Biotech Daily

Monday November 11, 2013

Daily news on ASX-listed biotechnology companies

*** ASX DOWN, BIOTECH EVEN: PATRYS UP 51%, PHYLOGICA DOWN 19%**

*** CLINUVEL US PHASE III EPP TRIAL MISSES PRIMARY ENDPOINT**

*** FDA FURTHER DELAYS INVION PHASE II INV102 SMOKING CESSATION TRIAL**

*** MESOBLAST STEM CELLS IMPROVE STROKE RECOVERY IN RATS**

*** FDA COMMITTEE BACKS COCHLEAR HYBRID HEARING IMPLANT**

*** PATRYS PAT-SM6, CARFILZOMIB TRIAL FOR MULTIPLE MYELOMA**

*** ANTISENSE RECEIVES $974k FEDERAL R&D TAX REFUND**

*** QRX $5m PLACEMENT, $3m SHARE PLAN**

*** PHYLOGICA UNDERWRITTEN $6m RIGHTS ISSUE, NOTES**

*** SUDA DEAL, CAPITAL RAISING HALT; HIRES TORREYA FOR DEALS**

*** JASON PETERSON, CELTIC CAPITAL CEASE CONSEGNA SUBSTANTIAL**

*** ROBERT THOMAS REPLACING STARPHARMA CHAIR PETER BARTLES**

*** IMPEDIMED APPOINTS DAVID ADAMS SECOND US DIRECTOR**

MARKET REPORT

The Australian stock market fell 0.25 percent on Monday November 11, 2013 with the S&P ASX 200 down 13.6 points to 5,387.1 points. Fifteen of the Biotech Daily Top 40 stocks were up, 15 fell, seven traded unchanged and three were untraded.

Patrys was best, up as much as 73.6 percent to 9.2 cents before closing up 2.7 cents or 50.9 percent at eight cents with 215.1 million shares traded. Atcor was up 26.7 percent; Anteo climbed 23.0 percent; Neuren was up 10 percent; Psivida was up 7.1 percent; Alchemia, Allied Health and Cellmid rose more than five percent; Benitec, Pharmaxis and Prana were up four percent or more; Acrux, Clinuvel and Resmed rose more than two percent; with Cochlear, IDT and Mesoblast up more than one percent.

Phylogica led the falls, down 0.4 cents or 19.05 percent to 1.7 cents; Tissue Therapies fell 8.5 percent; Genetic Technologies and GI Dynamics lost more than seven percent; Ellex was down 6.7 percent; Living Cell fell 5.6 percent; Avita and Universal Biosensors fell more than four percent; Impedimed and Starpharma were down more than three percent; Medical Developments, Nanosonics and Viralytics shed more than two percent.

CLINUVEL PHARMACEUTICALS

Clinuvel says its phase III US study of Scenesse failed to meet its primary endpoint, but treated patients showed “a strong trend” for sunlight exposure compared to placebo.

Clinuvel said that 87 adult erythropoietic protoporphyria (EPP) patients completed the six-month, randomized, multi-centre, double-blind, placebo-controlled study of Scenesse (afamelanotide) with results showing that Scenesse improved patients’ ability to expose their skin to light and improved their quality of life.

Clinuvel said the study’s primary study endpoint was to determine the extent to which patients could expose their skin to direct sunlight between 10am and 6pm.

The company said that the median total direct sunlight exposure was 64.13 hours (range 0.0 hours to 650.5 hours) in the active group compared with 47.5 hours (range 0.0 to 224.0 hours) for placebo-recipients ($p = 0.107$).

Clinuvel said the distribution of the number of days with sun exposure of various blocks of time was significantly different between the treatment groups ($p < 0.001$) with Scenesse recipients reporting more days when they had pain-free exposure of 60 minutes or more, the time of greatest risk of burns.

The company said that further data analyses, including objective photo-provocation, confirmed the ability of patients on active treatment to tolerate greater light exposure and spend more time outdoors.

Clinuvel said that the secondary endpoint of quality of life was evaluated using the validated questionnaire with significant improvement for active drug recipients.

The company said that the Scenesse safety profile was good with headaches and nausea the most common adverse events, with slightly more reports in the treatment group.

Clinuvel chief executive officer Dr Philippe Wolgen said the company had “focused on delivering a therapy for these patients for more than eight years”.

“Overall these results indicate that afamelanotide provides an effective treatment for patients with EPP and add to the body of evidence currently being reviewed for marketing authorization by European regulatory authorities,” Dr Wolgen said.

Clinuvel said that in 2011 phase II US and phase III EU erythropoietic protoporphyria trials showed Scenesse could reduce the severity of erythropoietic protoporphyria symptoms and enable patients to lead more normal lives and a marked improvement in quality of life was also reported (BD: Feb 9; Nov 4, 2011).

