



# Biotech Daily

Thursday February 20, 2014

*Daily news on ASX-listed biotechnology companies*

- \* **ASX FLAT, BIOTECH DOWN: BENITEC UP 27%, PRANA DOWN 16%**
- \* **LIVING CELL RESPONDS TO ABC-TV NEWS ON NTCELL TRIAL HALT**
- \* **ONCOSIL TO DEVELOP INJECTABLE ONCOCAL FOR SOLID TUMORS**
- \* **MESOBLAST MPC EURO PATENT FOR CARDIAC, VASCULAR INDICATIONS**
- \* **FEDERAL \$800k GRANT FOR SIENNA BLADDER CANCER TEST**
- \* **NHMRC \$402k FOR WOOLCOCK, AFT SINUS TREATMENT**
- \* **SIMAVITA LISTS ON ASX FOR INCONTINENCE DIAGNOSTIC**
- \* **PHARMAXIS H1 REVENUE DOWN, SALES UP, LOSS EVEN**
- \* **RHINOMED RAISES \$2.5m**
- \* **ACRUX H1 REVENUE UP 751% TO \$44m, PROFIT UP 1,257% TO \$25m, AMP 9%**
- \* **GENETIC TECHNOL H1 REVENUE DOWN 55% TO \$2m, LOSS UP 36% TO \$5m**
- \* **FURTHER APPROVAL FOR PHARMAUST PPL-1 DOG CANCER TRIAL**
- \* **PRIMA APPOINTS PROF BRADLEY MONK ADVISER**

## MARKET REPORT

The Australian stock market climbed 0.08 percent on Thursday February 20, 2014 with the S&P ASX 200 up 4.1 points to 5,412.3 points. Fourteen of the Biotech Daily Top 40 stocks were up, 16 fell, eight traded unchanged and two were untraded.

Benitec was the best, up 27 cents or 27.3 percent to \$1.26 with 1.3 million shares traded. Avita climbed 13.0 percent to 13 cents; Optiscan was up 9.1 percent; Uscom was up 5.6 percent; Impedimed and Genetic Technologies were up more than four percent; Acrux, Anteo, Clinuvel, Cochlear and Viralytics were up more than three percent; Ellex and Bionomics rose more than two percent; Neuren and Universal Biosensors were up one percent or more; with Resmed up 0.6 percent.

Prana led the falls, retreating 17.5 cents or 15.8 percent to 93.5 cents, with 2.3 million shares traded. Pharmaxis fell 14.8 percent to 11.5 cents; Oncosil lost 7.4 percent; Reva, was down 6.7 percent; Living Cell fell 5.1 percent; Alchemia, Cellmid and Nanosonics were down more than three percent; Patrys and Tissue Therapies shed more than two percent; with Mesoblast, Osprey, QRX and Sirtex down more than one percent.

## LIVING CELL TECHNOLOGIES

Living Cell has responded to an ABC-TV News item that claimed to “reveal” information published to the ASX by the company and reported in Biotech Daily last year.

The ABC-TV News at 7pm last night carried an item by reporter Sophie Scott in which she claimed she was revealing the fact that Living Cell had halted its trial of NTCell for Parkinson’s disease following the withdrawal from publication of a preclinical study.

The company announced the trial halt on December 19 and Biotech Daily published a full page account of the halt and its reasons on that day (BD: Dec 19, 2013).

Living Cell said at that time that the study, entitled ‘Restoration of motor control and dopaminergic activity in rats with unilateral 6-hydroxy-dopamine lesions’ was conducted between 2007 and 2009 and published in the Journal of Regenerative Medicine in 2011.

An abstract is available at: <http://www.ncbi.nlm.nih.gov/pubmed/21548737>.

Living Cell said that the publication was withdrawn “following an internal quality assurance audit which showed that the source data for the study held on file ... are incomplete and therefore the efficacy conclusions in the publication cannot be confirmed”.

The company said that the withdrawal of the rat efficacy data did not in itself present a safety risk regarding the use of NTCell in humans, but as a precautionary measure it had placed a hold on any further patient recruitment into the human phase I/IIa clinical study underway at Auckland City Hospital.

