# Biotech Daily

Tuesday April 17, 2012

*Daily news on ASX-listed biotechnology companies*

*** ASX, BIOTECH DOWN: AVITA UP 10%, OPTISCAN DOWN 12%**

*** PHARMAXIS: 'ASM8 DURATION CAUSED NON-SIGNIFICANT RESULT'**

*** LIVING CELL APPOINTS DR BARRY SNOW FOR PARKINSON'S TRIAL**

*** OSPREY RAISES $20m, TO LIST ON ASX**

*** ALLIED SIGNS NATURE TECH, VGXI FOR PHASE I HSV2 TRIAL MATERIAL**

*** CONSEGNA TO ACQUIRE 100% OF LEADING EDGE WITH 252m SHARES**

*** MESOBLAST HALF-WAY IN PHASE II STEM CELL SPINAL TRIAL**

## MARKET REPORT

The Australian stock market fell 0.31 percent on Tuesday April 17, 2012 with the S&P ASX 200 down 13.5 points to 4,288.8 points.

Nine of the Biotech Daily Top 40 stocks were up, 18 fell, six traded unchanged and seven were untraded.

Avita was the best, up 2.5 cents or 10.2 percent to 27 cents with 4.0 million shares traded.

Living Cell climbed 7.1 percent; Universal Biosensors was up 6.15 percent; Antisense and Benitec were up more than five percent; Allied Health was up 3.45 percent; Impedimed, and Tissue Therapies rose more than two percent; with Bionomics and CSL up more than one percent.

Optiscan led the falls, down 1.5 cents or 11.5 percent to 11.5 cents, with 194,498 shares traded, followed by Prima down 11.1 percent to 24 cents with 21.6 million shares traded.

Sunshine Heart lost 6.7 percent; Heartware fell 5.95 percent; Neuren and Reva were down more than three percent; Biota, Pharmaxis and QRX shed more than two percent; Alchemia, Bioniche, Genetic Technologies, Nanosonics and Starpharma were down more than one percent; with Acrux, Clinuvel, Cochlear, Mesoblast, Resmed and Sirtex down by less than one percent.

## PHARMAXIS

Pharmaxis says that longer than expected duration of activity led to no significant differences between ASM8 and control in a phase IIa trial of patients with allergic asthma.

Pharmaxis chief executive officer Dr Alan Robertson told Biotech Daily that of the patients who initially received just the control "there was no reduction in their response to the allergen".

Dr Robertson said he was reluctant to include this data because of the small numbers involved, but said there was "a longer duration of ASM8 effect than we were expecting".

"Why, we don't know, but we were trying to reduce the responses to their allergies and we did that by 44 percent," Dr Robertson said.

He said the trial showed that the 3mg dose was "about right", there was no inflammation in the lungs and the there were no safety issues.

Pharmaxis said that the double-blind, randomized, controlled, three-way crossover study evaluated the efficacy and safety of 14-day inhaled ASM8 in subjects with asthma.

The company said that the primary outcome was the effect of ASM8 on allergen-induced late allergic response at three to seven hours post-allergen challenge.

Pharmaxis said that the 16 subjects each received two doses of ASM8 (3mg or 7.8mg) or control at different times, once a day in a randomized, cross-over fashion.

The company said that broncho-constriction as measured by change in forced expiratory volume over one second (FEV1) during the late phase of the allergen challenge response was reduced by 44 percent ($p < 0.05$) when dosed with 3mg and by 41 percent ($p < 0.01$) when dosed with 7.8mg ASM8, as assessed by the change in area under the curve, at three to seven hours post challenge compared to screening.

Pharmaxis said that the results were consistent with a previous trial that reduced the late allergen response by 59 percent ($p < 0.005$) following four-day dosing with 8mg ASM8.

But the company said that the control, phosphate-buffered 0.9 percent saline, also reduced the late allergen response by 44 percent ($p < 0.005$), three to seven hours post dose, meaning there was no difference between doses.

Pharmaxis said that the statistically significant effect of the response when subjects were treated with vehicle was "indicative of a drug carryover effect that can result in studies of a cross-over design".

In a media release, Dr Robertson said the company was "encouraged by these new clinical data demonstrating the potential value of this approach for treating asthma".

"We now know that ASM8 is best dosed once per day and we know that 3mg is an appropriate dose for future trials," Dr Robertson said.

"The statistically significant response when patients were treated with vehicle indicates that future studies with drugs of this nature should be parallel in design," he said.