Thus far, no serious safety concerns have been identified from the use of afamelanotide in more than 900 patients, including more than 300 erythropoietic protoporphyria patients, involved in various clinical trials.

New York Mount Sinai School of Medicine dean of genetic and genomic medicine and lead investigator Prof Robert Desnick said the program was “the first to fully and rigorously evaluate a therapy for EPP, a disease which is poorly understood globally and presents uniquely in the clinic”.

“The results reflect numerically what our patients reported in the clinic: when treated with afamelanotide they can spend more time outside, experience less pain, and lead more normal lives,” Prof Desnick said.

“Professionally this is satisfying, as we may now, finally, be able to tell EPP patients that we can manage or prevent their painful symptoms and give them a freedom never before experienced,” Dr Desnick said.

Clinuvel said it was awaiting a response from the European Medicines Agency on a marketing authorization application for Scenesse for erythropoietic protoporphyria and was is seeking a meeting with the US Food and Drug Administration to discuss a New Drug Application in the first quarter of 2014.

Clinuvel was up four cents or 2.7 percent to $1.50.

INVION (FORMERLY CBIO)

Invion says the US Food and Drug Administration has changed responsible divisions for its 136-patient phase II trial of INV102 for smoking cessation.

Invion said that the FDA transferred the protocol for the trial in patients with chronic bronchitis from the Division of Pulmonary, Allergy, and Rheumatology Products to the Division of Analgesia, Anesthesia and Addiction Products and a 30-day review period was underway.

Invion chief medical officer Dr Mitchell Glass told Biotech Daily that the company had filed its new investigational new drug application to the Division of Analgesia, Anesthesia and Addiction Products and if there was no objection from the Department would begin the trial by the end of November.

Dr Glass said the phase II trial had already initiated sites began recruitment under the previous application but had not randomized or treated any patients.

Dr Glass said that the trial required 104 evaluable patients undertaking a 10-12 week treatment followed by a two-week washout and three months of follow-up.

Dr Glass said Invion expected to have substantial data from the trial by May 2014 and complete results by the end of 2014.

In February, Invion's then chief executive officer Dr William Garner said he expects results from the smoking cessation and two other phase II trials in 2013 (BD: Feb 27, 2013).

Dr Garner said at that time that the company's 120-patient, randomized, controlled, phase II trial of INV102, an oral beta adrenergic inverse agonist (beta blocker) also known as Nadolol, for smoking cessation, was about to begin "imminently".

Dr Garner said at that time that 15 percent of all people who attempted to give up cigarettes and failed, cited coughing of phlegm as the reason for failure.

He said that INV102 was able to repair epithelial lung cells so that when smokers stopped, there was a significant reduction in mucous-producing cells, reducing the cough.

Today, Dr Glass said that along with forced expiration volume over one second (FEV-1) to measure overall lung function, the trial would measure sputum output, reduction of inflammatory events and urinary nicotine levels.

Dr Glass said that the chronic obstructive pulmonary disease (COPD) patients in the trial were "a challenging group to treat" and the company hoped to expand from the oral capsule INV102 treatment for smoking cessation to an inhaled treatment to repair lung damage in chronic obstructive pulmonary disease patients.

Ina media release, Invion said that the Division of Analgesia, Anesthesia and Addiction Products (DAAAP) regulates applications for prescription drugs and biologics intended for the prevention and treatment of nicotine addiction, amongst other indications.

Dr Glass said that the INV102 for asthma trial, funded by a US National Institutes for Health grant, was being developed under an IND to the Division of Pulmonary, Allergy, and Rheumatology Products (DPARP) and would remain with that division.

"DAAAP will provide greater insight into smoking cessation drug development, and our excellent rapport with the Agency has resulted in open communication to ensure the smoothest possible transition between divisions," Dr Glass said.

"We will now be observing hereditary and non-hereditary variable responses to INV102 in the sputum of patients with chronic bronchitis, with the aim of supporting our two goals of enabling an end of phase II meeting for smoking cessation in COPD patients, and supporting our expedited development of inhaled INV102," Dr Glass said.

In February, Dr Garner said that the company needed to demonstrate that the patients on the active drug in the trial had a better tobacco quit rate than the controls.

Invion was up 0.5 cents or 4.55 percent to 11.5 cents.

MESOBLAST

Mesoblast says that preclinical trial results demonstrate that its mesenchymal precursor cell can improve functional recovery following a stroke in a rat model.

Mesoblast said the results showed that a single intravenous injection of human mesenchymal precursor cell significantly enhanced sensorimotor recovery when administered up to seven days after an ischemic stroke in rats.

The company said that the results were presented at the Neuroscience conference in San Diego, California running from November 9 to 13, 2013.