The study was co-authored by Living Cell founder and medical director Prof Bob Elliott and senior executive Dr Paul Tan.

Dr Tan has left the company and Dr Ken Taylor has been appointed head of the NTCell program (BD: Jan 21, Feb 6, 2014).

Today, Living Cell the ABC-TV News item contained “inaccurate statements ... [which] could create a false market” but did not specify the statements.

The company said that while the rat model study had been withdrawn and the data would be removed from all regulatory dossiers “none of the pre-clinical studies supporting the potential safety of NTCell in humans have been withdrawn [and] the phase I/IIa clinical trial of NTCell for Parkinson’s disease has not been stopped”.

Living Cell said that the one patient in the trial was continuing with all assessments and the clinical team reported that the patient was in good health.

The company said that the New Zealand regulatory authority Medsafe authorized the trial following a substantive and proper review of the clinical trial application, which contained evidence gathered from multiple pre-clinical studies of the potential safety and efficacy of NTCell and “only one of these pieces of evidence is affected by the withdrawal of the publication”.

The company said that the most compelling evidence of the potential of NTCell as a treatment for Parkinson’s disease came from the study of the effects of NTCell in a monkey model of Parkinson’s disease, published last year (BD: Sep 16, 2013).

“This publication has not been withdrawn and the data remains in the regulatory submission,” Living Cell said.

The company said that recruitment to the trial had been paused voluntarily to allow the data safety monitoring board time to reach its own determination on whether the withdrawal of the single rat efficacy study from the pre-clinical evidence base created a significant safety risk to any subsequent patients who may be recruited.

Living Cell managing director Dr Andrea Grant said the company took its responsibility to patients, clinicians and regulators “very seriously ...[and] as a precaution we have simply paused recruitment

Living Cell closed down 0.4 cents or 5.1 percent to 7.4 cents.

## ONCOSIL MEDICAL

Oncosil says it has begun a development program for Oncocal, a next generation delivery platform for its localised radiation technology for the treatment of solid tumors.

Oncosil said that Oncocal was an injectable source of radioactive phosphorus-32 intended to deliver safer local radiation therapy for solid tumors and would complement the Oncosil product candidate for pancreatic cancer.

Oncosil said that chief scientific officer Dr Peter Knox developed Oncocal as part of research into new ways to deliver radiation therapy to solid tumors, with Oncocal injected directly into the tumor, where it emitted beta radiation locally for about three months.

The company said that a new patent had been filed.

Oncosil said that when Oncocal was injected it formed an insoluble and immobile salt in combination with interstitial fluid, which remained where it was placed.

Oncosil said that Oncocal was expected to use similar reactor time to Oncosil, but was likely to generate more phosphorus-32, which would in-turn deliver a cost saving to the company and the volume of material to be used in a treatment was expected to be much smaller, with processing expected to be simpler.

Oncosil said that the starting material was low cost and available at pharmaceutical grade and once out of the reactor, the material was diluted with phosphate and dispensed, which could be carried out by a number of different sub-contractors.

The company said that Oncocal's viscosity was low, so medical practitioners could use smaller gauge needles and it could be distributed through the tumor more effectively.

Oncosil fell one cent or 7.4 percent to 12.5 cents.

## MESOBLAST

Mesoblast says the European Patent Office has granted a patent covering its adult mesenchymal precursor cells for the treatment of cardiac and vascular conditions.

Mesoblast said the patent, entitled 'Perivascular Mesenchymal Precursor Cell Induced Blood Vessel Formation', provided exclusive rights in Europe until March 29, 2024, with potential for extension based on duration of clinical development.

The company said that the patent covered the use of Mesoblast's allogeneic or 'off-the-shelf' mesenchymal precursor cells for cardiac and vascular conditions, including acute myocardial infarction, congestive heart failure, angina, peripheral arterial disease, and cerebro-vascular stroke.

Mesoblast said it was developing products to treat cardiac and vascular conditions using both highly purified and immunoselected mesenchymal precursor cells as well as the culture-expanded mesenchymal stem cells to which they give rise.

