Biotech Daily

Wednesday June 22, 2016

Daily news on ASX-listed biotechnology companies

*** ASX, BIOTECH EVEN: LIVING CELL UP 16%; ACTINOGEN DOWN 6%**

*** CLINUVEL BEGINS COMMERCIAL ROLL-OUT OF SCENESSE FOR EPP**

*** PRIMA: PHASE IIb 6-PATIENT DATA – 'IMP321 SAFE, WELL-TOLERATED'**

*** WEHI BREAST CANCER PREVENTION TRIAL 25% ENROLLED**

*** AUSTRALIAN PATENT FOR NEUREN'S AUTISM SPECTRUM TROFINETIDE**

*** PRANA ANALYSIS INDICATES PBT2 HUNTINGTON'S EFFICACY**

*** BIO-MELBOURNE 'LESSONS FROM THERANOS' BRIEFING**

MARKET REPORT

The Australian stock market slipped 0.07 percent on Wednesday June 22, 2016 with the ASX200 down 3.5 points to 5,270.9 points.

Sixteen of the Biotech Daily Top 40 stocks were up, 16 fell, seven traded unchanged and one was untraded.

Living Cell was the best, up one cent or 15.6 percent to 7.4 cents with 9.7 million shares traded, filing a response to an ASX query after the market closed that further data from its NTCell for Parkinson's disease trial would be presented at a conference tomorrow.

Neuren climbed six percent; Genetic Technologies was up 5.6 percent; Atcor, Cellmid, Clinuvel, Factor and Oncosil were up more than three percent; Biotron, Medical Developments, Nanosonics, Pharmaxis, Prana and Resmed rose one percent or more; with Ellex, Pro Medicus and Viralytics up by less than one percent.

Actinogen led the falls, down half a cent or 6.25 percent to 7.5 cents with 75,500 shares traded.

Antisense and Mesoblast fell more than five percent; Acrux and Prima lost four percent or more; Avita and Impedimed shed more than two percent; Admedus, Airxpanders, Bionomics, Orthocell, Polynovo and Sirtex were down more than one percent; with Cochlear, CSL, IDT, Reva and Starpharma down by less than one percent.

CLINUVEL PHARMACEUTICALS

Clinuvel says that Scenesse, or afamelanotide 16mg, has been launched in the Netherlands for patients with erythropoietic protoporphyria.

Clinuvel said that it had made the first commercial delivery of Scenesse under European marketing authorisation and patients with the rare genetic disorder, which caused light intolerance, would be treated "from this week", with the first treatments in Austria and Germany expected in July.

The company said that the Netherlands had "one of the largest known adult [erythropoietic protoporphyria] patient populations in Europe".

Clinuvel said that following acceptance of the drug as a specialty hospital product by Dutch authorities, Scenesse was the first approved standard of care for light intolerant (EPP), enabling reimbursement of the product for all adult patients in the Netherlands.

The company said that insurers in Germany and Austria had facilitated access to Scenesse for EPP patients.

Clinuvel said that all patients treated with Scenesse were being encouraged to participate in a post-authorization non-interventional treatment protocol as part of a disease registry required by the European Medicines Agency and it had established a pharmaco-vigilance system to monitor long term patients' safety during the commercial phase of the product.

The company said it was working to make Scenesse available at a uniform commercial price in European, focusing on those countries where the product had been used in clinical trials or compassionate use programs.

Clinuvel chair Stan McLiesh said that the "today is an extremely important day for EPP patients, their families and the physicians who treat them".

"I'm proud that our teams have successfully navigated the onerous European and national systems to arrive at this point, and I look forward to broadening the availability of Scenesse in the coming months," Mr McLiesh said.

Clinuvel climbed 14 cents or 3.4 percent to $4.25.

PRIMA BIOMED

Prima says that data from the first six patients in its phase IIb trial of IMP321 with paclitaxel for metastatic breast cancer, shows the compound is safe and well-tolerated.

