# Biotech Daily

Monday May 17, 2010

*Daily news on ASX-listed biotechnology companies*

*** ASX TUMBLES, BIOTECH DOWN: AVEXA UP 15%; PATRYS DOWN 9%**

*** PHARMAXIS: BRONCHITOL IMPROVES CF LUNG FUNCTION 8%**

*** ITALY GIVES CLINUVEL DRUG SPECIAL APPROVAL, FIRST REVENUES**

*** CORRECTION: LINKAGE GRANTS, NHMRC FUNDING**

*** CBIO, SPRINGTREE AGREE TO $12.45m CONVERTIBLE NOTE**

*** CBIO SHARE PLAN**

*** PHARMAUST OPTIONS RAISE $216k**

*** GIACONDA SALE OF MYOCONDA TO AMTI DELAYED, AGAIN**

## MARKET REPORT

The Australian stock market lost 3.1 percent on Monday May 17, 2010 with the S&P ASX 200 down 143.9 points to 4467.2 points.

Nine of the Biotech Daily Top 40 stocks were up, 17 fell, 10 traded unchanged and four were untraded.

Avexa was best, recovering 0.4 cents or 15.4 percent to three cents with 27.4 million shares traded, followed by Antisense up 12.5 percent to 1.8 cents with 2.4 million shares traded.

Compumedics and Genera climbed more than three percent; Clinuvel rose 2.2 percent; with Bionomics, Cochlear and Heartware up more than one percent.

Patrys led the falls, down one cent or 9.1 percent to 10 cents with 102,250 shares traded.

QRX lost 8.3 percent; Sirtex was down 6.5 percent; Biota, Cellmid, Mesoblast and Nanosonics fell four percent or more; Cathrx, Pharmaxis and Prana were down more than three percent; Benitec and CSL shed more than two percent; with Chemgenex, Psivida, Universal Biosensors and Viralytics were down more than one percent.

PHARMAXIS

Pharmaxis says headline results for the final stage of its phase III trial of Bronchitol in for cystic fibrosis shows lung function improved by 7.9 percent over 18 months.

Pharmaxis said lung function, measured by forced expiratory volume over one second (FEV1) of Bronchitol (inhaled mannitol) treated patients improved by 129 mL (7.9%) after 18 months relative to their lung function on entering the trial ($p<0.01$).

Pharmaxis chief executive officer Dr Alan Robertson said the sustained results were "impressive and of significant clinical importance".

"For cystic fibrosis patients, consistent loss of lung function, averaging one to two percent per year, is the leading cause of death," Dr Robertson said.

"The improvements now shown with Bronchitol treatment over an 18-month period hold out the promise that Bronchitol can modify the course of this disease," Dr Robertson said.

Pharmaxis said the Bronchitol trial was conducted in two phases with the first six month, placebo-controlled, blinded phase reported in May 2009 (BD: May 4, Jun 16, 2009) and meeting its primary endpoint by improving lung function as measured by a change in FEV1 by a clinically significant 6.5% ($p<0.001$).

Pharmaxis said that in an extension to the regulatory phase III trial, patients were invited to continue on active drug, or be switched from control to drug for up to a year after the first six-month blinded phase had concluded, and some patients who finished the trial have been on Bronchitol for 18 months.

The second 12-month unblinded, non-placebo controlled phase was to determine the safety of Bronchitol in patients with cystic fibrosis following 18 months of treatment and to assess the long term effects on lung function.

"Bronchitol is the first inhaled drug, formulated as a dry powder to report results of this nature in [cystic fibrosis]," Dr Robertson said.

"It offers convenience for patients who otherwise have to deal with complex daily treatment regimens," Dr Robertson said.

Pharmaxis said 97 subjects consented to participate in the open label phase and of these, 81 patients completed the first six months of the open label phase.

Of these 42 continued in the second six months of the open label phase of which 38 completed the trial.

The average age of the original 97 subjects in the open label phase was 23 years and the mean lung function on entry was 65.5 percentof the predicted normal FEV1.