The company said the cell types might be effective in cardiovascular disorders by release of factors which could act on target tissues to induce blood vessel formation and enhance vascular flow, prevent heart muscle death, reduce fibrous scar tissue and modulate key inflammatory cells, including monocytes and T-cells.

Mesoblast said the patent "significantly extends and broadens" its intellectual property position in Europe to cover its optimized mesenchymal precursor cells products for the treatment of cardiac and vascular diseases, beyond the previously granted patents which covered allogeneic mesenchymal stem cell products for treating damaged heart muscle to improve heart function.

The company said its US phase III trial for congestive heart failure was underway, with phase II trials in Europe and the US for acute myocardial infarction using catheter-delivered mesenchymal precursor cells and intravenous mesenchymal stem cells.

Mesoblast fell seven cents or 1.2 percent to \$5.79 with 517,399 shares traded.

### SIENNA CANCER DIAGNOSTICS

Sienna says it has been awarded an \$800,000 Commercialisation Australia grant for a cost-effective urine test for bladder cancer.

The Melbourne-based Sienna said that the test could potentially reduce the need for invasive and expensive hospital tests.

The company said that the grant would enable it to advance its clinical evaluation program at the Baltimore, Maryland-based Johns Hopkins Hospital, progress its initial licencing agreements in the US and recruit senior staff to drive its global product launch.

Sienna said since 2008 it had undertaken research and development and clinical studies to develop a proprietary biological marker, that is a chemical reagent or stain, that could be used by pathology labs to test for bladder cancer using a urine sample.

The company said that current bladder cancer testing involved a cystoscopic examination of the bladder with entry through the urethra that could cost thousands of dollars and urine cytology that could lead to inconclusive results in up to 25 percent of samples.

Sienna said it wanted to provide a definitive, simple and cost-effective urine-based diagnostic for less than \$US150 a test.

Sienna managing director Dr Kerry Hegarty said the company hoped to be “the world leader in the development of a range of telomerase-based diagnostic tests for cancer”.

“In the US, bladder cancer is the fifth most common cancer, yet among the most expensive to detect and treat,” Dr Hegarty said.

“Our first goal is to deliver the world’s most cost-effective bladder cancer test using a simple urine sample and then apply our expertise more broadly in telomerase and other biomarkers to help detect other cancers such as lung, breast, prostate and thyroid,” Dr Hegarty said.

Sienna said that subject to licencing agreements, the test could be available in 2015.

Sienna is a public unlisted company.

### WOOLCOCK INSTITUTE OF MEDICAL RESEARCH

Sydney’s Woolcock Institute of Medical Research says its researchers have won a \$401,708 National Health and Medical Research Council grant for a sinus treatment.

The Woolcock Institute said that its staff would work with the Lane Cove, Sydney-based AFT Pharmaceuticals to develop a novel device that specifically targets the sinuses to treat chronic sinus infection.

Woolcock researcher Prof Greg King said that the goal was to develop a targeted treatment that carried a formulation “capable of simultaneously removing mucus, dispersing biofilms and killing bacteria hiding deep in the sinuses”.

The Institute said that 16 percent of Australians suffered the severe discomfort and pain of recurring sinus infections, triggered when mucus made in the sinuses failed to drain properly.

The Institute said that the infection caused throbbing facial pain and patients had difficulty breathing through their noses.

Woolcock head of respiratory technology Prof Paul Young said that about 1.9 million Australians were diagnosed with chronic sinus infection in 2010–’11.

“And yet despite that, there is no current marketed nasal aerosol medicine for the treatment of CSI, or any efficient device capable of specific targeting,” Prof Young said.

The Institute said that sinusitis sufferers were prescribed antibiotics, mucus dissolvents or steroids administered with a nasal pump or modified nebuliser, which were approaches that gave virtually no access to the sinuses, and thus limited therapeutic efficacy.

## SIMAVITA

Simavita has listed on the ASX with the code SVA to commercialize its incontinence diagnostic (BD: Dec 3, 12, 18, 2013).

The ASX said that Simavita had 58,405,261 Chess depositary interests on offer.