Prima said that its active immunotherapy paclitaxel (Aipac) trial was a multi-national, randomized, double-blind, placebo-controlled, chemo-immunotherapy study of IMP321 with paclitaxel for hormone receptor-positive metastatic breast cancer.

The company said the six patients received 6mg doses of IMP321 with paclitaxel, and the dose was "safe and well tolerated with no drug related serious adverse events" and the data demonstrated activation of blood monocytes, dendritic cells and CD8 T-cells.

Prima chief medical officer Dr Frederic Triebel said the data from the initial open-label run-in cohort of six patients confirmed "the safety, pharmaco-kinetics and pharmaco-dynamics of IMP321 and we are encouraged to have met our anticipated timelines for recruitment".

"We will now start enrolling nine additional patients in the second cohort with 30mg of IMP321, with the results of both cohorts to be presented and compared in the fourth quarter of 2016," Dr Triebel said.

"Then the randomization phase with the recommended phase IIb dose will begin enrolling approximately 196 patients," Dr Triebel said.

Prima chief executive officer Marc Voigt said the interim results "significantly de-risk the remainder of the trial".

The company said that the trial's primary endpoint was progression-free survival.

Prima fell 0.2 cents or 4.3 percent to 4.5 cents with 1.4 million shares traded.

THE WALTER AND ELIZA HALL INSTITUTE OF MEDICAL RESEARCH

The Walter and Eliza Hall Institute has provided details of its 40-patient, phase I trial of denosumab to prevent breast cancer in women carrying the BRCA1 gene mutation.

Yesterday, the Institute reported pre-clinical results published in Nature Medicine showing that denosumab, which was used to treat osteoporosis and breast cancer that had spread to the bone, could inhibit RANKL, or tumor necrosis factor superfamily member 11, in BRCA1-deficient mice, curtailing mammary tumorigenesis (BD: Jun 21, 2016).

The Institute said that if the mouse data was confirmed in human studies, it would provide a non-surgical option to prevent breast cancer in women with elevated genetic risk, and a human clinical trial was underway.

Today, the Institute provided Biotech Daily with access to the details of the human clinical trial, entitled 'A pre-operative window study evaluating the biological effects of the RANK-Ligand (RANKL) inhibitor Denosumab on normal breast tissue from BRCA1 and BRCA2 mutation carriers', and also known as the BRCA-D trial.

The trial is being conducted at the Royal Melbourne Hospital, with Melbourne's Peter McCallum Cancer Centre recently joining the trial.

A Royal Melbourne Hospital executive told Biotech Daily that the 40 patients were divided into three arms, with 20 BRCA1 gene mutation patients, 10 BRCA2 gene mutation patients and 10 women who were at high risk of breast cancer but were not carriers of either the BRCA1 or BRCA2 gene mutation.

The executive said that the trial began in December 2014 and 11 patients had been recruited so far, which was considered a faster than expected rate.

The trial details, published on the Cancer Council of Victoria's Cancer Trials Link website said the trial was enrolling pre-menopausal women aged between 18 years to 50 years who had documented BRCA1 or BRCA2 mutation and were considering prophylactic mastectomy, or were willing to undergo two breast biopsies on separate occasions.

The Cancer Trials Link website said that the proof-of-concept pilot study intended to determine if short-term treatment with denosumab was a feasible chemo-prevention option against breast cancer for BRCA1 and BRCA2 mutation carriers.

The Link said that all women in the study would receive four doses of 120mg denosumab subcutaneously, or into the skin, over three months and following treatment the women would proceed to surgery as planned, or have a second breast biopsy.

The Trials Link said that patients would be followed up for one month after treatment.

NEUREN PHARMACEUTICALS

Neuren says it has been granted an Australian patent covering trofinetide for autism spectrum disorders.

