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Marc Sinatra's Bioguide

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LIVING CELL– FINDING THE PATH TO MARKET

Overview: Shortages of human organs for transplant has lead to interest in animal to human xenotransplantation.

To date, the few studies that have looked at xenotransplantation have failed to deliver results which held much promise. The chief difficulty has been rejection of the transplanted organs due to attack by the host's immune system. Even if rejection could be overcome a raft of other issues would remain, such as whether the animal organ would function suitably in a human.

Living Cell Technologies (LCT) has taken a different approach and chosen to concentrate on transplanting cells rather than whole organs. Although this does limit the potential treatments, it also simplifies the system because it means you are only reliant on the animal tissue to replace the single function that is missing in the human. Importantly, it has also allowed LCT to engineer a coating that protects the animal tissue from rejection.

Having developed the most promising xenotransplantation system yet, what other hurdles will LCT have to leap to get its products on the market?

Financials: Market cap: $60m; cash: $13.6m; last quarter cash burn: $1.4m.

Directors: Non-executive chairman, Simon O'Loughlin; CEO, Dr Paul Tan; medical director, Prof Robert Elliott; non-executive directors, David Collinson, Dr Robert Caspari, Dr David Brookes and Laurie Hunter.
LCT's board has a mix of skills covering the required areas of knowledge. Some directors' expertise is not in key areas, leaving scope to reduce the size of the board.

Products in Development: LCT's products consist of two components. Neonatal porcine cells derived from a highly controlled, herd of pigs owned by LCT and a coating derived from seaweed. This alginate coating protects the cells from the host's immune system, while allowing all of the vital elements to pass through the coating.
1) Diabecell: Utilises porcine coated islet of Langerhans cells which are injected into the peritoneum to treat type 1 diabetes. The cells are expected to detect rises in body sugar and release insulin accordingly. To date, Diabecell has been shown to be safe and reduce insulin requirements in mouse and primate models of type1 diabetes. In humans, a study of 18 people revealed no adverse events nine years after treatment. Interim results of an ongoing Russian phase I/IIa trial of Diabecell has demonstrated significantly reduced insulin requirements for two patients six months after transplantation and a 10 percent reduction in the requirements of a third patient shortly after transplantation. A fourth patient has not complied with the protocol. Two more patients will be recruited. A phase I/IIa study will be commenced in New Zealand when final regulatory approval is received.

2) Neurotrophincell: Putative treatment for neurological disorders, such as Huntington's disease, stroke and sensorineural hearing loss, based on porcine choroid plexus cells. The choroid plexus produces cerebrospinal fluid which contains factors important for the health of neurons in the brain. Neurotrophincell has been shown to reduce cell death by a factor of five in a primate model of Huntington's disease. It is also the subject of collaboration with The Bionic Ear Institute for sensorineural hearing loss.
3) Fac8cell: Comprises porcine hepatocytes and its first target indication is haemophilia. Hepatocytes produce clotting factor VIII which is absent or faulty in people with haemophilia. Fac8cells have been shown to be healthy and functional after implantation in the abdomen of mice.

Significant Product Markets: The worldwide drug treatment market for diabetes was $US21 billion in 2006. In 2005, there were 14.6 million people in the US with diagnosed diabetes. Five to ten percent of these people have type 1 diabetes. There are many products in development for type1 diabetes, but they are primarily aimed at preserving the remaining insulin producing cells, rather than increasing the amount of insulin produced. US company Microislet is developing a product very similar to Diabecell and may provide some competition down the track, but LCT appears to be well in front.
There is no current treatment for Huntington's disease, except those to control symptoms and estimates of the potential market for a treatment are hard to come by. Based on what is available, a market of $US500-900 million is reasonable. Many groups are looking for a treatment, but the vast majority are in the very early stages. A couple of projects are in late stage clinical trials, but these are likely to provide incremental advances at best.

Verdict: LCT is the leader in the field of xenotransplantation and the promising, but very limited data they have collected is supported by other independent studies.
The biggest hurdle LCT faces may not be whether their products work, but getting to do the studies to show they work. The big concern with xenotransplantation is that it may provide the opportunity for an animal disease to jump to humans. With most treatments, very rare serious adverse events only affect the patient. Where an infectious disease could jump to humans, one serious adverse event could put the entire population at risk. Consequently, adequately demonstrating safety to a level that will satisfy regulators may be very difficult and take a very long time.

To get around some of these barriers, LCT has chosen to start its clinical trial program in Russia where the regulatory framework is more liberal. Importantly, LCT is developing its business to US Food and Drug Administration standards. For example, LCT has gained International Accreditation New Zealand support for its world's first xenotransplantation testing facilities, GMP certification of its manufacturing process and is working with the highly regarded Barbara Davis Center for Childhood Diabetes to conduct US clinical trials.

Where clinical trial results are the key risk points for most life science companies, approvals to conduct clinical trials in key markets will be additional risk points for LCT and the value of LCT will rise significantly if and when these approvals are granted.

I value LCT at 29 cents per share.

Living Cell was up half a cent or 2.04 percent to 25 cents.

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