Mesoblast said that in a sub-study, mesenchymal precursor cells increased neuronal activity and reduced the volume of infarct tissue.

The company said that 72 adult male nude rats underwent permanent right middle cerebral artery occlusion which resulted in focal right cerebral infarction and impairment of the contralateral sensorimotor function.

Mesoblast said that the rats were randomized into six groups of 12 animals and injected intravenously with either media alone (controls) or one million human mesenchymal precursor cells administered at six, 12, 24 or 48 hours post-occlusion, or day seven post-occlusion.

The company said that a single dose of one million human mesenchymal precursor cells administered intravenously at 6, 12, 24 or 48 hours post-occlusion significantly improved forelimb and hindlimb recovery compared to controls by day 30 ($p < 0.01$ for all doses) as well as body swing.

According to the National Institutes of Health website the elevated body swing test consists of lifting the rat by the base of the tail, whereby it swings its body to one side or the other; with repeated trials performed to see if it has a bias toward one side, which would be interpreted as a motor deficit.

Mesoblast said that administration of mesenchymal precursor cells as late as seven days post-occlusion significantly improved both forelimb recovery and body swing compared to controls ($p < 0.01$ for both parameters), to similar levels at day 30 as seen with earlier cell administration.

The company said that a sub-study of 16 additional subjects, using anatomical and functional magnetic resonance imaging to evaluate neuronal activity and infarct volume, seven days after a single intravenous injection of either one million mesenchymal precursor cells or control media was administered 24 hours post-occlusion.

Mesoblast said using blood oxygen level-dependent functional magnetic resonance imaging to determine neuronal activity following fore or hind paw stimulation, treated ratsd showed a significant increase in neuronal activity compared with controls in both the infarct area ($p < 0.01$) and in the primary motor cortex on the side of the infarct ($p < 0.05$).

The company said that using anatomical magnetic resonance imaging, mesenchymal precursor cell-treated animals had a 17 percent reduction in infarct volume compared with controls ($p < 0.05$) but this effect was not seen in the overall group using post-mortem histologic assessment, which might be a less sensitive measurement.

Mesoblast said that stroke was a leading cause of death in the US and a leading cause of serious long-term disability, costing the US an estimated $US38.6 billion a year.

Mesoblast chief executive Prof Silviu Itescu said the results “suggest that our proprietary stem cells have the potential to be used within a broad and clinically meaningful therapeutic time window for neuro-protection and tissue repair after an ischemic stroke”.

Mesoblast was up nine cents or 1.5 percent to $6.27 with 456,002 shares traded.

COCHLEAR

Cochlear says that a US Food and Drug Administration advisory committee has voted in favor of the Cochlear Nucleus Hybrid L24 implant system.
Cochlear said it was a “first of its kind” system for the treatment of adults with severe to profound sensori-neural hearing loss in the high frequencies and normal hearing to only mild hearing loss in the low frequencies, often referred to as “ski-slope” hearing loss.
The company said that the FDA panel voted in favor of the new hybrid device based on substantial clinical evidence demonstrating the safety and efficacy of the Nucleus Hybrid System in patients who met the candidacy criteria.
Cochlear said that the FDA was not bound by the recommendations of its advisory committees, but would consider the guidance during the final review of the new device.
The company said the Nucleus Hybrid System combined the natural hearing through acoustic amplification of low frequencies with the electrical stimulation of a cochlear implant for high frequencies in one device.
Cochlear said it was designed to deliver patients superior quality and clarity of sound in even the most difficult hearing situations, especially hearing in noisy environments.
Cochlear was up 58 cents or one percent to $57.14 with 348,026 shares traded.

PATRYS

Patrys says it is initiating an investigator-sponsored trial evaluating PAT-SM6 in combination with carfilzomib, in patients with relapsed and refractory multiple myeloma.
Patrys said the trial at Germany’s University of Würzburg was being funded by Amgen subsidiary Onyx Pharmaceuticals which owned the carfilzomib proteasome inhibitor marketed in the US under the brand name Kyprolis for injection and would be led by the University’s Department of Medicine’s director Prof Hermann Einsele.
The company said that clinical and preclinical studies of PAT-SM6 had shown evidence of activity in patients with relapsed and refractory multiple myeloma.
Patrys said that in its ongoing phase I/IIa clinical trial PAT-SM6 had demonstrated a lack of serious side-effects in treated patients, which might allow it to be safely administered in combination with carfilzomib and have the potential to improve current treatments for multiple myeloma (BD: Jul 24, Aug 13, 2013).
Patrys said Onyx would provide carfilzomib study drug for the trial.
Patrys chief executive officer Dr Marie Roskrow said that the opportunity to initiate an investigator-sponsored trial of PAT-SM6 in combination with Onyx’s carfilzomib was “a very exciting and major step forward for Patrys as it provides further external validation of our PAT-SM6 program”.
“Given the promising results we are seeing in the current Patrys’ phase I/IIa [multiple myeloma] trial, we are delighted to have the opportunity to further the clinical development of Patrys’ lead product by combining it with carfilzomib,” Prof Hermann Einsele said.
Patrys jumped as much as 3.9 cents or 73.6 percent to 9.2 cents before closing up 2.7 cents or 50.9 percent at eight cents with 215.1 million shares traded.