"The moderate to severe sector of the asthma market, which is the target of ASM8, represents a significant commercial opportunity," Dr Robertson said.

Pharmaxis said that ASM8 was a combination product of two RNA-silencing oligo-nucleotides targeted at a number of receptors for mediators of inflammation in asthma.

The chair of medicine at Ontario Canada's McMaster University Prof Paul O'Byrne said the approach to reducing multiple inflammatory mediators "may be an important clinical option for patients with severe allergic asthma and this trial indicates that additional studies are warranted".

The company said that the full results including a variety of exploratory endpoints was being evaluated and would be presented at a forthcoming scientific congress.

Dr Robertson told Biotech Daily that he hoped to begin a phase IIb trial with about 200 patients later this year.

Pharmaxis fell three cents or 2.3 percent to $1.255 with 1.4 million shares traded.

## LIVING CELL TECHNOLOGIES

Living Cell says that Auckland District Health Board's Dr Barry Snow will be the principal investigator in its phase I clinical trials of NTCell for Parkinson's disease.
Living Cell said that Dr Snow led the Board's movement disorders clinic and was also the Board's medical director.
Living Cell chief executive officer Dr Andrea Grant said Dr Snow was an authority on Parkinson's disease "held in high regard by the Parkinson's community" who appreciated his commitment to finding better treatments for people affected by the disorder.
The company said the appointment was subject to regulatory and Board research review committee approvals.
"Parkinson's is a disorder which clinicians can help manage but can't reverse, so this represents an exciting new potential option for patients," said Dr Snow.
"It's also important that these clinical trials are conducted here because public awareness of the disorder is raised when New Zealand patients get involved in this type of research, which in turn helps improve the way the disorder is looked after generally," Dr Snow said.
Living Cell said it was preparing an application to New Zealand's medicines safety authority Medsafe to start phase I clinical trials, expected to begin by April 2013.
The company said the trial would be a phase I, open-label investigation of the safety and clinical effect of NTCell in people with Parkinson's disease and would last a minimum of 60 weeks and involve patients diagnosed with Parkinson's for at least four years.
Living Cell said that the treatment involved transplanting choroid plexus cells from the Auckland Island pig herd into the brain.
The company said that choroid plexus cells were naturally-occurring support cells for the brain and when transplanted could help protect the brain and repair damaged nerve tissue.
Living Cell said the cells would be encapsulated with its Immupel technology to prevent the immune system from rejecting them as foreign.
The company said that patients would receive either NTCell treatment or the current gold standard of treatment for their symptoms, deep brain stimulation.
"The results of our preclinical studies suggest that NTCell can protect brain tissue which would otherwise die, potentially delaying or even preventing the debilitating effects of Parkinson's," Dr Grant said.
"These unprecedented preclinical results show recovery in the part of the brain affected in Parkinson's disease, as well as a greater than 50 percent improvement in symptoms," Dr Grant said.
Living Cell said that pre-clinical results showed an increase in dopamine producing neurons, improvements in movement and neurological defects, together with good tolerance with no evidence of inflammation or other adverse reaction.
The company said that improvements were seen within two weeks and lasted for at least six months, the trial endpoint.
Living Cell was up half a cent or 7.1 percent to 7.5 cents.

## OSPREY MEDICAL

Osprey says it has raised $20 million through its initial public offering of 50 million CHESS depositary interests at 40 cents each and expects to list
Osprey said it would list on the ASX subject to satisfying listing conditions under the code OSP, with a market capitalization of $40 million.
The company said the funds would be used to develop the Cincor cardiac dye removal system developed at the Baker IDI Heart and Diabetes Institute (BD: Mar 8, 2012).

## ALLIED HEALTHCARE GROUP

Allied Health subsidiary Coridon has licenced Nature Technology Corp and contracted VGXI Inc to produce clinical material for a phase I herpes simplex virus 2 study.

Allied said that its 37.3 percent subsidiary founded by Prof Ian Frazer expected to begin the trial “later this year” and had created a licence agreement with the Lincoln Nebraska-based Nature Technology Corporation (NTC) to use its vector technology and Hypergro fermentation technology, which provided high yield and cost effective DNA production.

Allied chief operating officer Dr Julian Chick told Biotech Daily that the licence agreement provided that “in the event that the technology is commercialized NTC get a small royalty”.

Allied said that Coridon had contracted the Houston Texas-based VGXI Inc in the US for production of clinical material for the phase I study.

The company said that Nature Technology designed safe, minimalized and antibiotic-free selection vectors, which had superior expression of recombinant proteins in mammalian cells.

