CEO on NTCELL Deal

Open Briefing interview with MD & CEO Andrea Grant

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In this Open Briefing®, Andrea discusses:

- Deal structure provides upfront cash, 50 pct of downstream profits
- Mechanism of activity and potential of NTCELL implants
- Potential developments in NTCELL and other products in next 12 to 18 months

Record of interview:

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Living Cell Technologies Limited (ASX: LCT) today announced an agreement with Otsuka Pharmaceutical Factory Inc. to co-develop NTCELL for Parkinson's disease and other neurological disorders. Under the agreement, LCT will receive a A$3 million upfront payment and A$2 million after the first patient is safely implanted with NTCELL. If the upcoming Phase I trial of NTCELL in Parkinson's disease is successful, Otsuka will invest A$20 million into Diatranz Otsuka Limited (DOL), the 50:50 joint venture between the two companies, to fund development of NTCELL through to market approval. Why have you structured the deal in this way, rather than in the more normal life sciences industry format of out-licensing, with royalties payable on product sales?

MD & CEO Andrea Grant
We think this is a great deal structure for three reasons. It provides us cash up front as well as cash for the development of NTCELL through to market, it gives us a greater income on commercialisation than a royalty ever would and finally provides a true partnership where we share control of the product development.

With regard to the cash up front, we get A$3 million on signing this agreement, and will then receive another A$2 million after the first patient in the Phase I Parkinson's trial has safely received the first NTCELL implant. The first implant is scheduled for the March 2013 quarter and so we expect we'll receive the A$2 million milestone payment before the end of the current financial year. In addition, Otsuka will pay the Phase I development costs for NTCELL, which we estimate at about A$2 million over 18 months.

The second reason we like this deal structure is that it gives us a greater share of the downstream income than a royalty structure would. The reality is that at this stage of development, prior to Phase I, a biotech would be doing really well to get a 1 percent royalty. As long as our profit as a proportion of revenue is greater than 2 percent, we'll do better than a 1 percent royalty – and obviously we believe NTCELL's profit will far exceed that proportion of revenue!

The third reason is that we'll be part of a true partnership in which we remain in shared control of the future development of NTCELL. This is to not be underestimated. In a typical biotech licensing deal, a pharmaceutical company takes the license and assumes responsibility for the remaining development of the product. The biotech essentially loses control over the product and the big pharma throws away all the built-up knowledge, expertise and passion of the product's originator. The product becomes one of hundreds in

the pharmaceutical pipeline and if the R&D priorities of the big pharma change, all the biotech can do is hope its product isn't culled.

We know already from our 12 month involvement in DOL that we have a true partnership with Otsuka, which places value on our expertise and our knowledge of our product. With Otsuka, we have shared governance over the product, and that allows us to remain in charge of our own destiny. Most biotechs aren't given the chance to structure a deal this way.

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How will the profile of your potential cash flows from NTCELL compare with those under the more usual licensing structure?

MD & CEO Andrea Grant
Our near term cash flow outlook is improved dramatically. We've taken the cost of the NTCELL Phase I trial out of our expenses: that's around A$2 million over the next 18 months. And, if the Phase I trial is successful, we'll also take the remaining NTCELL development cost through to market approval out of our cash needs entirely. The upfront payments of up to A$5 million will allow us to invest in some of the newer products in our pipeline, and get them ready for pre-clinical or clinical development and to look for partnership or investment opportunities.

Longer term, our cash flows will reflect the much higher potential income share under our deal with Otsuka than we'd get under a royalty agreement made at this early stage of the product's development.

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NTCELL is a choroid plexus cell product and pre-clinical studies have shown it to increase dopamine-producing neurons in the brain. How does NTCELL's activity compare with treatments currently available for Parkinson's disease and what is the potential market for Parkinson's treatments?

MD & CEO Andrea Grant
The small molecule market for Parkinson's treatment is currently estimated at around US$2 billion globally. Those treatments are essentially dopamine replacement therapies and are mainly used in early to mid stage Parkinson's but not in the later stages of the disease. That's because they become ineffective as the neurons that should be manufacturing dopamine die away and the region of the brain where they would normally be becomes extensively damaged. Essentially the existing small molecule drugs only treat the symptoms of the disease in the early stages; they don't halt the progression of the disease or reverse the cell degeneration.

