to prevent the immune system from destroying the graft. Side effects of using such drugs include an increased risk of infection, and possibly of cancer. Another limiting factor with the use of these cells is their very limited supply. It is wonderful to see that strategies are being trialled to overcome these limitations. These include the platform technology of encapsulation to isolate the grafted cells from the recipients' immune system, thereby preventing the need for anti-rejection therapy. They also include the use of a much

larger source of donor insulin-producing cells than can be obtained from donor humans, namely those from pigs. These can be obtained when required, and do not require a person to die for the cells to be available.

I have been working on cell therapies for type 1 diabetes for 30 years now. The international community is closer to a successful cell therapy than it ever has been to overcome the need for insulin administration for the large numbers of people with insulin-dependent diabetes.

***Where does DIABECCELL fit globally and what do you believe the opportunities for LCT are?***

The first insulin-producing pig cells were transplanted into diabetic humans in the early 1990's in Sweden. Whilst it has taken some time to develop the technology of large scale delivery of these pig cells to humans without the need for anti-rejection therapy, LCT has achieved this goal with DIABECCELL. Clinical trials with these cells show great promise in achieving the end point of having recipients cease insulin administration. LCT has shown much initiative in pioneering the use of pig cells as a human therapy, and has shown dogged persistence in doing so. It is because of this strategy that LCT is now very well placed to be the first company in the world to deliver a product to the market place that will overcome the need for insulin to be injected in the large numbers of people with type 1 diabetes.

## Global DIABECCELL clinical program

We are preparing to implant our first patients with DIABECCELL in the Argentina trial which will mark a further important milestone in our global, multi-jurisdictional clinical trial. As the only company in the world to be in the clinic with a xenotransplantation technology we are breaking new ground. In particular, our success in securing regulatory approval in multiple jurisdictions is providing the best practice pathway for others working in this field.

Our Argentina Phase 2b DIABECCELL clinical trial will be held in Buenos Aires, Argentina. Up to eight adult patients with type 1 diabetes, including those with unstable diabetes and severe hypoglycaemia will each receive two implants of DIABECCELL, three months apart. The dose for the second group of 4 may be varied.

These patients will be in addition to the 14 patients who received DIABECCELL in the New Zealand trial and have shown promising results and the favourable results from the two year follow-up of patients involved in the Phase 1/2a clinical trial in Russia. "We've been really impressed with the results so far and we continue to track the patients as part of a two year follow-up which shows continued safety and benefit," says Prof. Bob Elliott, LCT founder and medical director. "The aim of this additional trial is to generate data showing the benefits of this treatment with multiple implants in patients with unstable diabetes and other complications.

The cell encapsulation process has also been improved and the islets are produced by a scaled up procedure. We will monitor the effect on severe unaware hypoglycaemia and, in the long-term, other diabetic complications of the eye and kidneys.”

The trial is being overseen by Dr Adrian Abalovich and Prof. Boris Draznin, Professor of Medicine, Endocrinology, and Diabetes at the University of Colorado Health Sciences Centre, both respected physicians in diabetes research. The first results are expected to be released early next year. This will be important information as the company moves toward beginning a Phase 3, also called a pivotal trial, early in 2013.

Impending legislative change in Russia to introduce more comprehensive laws governing xenotransplantation has for the moment delayed premarketing activities in Russia. The Ministry of Healthcare in Russia has invited LCT to assist in the formulation of these legislative changes.

## Partnering for success

Over the last 6 months we've announced a number of exciting partnerships that open up new markets and opportunities for DIABECCELL to further enhance our clinical program with the expansion of our clinical trials.

In April Otsuka Pharmaceutical Factory, Inc. (“Otsuka”) became an investor and strategic partner. We are working with Otsuka towards establishing a collaborative agreement for the research, development and commercialisation of DIABECCELL.

We also continue to work closely with strategic LCT investor Jiangsu Aosaikang Pharmaceutical Co to establish a collaborative research and license agreement for DIABECCELL for the China market.

Finally, we acknowledge the support of our activities by the US based, Juvenile Diabetes Research Foundation International and Children with DIABETES Foundation and in New Zealand, Cure Kids and Foundation for Science Research and Technology.

## LCT in the News

Many of you may have heard about a feature length documentary created about LCT which followed a

number of patients involved in the New Zealand clinical trial. The documentary aired on New Zealand's TV1 on Sunday, 26 June.

One of the patients featured in the documentary, Karen Skinner, was also featured in the most recent edition of New Zealand's leading diabetes magazine called 'dia-log.' Mrs Skinner talks about living with diabetes and the decision to enter into a clinical trial, something she had been excited about for many years when she heard about Prof. Elliott's early work with islet cells. She writes, "Now I move forward. My blood sugar levels are much more stable. Hypos are few and far between and I can now anticipate when my blood sugar levels are dropping. My injected insulin requirements have been reduced by 35%."

## Our share price performance

There is no doubt that our current share price is a reflection of prevailing market unease and volatility and we have a significant challenge to communicate more effectively to the investment community the significant value in our DIABECCELL technology and the commercial pathway to market launch. The world's researchers recognise our leadership position and we must now work hard to translate that awareness to new investors. To our loyal, long term investors we recognise your frustration, and thank you for standing by us.

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