Neuren said that the patent, entitled 'Treatment of Autism Spectrum Disorders using Glycyl-L-2-Methylprolyl-L-Glutamic Acid' provided coverage for disorders including Rett syndrome, Fragile X syndrome, Asperger syndrome and autism, until January 2032.

The company said that the new patent derived from an international patent application filed in 2012 and it had patent applications pending in the US, Europe, Canada, Japan, Brazil and Israel, all of which were derived from the same international application.

Neuren executive chairman Dr Richard Treagus said that the patent was "commercially important and timely" because the company had a patent protecting trofinetide for Rett syndrome and Fragile X syndrome and it was the first patent to be granted from a series of applications in the major pharmaceutical markets covering trofinetide broadly in autism spectrum disorders until 2032.

Neuren was up 0.3 cents or six percent to 5.3 cents with four million shares traded.

PRANA BIOTECHNOLOGY

Prana says that further analysis of its Huntington's disease trial shows that 90 percent of patients self-reporting improvement in thinking were in the active PBT2 drug group.
In 2014, Prana said the 109-patient phase II US trial met its primary endpoint of safety and tolerability in the (BD: Feb 18, Nov 18, 2014).
The company said at that time that patients were randomly assigned to receive daily doses of PBT2 250mg, PBT2 100mg, or placebo for 26 weeks and found that "PBT2 was generally safe and well tolerated in patients with Huntington's disease".
The study found that compared with placebo, neither PBT2 100 mg ($p = 0.772$) nor PBT2 250 mg ($p = 0{\cdot}240$) significantly improved the main composite cognition score.
The study said that compared with placebo, the trail making test part B score was improved in the PBT2 250 mg group ($p = 0.42$) but not in the 100 mg group ($p = 0.925$) and neither dose significantly improved cognition on the other tests.
Today Prana said that the new analysis by researchers and statisticians at the Washington, DC-based Georgetown Medstar University would be reported at the International Movement Disorder Society Congress in Berlin on June 22, 2016.
Prana director Prof Ira Shoulson said the analyses were "reassuring, and the findings provide clinical meaningfulness to the objective and statistically significant improvement we saw in trail making B testing of cognitive performance from the trial".
In 2015, the US Food and Drug Administration issued a partial clinical hold limiting the dose of PBT2 that could be given to Huntington's disease patients (BD: Feb 13, 2015).
Prana said at the time that the FDA had provided options to remove the hold and it would conduct additional animal neurotoxicity studies or identify a strategy for safely using a clinically relevant dose in humans in the planned phase III trial, and the FDA had not raised any concerns about PBT2 safety data in human trials conducted to date.
Prana was up 0.1 cents or one percent to 9.8 cents.

BIO-MELBOURNE NETWORK

The Bio-Melbourne Network says its July 5 Bio-Briefing will discuss the impact of the Theranos case on the sector.
The Network said the once-heralded Palo Alto, California-based start-up was under criminal investigation and facing a class action over its Edison method of blood-testing.
The Network said that the Bio-Briefing, entitled 'Bio-Tech Hype - Lessons from Theranos', would be co-hosted with Phillips Ormonde Fitzpatrick and discuss the implications for in-vitro diagnostics, personalized medicine and disruptive healthcare technology.
Bio-Melbourne Network chief executive officer Dr Krystal Evans said that was "great interest in the legacy of the Theranos story on the regulatory, governance and the investor landscape for health-care start-ups and the broader industry".
The Network said that Bio-Briefing would have a panel discussion with Universal Biosensors chief scientific officer Dr Alastair Hodges and business development manager Pierre Nathie, LBT chief executive officer Lusia Guthrie and Theranos former vice-president of human health products David Lester.
The Bio-Briefing will be held at the offices of Philips Ormonde Fitzpatrick, 333 Collins Street, Melbourne on July 5, 2016, with registration from 3:45pm with the panel discussion from 4pm to 5.30pm followed by a networking session.
To register go to: http://biomelbourne.org/event/healthtech-hype-lessons-theranos/.