That's where NTCELL is very powerful. It has the potential to reverse the degeneration that you see in Parkinson's and to halt the progression of the disease. It's a cell that manufactures and secretes hundreds of different growth factors, antioxidants and anti-inflammatory proteins that are crucial for nerve health and cell regeneration. These growth factors appear to cause the formation of new nerves and the regeneration of the damaged region of the brain. Certainly in the animal studies we've done, the degree of regeneration was quite dramatic, so dramatic that Otsuka just had to jump on board even at this early stage.

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The Phase I trial will be conducted in New Zealand. To what extent will the trial results have validity in other jurisdictions?

MD & CEO Andrea Grant
They will be 100 percent valid. New Zealand is preferred by many pharmaceutical companies as a location for Phase I trials because its ethics and regulatory approval system

is very efficient and follows international standards. The New Zealand medical regulatory system follows guidelines set by the EU, US Food and Drug Administration (FDA) and International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH).

Also, the New Zealand Medicines and Medical Devices Safety Authority (Medsafe) is a member of the Pharmaceutical Inspection Co-operation Scheme (PICS) which is a cross-recognition scheme for GMP manufacturing of pharmaceuticals. This means our GMP license is already mutually recognised in the EU and it likely will be in the US when the FDA joins PICS.

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What level of confidence do you have that the A$20 million Otsuka will invest in DOL for the development of NTCELL will be sufficient to progress the drug to market approval?

MD & CEO Andrea Grant
We've got a high level of confidence that we've developed our cost estimates accurately, and that this funding will be sufficient.

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As at 30 September, LCT had cash of A$2.9 million. How will you employ the funds received from Otsuka?

MD & CEO Andrea Grant
As I mentioned, we have a number of internal programs that are in R&D and we want to move those through pre-clinical trials so they're ready for clinical development. Also, there are a number of external partnerships and pre-clinical assets we're now in a position to look at in order to further broaden and diversify our pipeline.

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LCT will retain the rights to develop NTCELL for non-neurological applications. What are the potential applications of NTCELL outside the neurological setting and what is the status of your development of NTCELL for other diseases?

MD & CEO Andrea Grant
Because NTCELL is a technology that causes sick and damaged tissues to regenerate and return to normal function, any non-neurological condition that has a degenerative cellular component is available to us. For example, there's already a significant amount of published data that shows NTCELL is highly active in wound healing and there's a substantial amount of pre-clinical efficacy data demonstrating that.

We're also talking to Otsuka about whether it would want to extend our work on NTCELL in the neurological space into other conditions such as Huntington's, where we already have a substantial amount of pre-clinical data, or Alzheimer's' disease and spinal cord injury.

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In November 2011, Otsuka invested A$25 million in DOL to fund the development of LCT's DIABECELL treatment for type 1 diabetes. Otsuka's further investment of A$20 million to fund the development NTCELL implies a value of A$90 million for the DOL entity, and A$45 million for LCT's 50 percent stake. This compares with LCT's market capitalisation, prior to the announcement of the NTCELL deal, of A$18 million. What will be the key value accretion points for DOL, and LCT, over the next 12 to 18 months?

MD & CEO Andrea Grant
We're hoping the market will recognise the deal as a value inflection driver and that our market cap will reflect that additional value.

Apart from that, we have a number of key value incretion points coming up in the next 12 to 18 months. The A$2 million milestone payment for NTCELL should occur in the June quarter next year and that will be a clear signal that the Phase I trial is on the right track. After that, we're considering doing a six-month follow-up of the patients, which would happen around the March quarter of 2014. Given that in the monkey study of NTCELL we saw an effect within two weeks, we'll probably be able to glean quite a lot of information at the six month point on how well NTCELL is performing in human disease.

Most importantly, at some point between signing this deal and the end of the Phase I trial, provided NTCELL works, we'd expect Otsuka to invest the agreed A$20 million into DOL.

Also next year, we'll be looking to secure at least one partnership that speaks to a broadening and diversification of our pipeline. And by the middle of 2014 we'd hope to have a second product ready for the clinic.

With DIABECELL, which is being developed by DOL, we've completed Phase II studies and we're moving into the final registration trial phase. Completion of transplants in those trials by the end of the year will be key to delivering our goal of commercialising DIABECELL in 2016.

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Thank you Andrea.

For more information about Living Cell Technologies, visit www.lctglobal.com or call Andrea Grant on (+64 9) 276 2690.

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