In December, Simavita said its 'smart incontinence management' or Sim platform technology was an instrumented incontinence assessment application providing evidence-based incontinence management care plans to the residential aged care market.

Simavita opened at 50 cents and closed up 12 cents or 24 percent at 63 cents.

## PHARMAXIS

Pharmaxis says total revenue for the six months to December 31, 2013 fell 9.7 percent to \$5,370 with sales up 67.5 percent and net loss after tax even at \$20,698,000.

Pharmaxis said that revenue from the sale of Aridol and Bronchitol for the six months to December 31, 2013 was up 67.5 percent to \$2,382,000 compared to the previous corresponding period, but interest income and Federal Government Research and Development Tax Incentives were down 33.9 percent to \$2,988,000 for the period.

Pharmaxis chief executive officer Gary Phillips told a teleconference that sales in the three months to December 31, 2013 were "disappointing"

"The lack of growth in Germany is hurting us," Mr Phillips said.

Mr Phillips said that in Germany sales were not meeting expectations with patients starting on Bronchitol and then stopping, while compliance and adherence in the UK was significantly better.

Mr Phillips said he was looking forward to the "tie-breaker" US trial of Bronchitol for cystic fibrosis and that the US market was more like the UK market in terms of centres and expected compliance.

He said he expected patient recruitment for the trial to begin by July 2014.

Mr Phillips said that Bronchitol unit sales increased by three percent in the three months to December 31, 2013 compared to the three months to September 30, 2013, but revenue increased four percent on stronger margins.

He said distributors had been appointed in Russia, the Czech Republic, Slovakia and Turkey and Israel was expected to be added in the coming months.

Mr Phillips said that there was interest in the company's early stage assets from major pharmaceutical companies and described the company as a leader in amine oxidase inhibitors which could have efficacy in a wide range of indications.

"The amine oxidase platform technology and its associated intellectual property position is a source of long term value for Pharmaxis," Mr Phillips said.

Following the teleconference, Pharmaxis chief financial officer David McGarvey told Biotech Daily that the "very interested" companies had decided "that the disease area and our scientific approach is a strategic fit" and before any licencing or partnership the companies needed to undertake due diligence on the program including patent position, chemistry, tests for safety, efficacy and drug profile.

Mr McGarvey confirmed that research and development spending decreased from \$12,690,000 for the six months to December 31, 2012 to \$6,493,000 for the six months to December 31, 2013.

The company said its net tangible assets per share fell 52 percent to 12 cents and diluted loss per share was constant at 6.7 cents.

Pharmaxis said it had cash and cash equivalents of \$50,685,000 at December 31, 2013 compared to \$63,943,000 at June 30, 2013.

Pharmaxis fell two cents or 14.8 percent to 11.5 cents with 4.7 million shares traded.



### RHINOMED

Rhinomed says it has raised about \$2.5 million through a placement to professional and sophisticated investors at 4.2 cents a share.

Rhinomed chief executive officer Michael Johnson said the funds would “accelerate the drug delivery program focused on the delivery of Sumatriptan [for migraine] using the Breatheassist technology platform ... [and] support our ongoing efforts in sport and sleep”. Rhinomed said that Peloton Capital and Morgans Corporate were the placement’s joint-lead managers.

Rhinomed fell half a cent or 9.4 percent to 4.8 cents with 5.5 million shares traded.

### ACRUX

Acrux says revenue for the six months to December 31, 2013, was up 751 percent to \$43,657,000 with net profit after tax up 1,257 percent to \$24,590,000.

Acrux chief financial officer Tony Dipietro told Biotech Daily that excluding the \$US25 million (\$A28.2 million) milestone royalty income was up 225 percent from \$4.4 million for the six months to December 31, 2012 to \$14.3 million for the six months to December 31, 2013.

Despite the results, a Macquarie Bank research note described the company as an “underperform” with a price target of \$1.80,

Acrux said that a dividend of eight cents per share was paid for the period to June 30, 2013 on September 23, 2013.

The company said that net tangible assets per share was up 100.0 percent to 22 cents and diluted earnings per share was up 1,500 percent to 15 cents.

Acrux said that cash and cash equivalents at December 31, 2013 was \$54,162,000 compared to \$29,665,000 at June 30, 2013.