Allied said that the vectors had been designed to comply with US Food and Drug Administration and European Medicines Agency regulatory guidance and the use of the Nature Technology vector improved the overall benefit of the vaccine by driving the in-vivo transcription and translation of the genetic material.

Coridon said that VXGI specialized in the production of DNA plasmids for human clinical trials and had begun manufacturing the herpes vaccine using Nature Technology’s Hypergro technology.

Allied chief executive officer Lee Rodne said that “access to this technology will allow the Coridon vaccine to fully maximize its gene expression and therefore improve the performance of the vaccine”.

“These are important steps forward to the initial phase I study for Coridon which will provide validation of the Coridon technology,” Mr Rodne said.

Allied said that Coridon’s herpes vaccine was 100 percent effective in protecting animals against herpes simplex virus 2 infection, incorporated Nature Technology’s NTC8485 antibiotic-free expression vector and guidance from US and European regulatory bodies sought to eliminate non-essential sequences and to avoid the use of antibiotic resistance genes where feasible.

Coridon chief executive officer Neil Finlayson said the Nature Technology licence was “an important agreement for the company to be able to access this leading technology and cements the relationship we have built up with NTC dating back to late 2009”.

Allied said that Coridon’s DNA vaccine technologies were the next generation of vaccines for the prevention and treatment for a range of infectious diseases and cancers in humans.

Allied was up 0.1 cents or 3.45 percent to three cents with three million shares traded.

## CONSEGNA GROUP

Consegna says it will complete the acquisition of Leading Edge Instruments by exercising two call options for a total of 19 percent of the company.

Consegna said that Leading Edge was an unlisted public company that controled the Breatheassist and Vibrovein technologies.

The company said it would issue 252,000,000 Consegna shares to Leading Edge shareholders for the acquisition and would then hold 100 percent of the issued capital of Leading Edge.

Consegna was unchanged at 3.2 cents.

## MESOBLAST

Mesoblast says it has enroled half of the 100 patients in its phase II trial of allogeneic, mesenchymal precursor cells for degenerated intervertebral discs and low back pain.
Mesoblast said that the “rapid rate of enrolment” since it began in August last year, attested to the major unmet medical need and to the relative simplicity of its non-surgical procedure (BD: Aug 22, 2011).
The company said it expected to complete enrolment by October 2012.
Mesoblast said that the double-blind, placebo-controlled phase II trial was being conducted at 15 sites across the US and would randomize the 100 patients with intervertebral disc disease to receive a non-surgical, percutaneous injection into the intervertebral disc of either low or high dose mesenchymal precursor cells with hyaluronic acid carrier, hyaluronic acid carrier alone or saline alone.
The company said the trial aimed to extend its preclinical results and show that a single injection of mesenchymal precursor cells could reduce low back pain and improve function over six months, improve disc anatomy and eliminate the need for a surgical procedure.
Mesoblast said that up to 15 percent of people in industrialized countries had chronic low back pain lasting more than six months and for those with progressive, severe and debilitating pain due to degenerating intervertebral discs, the only option was major back surgery involving spinal fusion, artificial disc replacement, or other surgical procedures.
The company said its non-surgical adult stem cell treatment used an intervertebral disc injection of allogeneic or off-the-shelf cells that took less than 15 minutes.
Mesoblast said the phase II trial design, endpoints and dose ranges were based on its preclinical study using allogeneic sheep mesenchymal precursor cells for non-surgical restoration of damaged intervertebral discs.
The company previously said the sheep trials showed that a single injection of cells into damaged inter-vertebral discs resulted in significant reversal of the degenerative process, regrowth of disc cartilage and normalization of disc pathology, anatomy and function for at least six months (BD: Sep 10, 2009; Aug 22, 2011).
Mesoblast said the study, co-authored by chief executive Prof Silviu Itescu, was entitled ‘Immunoselected STRO-3+mesenchymal precursor cells and restoration of the extracellular matrix of degenerate intervertebral discs’ and was published in the March 2012 issue of the Journal of Neurosurgery.
The article is available at http://thejns.org/doi/full/10.3171/2012.1.SPINE11852.
Mesoblast said the lowest dose of mesenchymal precursor cells caused the damaged discs to become statistically equivalent to the non-degenerated normal control discs at six months when evaluated by magnetic resonance imaging and histo-pathological analyses.
The company said that the degenerated discs treated with nothing or the carrier control remained statistically worse in each parameter tested than the non-degenerated normal control discs.
Mesoblast fell five cents or 0.7 percent to $7.25.