Pharmaxis said the majority of adverse events were mild to moderate in severity and many of the frequently reported adverse events were a consequence of the underlying disease.

Reported possible treatment related adverse events included the condition being aggravated (4.1%), cough (5.3%) and haemoptysis (5.3%).

Pharmaxis said the trial was conducted in 40 centres in the UK, Ireland, Australia and New Zealand and further data including other lung function parameters would be presented at the European Cystic Fibrosis Conference in Valencia, Spain in June 2010.

Bronchitol is designed to hydrate the airway surface of the lungs and promote normal lung mucus clearance.

It has orphan drug designation and fast track status from the US Food and Drug Administration and orphan drug designation from the European Medicines Agency.

A marketing application has been submitted and is under review by the EMA.

Pharmaxis fell 11 cents or 3.3 percent to $3.18.

CLINUVEL

Clinuvel expects to earn its first commercial revenues from afamelanotide (formerly CUV1647) following special Italian approval for erythropoietic protoporphyria.

Clinuvel said that following requests from Italian patients for the ultra-violet light protective drug, the Agenzia Italiana del Farmaco (Italian Medicines Agency) has allowed its use under Law 648/96 and the Italian National Health System would reimburse afamelanotide for patients with erythropoietic protoporphyria (EPP)

The company said that under Law 648/96 the Agency could approve access in exceptional circumstances to, and reimbursement for, treatments which are unapproved in Italy, or treatments in clinical development for patients with disorders where no alternative therapy exists.

Clinuvel said the 36 drugs had been approved under Law 648/96 since its introduction in 1996 and afamelanotide was the first drug under clinical investigation and pharmaceutical therapy in the symptomatic treatment of porphyria to be approved under the law.

Clinuvel said that the prevalence of EPP in Italy was unknown, but 60 patients would have access to afamelanotide treatment.

Clinuvel chief executive officer Dr Philippe Wolgen said the 648/96 Italian listing was “a confirmation of our global regulatory strategy and will see the company generate revenue from afamelanotide for the first time”.

“The revenues will enable us to accelerate the development of a paediatric dose for juvenile EPP patients,” Dr Wolgen said.

Clinuvel said the Italian decision precedes approval of formal marketing authorization by European or US drug regulatory agencies.

Clinuvel spokesman Lachlan Hay told Biotech Daily that all 101 patients in the European and Australian phase III EPP trial had been dosed and results were expected by July 2010.

Mr Hay said that the FDA-approved 60-patient, orphan status phase II EPP trial began earlier this year (BD: Mar 30, 2010).

“Five years ago, Clinuvel made a decision to develop afamelanotide specifically for patient populations who were most severely and acutely affected by UV and light,” Dr Wolgen said.

“Today’s news comes as a welcome surprise but supports our choice to develop afamelanotide in these categories of patients,” Dr Wolgen said.

“Following the completion of the complex study of afamelanotide in Italian EPP patients, we continued to support their treatment through a compassionate use program,” Dr Wolgen said.

“Unfortunately, the indefinite free supply of afamelanotide was not sustainable for Clinuvel as a small enterprise and this approval by the Italian regulators not only makes the drug available to these trial patients, but also provides further incentive to the company to focus on orphan drug development,” Dr Wolgen said.

Clinuvel said with erythropoietic protoporphyria was characterized by intolerable pain, skin burns, blisters and scars when exposed to normal levels of light and sun.

The disease is incurable and affects patients for life.

The company said erythropoietic protoporphyria patients were forced to spend most of their lives indoors and sunscreens were of no use as they don’t block out visible light in the blue spectrum, which was the cause and trigger of toxic reactions.

Clinuvel said afamelanotide worked by activating pigment, melanin, in the skin providing a barrier between light and skin cells.

Clinuvel was up half a cent or 2.2 percent to 23.5 cents with 2.1 million shares traded.

## CORRECTION: LINKAGE GRANTS, NHMRC FUNDING

In Last Thursday's edition (BD: May 13, 2010) Biotech Daily expressed concern over the demise of the International Science Linkage program, along with an apparent reduction in National Health and Medical Research Council funding.