Acrux was up seven cents or 3.5 percent to \$2.07 with 2.2 million shares traded.

### ACRUX

AMP and related bodies have increased their shareholding in Acrux from 12,796,720 shares (7.68%) to 14,717,740 shares (8.84%).

The shares were bought and sold between July 22, 2013 and February 19, 2014 in scores of small to medium-sized trades at a range of prices.

### GENETIC TECHNOLOGIES

Genetic Technologies says revenue for the six months to December 31, 2013 was down 55 percent to \$2,435,599, with a net loss after tax up 36 percent to \$5,078,100.

Genetic Technologies said that the fall in revenue was “primarily due to a material fall in revenue generated from the granting of licences by the company to its non-coding technology”.

The company said that the increased loss was “largely attributable to an increase in selling and marketing expenses associated with ... its Brevagen test”.

Genetic Technologies said its net tangible assets per share was up 27.7 percent to 1.2 cents and diluted loss per share was up 26.25 percent from 0.8 cents to 1.01 cents.

The company said it had cash and cash equivalents of \$8,216,347 at December 31, 2013 compared to \$1,721,293 at June 30, 2013.

Genetic Technologies was up 0.3 cents or 4.7 percent to 6.7 cents with 1.1 million shares traded.

## PHARMAUST

Pharmaust says it has received a further New South Wales approval to begin its trial of PPL-1 for canine cancer.

In December Pharmaust said it had been accredited as an animal research facility to begin trials by April 2014 (BD: Dec 19, 2013).

Today, the company said it had approval from the New South Wales Department of Primary Industries director-general's animal care and ethics committee to begin the evaluation of PPL-1 in the treatment of canine cancers under principal investigator Dr Angela Frimberger of the Veterinary Oncology Consultants, to evaluate the product in a variety of canine cancers.

Pharmaust said that with Veterinary Oncology Consultants at the Homebush, Sydney-based Animal Referral Hospital would conduct a clinical trial in a small number of pet dogs to test the safety and efficacy of PPL-1 for treating naturally occurring superficial soft tissue sarcomas, chemo-resistant lymphomas and metastatic melanomas.

The company said that the dogs in the trial would be treated by their owners at their homes and to determine the safest and most effective dose, the trial would incorporate incremental increases in drug quantity to different groups of dogs, pending safety in the previous dose and all dog owners, researchers, administrators and sponsors would know what drug and how much was being administered.

The company said that tumor size would be measured before and after treatment using callipers, computed tomography scan or radiographic imaging.

Pharmaust executive chairman Dr Roger Aston said the US pet market had about 77.5 million dogs and 93.6 million cats and about 60 percent of dogs over six years of age had some form of cancer, with cancer therapies estimated at \$US550 million in 2011.

Dr Aston said PPL-1 was already approved for veterinary use by Pharmaust's partner.

"We believe that if successful in this trial, PPL-1 will be able to be approved quickly for the treatment of dog cancers following a further pivotal study," Dr Aston said.

Pharmaust was unchanged at 1.7 cents with 33.6 million shares traded.

## PRIMA BIOMED

Prima says it has appointed gynaecological oncologist Prof Bradley Monk to its clinical advisory board to advise on CVac for epithelial ovarian cancer.

Prima said that Prof Monk was a member of steering committees for the US National Cancer Institute and Gynecological Oncology Group.

The company said that Prof Monk chaired many steering committees for clinical trials in ovarian cancer including Amgen's for AMG 386 or trebananib.

Prima said that Prof Monk was a principal investigator on industry and NCI sponsored ovarian cancer trials and his current appointments include professor in the Department of Obstetrics and Gynecology at the University of Arizona College of Medicine.

Prof Monk holds a Doctor of Medicine from the University of Arizona.

Prima said Prof Monk was a member of journal editorial boards, including Gynecologic Oncology and Clinical Ovarian Cancer and a reviewer for journals including Clinical Cancer Research, Obstetrics and Gynecology, Cancer and Journal of Clinical Oncology.

Prima was unchanged at 4.6 cents with two million shares traded.