ANTISENSE

Antisense says it has received $974,187 from the Australian Tax Office under the Federal Government Research and Development Tax Incentive program.
Antisense said the rebate related to research and development expenditure for the year to June 30, 2013
Antisense remained suspended at 17 cents pending a consolidation from 1.7 cents.

QRX PHARMA

QRX says it is conducting a placement to raise about $5.0 million through the issue of shares at 60 cents a share with a share plan to raise a further $3 million.
QRX said the placement was being conducted on November 11 and 12, 2013.
The company said that the share plan record date was November 8, the plan would open on November 19 and close on December 6, 2013, with parcels of shares available to eligible shareholders up to $15,000.
QRX said the funds were to take the company through the anticipated US Food and Drug Administration Prescription Drug User Fee Act (PDUFA) date for Moxduo immediate release and provide working capital required for commercialization if approved.
QRX was in a trading halt and last traded at 71 cents.

PHYLOGICA

Phylogica says it has an underwriting agreement to raise $6 million through a non-renounceable two-for-three share rights issue at 1.5 cents a share.
Phylogica said it had agreed variations with holders of existing converting notes to enable the maximum benefit to the company from the rights issue.
Phylogica said that largest shareholder, Bernard and Dianne Hockings, as trustees of the B Hockings Superannuation Fund, agreed to underwrite the rights issue and the Hockings intended to seek sub-underwriters for a significant portion of their commitment.
Phylogica said it planned that the rights issue and associated documents would be lodged by November 18, 2013 to begin the formal rights issue timetable.
The company said the capital raising would provide funds for two years “assuming expenditure and continuation of the research and development tax rebate remain at current levels”.
Phylogica said that the Hockings were also the largest holder of the convertible notes, with 78 percent of those on issue.
The company said that the proposed timetable would enable the Hockings to manage their holding of shares so that on issue of the shares resulting from conversion of their notes, their holding would not exceed 20 percent.
Phylogica said the note holders had agreed to conclude the rights issue Christmas 2013.
Phylogica fell 0.4 cents or 19.05 percent to 1.7 cents.

SUDA

Suda has requested a trading halt “pending the release of announcements concerning a material transaction and capital raising”.
Trading will resume on November 13, 2013 or on an earlier announcement.
Separately, Suda said it had retained Torreya Partners to assist with partnering and asset sales, starting with the Artimist anti-malaria drug and SUD-001 for migraine.
Suda last traded at 3.7 cents.

CONSEGNA GROUP

Jason Peterson and Celtic Capital say they have reduced below five percent in Consegna.
Mr Peterson said that his company was diluted and reduced its holding between April 10 and September 25, 2013, with the single largest sale 5,082,055 shares for $188,036 or 3.7 cents a share.
Consegna was up 0.2 cents or 3.1 percent to 6.7 cents with one million shares traded.

STARPHARMA HOLDINGS

Starpharma says Robert Thomas will be appointed a non-executive director in December 2013 and assume the role of chairman in mid-2014 when chairman Peter Bartels retires.
Starpharma said Mr Thomas had a strong background in financial services and continued as a director on a number of listed companies in Australia and the US.
The company said that Mr Thomas had more than 35 years experience at Potter Partners (now UBS), County Natwest and Citigroup and was currently chairman of TAL Limited (formerly Tower Australia), Gragher Capital Securities and the NSW State Library.
Starpharma said that Mr Thomas was the immediate past chairman of Heartware and remained a non-executive director of Heartware as well as Reva, Virgin Australia and Biotron.
The company said that Mr Thomas held a Bachelor of Economics from Monash University.
Starpharma fell 3.5 cents or 3.85 percent to 87.5 cents.

IMPEDIMED

Impedimed says it has appointed David Adams as its second US director, effective from today November 11, 2013.
Impedimed said that Mr Adams was previously Medtronic's integration and divestitures vice-president and has experience with acquisitions and integrations, as well as legal matters, taxation mergers and acquisitions and strategic planning.
The company said Mr Adams was previously Medtronic cardiovascular business development and vascular business development and ventures vice-president.
Impedimed said that Mr Adams held a Bachelor of Science in accountancy from the University of Illinois and a Juris Doctorate from William Mitchell College of Law.
Impedimed fell half a cent or three percent to 16 cents.