Biotech Daily has received several reader inquiries and the Department of Innovation says the International Science program was a 10-year program always expected to close on June 30, 2011 and has funds to reach that destination.

The Department of Innovation said the International Science Linkage program was entirely separate from the Australian Research Council Linkage program.

The Australia Research Councils' Linkage program is worth about $130 million in grants each year and has not been affected by last week's Budget.

A spokesman for the National Health and Medical Research Council directed Biotech Daily to the Council's website which said the 2010 budget reaffirmed the Government's commitment to NHMRC's forward estimates and there were no changes in Government policy on the Medical Research Expenditure Account.

http://www.nhmrc.gov.au/media/noticeboard/notice10/budgetstatement2010.htm.

The NHMRC website said it was aware of concerns over the interpretation of the Government's Portfolio Budget Statements 2010-11 and said there were separate tables with estimates of forecast expenditure and amounts of funding appropriated to medical research reflecting past funding decisions.

Research Australia chief executive officer Rebecca James told Biotech Daily she accepted Federal Government assurances of support for health and medical research.

That both Biotech Daily and Research Australia were confused by the detail surrounding the Budget indicates how difficult it must be for the general public to understand and interpret the Budget meaningfully.

Perhaps greater transparency might be the key.

Certainly, had the Federal Government adopted the Biotech and Related Industries Group Commercialisation Australia proposal (Jul 6, 2009) for a one-stop information shop for all grants, this level of confusion could not have occurred.

**David Langsam**
**Editor**

## CBIO

CBio has secured up to $12.45 million in a three year convertible loan agreement with the New York-based Springtree Special Opportunities Fund.

CBio said the minimum available under the facility was $5.45 million, with the first tranche of $200,000 available within the coming days.

The company said the remaining funds could be drawn down each month in tranches of between $150,000 and $350,000, subject to the requisite shareholder approvals.

CBio said the loans would be repaid through the issue of shares and options according to a formula contained within the agreement.

CBio managing director Jason Yeates said the funding was "a significant aspect of the company's business plan into 2011 and beyond".

"It provides working capital needed to complete the current phase II clinical trial as well as funding resources needed to explore a number of development activities planned for 2011," Mr Yeates said.

CBio said it was completing a 150-patient, phase IIa rheumatoid arthritis trial in Australia, New Zealand and Europe with results expected in mid-2011.

CBio was up 1.5 cents or 3.85 percent to 40.5 cents.

## CBIO

CBio said it would conduct a share plan at 35 cents a share, but did not state how much it hoped to raise.
CBio recently had its initial public offer and raised $7.1 million despite telling the market it expected to raise up to $30 million at $1 a share.
Shareholders eligible at the record date of May 21, 2010 would be able to apply for parcels of shares from $2,000 to $15,000.
CBio said the share plan would open on May 27 and close on June 16, 2010.
The company said the funds were primarily for the remaining costs associated with completing the rheumatoid arthritis clinical trial, including the compilation of final study reports.

## PHARMAUST

Pharmaust says it has raised $215,738 through the issue of 107,869,225 options at 0.2 cents per option.
When announced last month, Pharmaust said it hoped to raise up to $254,708 through the one option for two shares rights issue.
Pharmaust was unchanged at 6.5 cents.

## GIACONDA

Giaconda and Australian Medical Therapy Investments are continuing negotiations over the sale of Myoconda despite passing today's revised deadline for completion.
The Australian Medical Therapy Investments managing director Colin Goldrick told Biotech Daily the terms of the proposed sale remained the same as previously published and negotiations were continuing.
Giaconda has previously told the ASX that the Myoconda intellectual property would be sold to AMTI for $928,000 (excluding GST) plus five percent of the net sales earned by AMTI from a commercialized treatment (BD: Mar 9, 19, 2010).
Myoconda is a combination of three antibiotics and has been developed for treating Crohn's disease.
Giaconda was untraded at 4.6 cents.