Biotech Daily

Thursday February 16, 2017

Daily news on ASX-listed biotechnology companies

*** ASX, BIOTECH UP: ELLEX, STARPHARMA UP 6%, LIVING CELL DOWN 5%**

*** MESOBLAST 39-WEEK 'DURABLE' RA RESPONSE TO STEM CELLS**

*** MELBOURNE, QIMR, GARVAN GENE-MAP PANCREATIC TUMORS**

*** GI DYNAMICS: 'ENDOBARRIER SAFE, REDUCES ADOLESCENT BMI 10%'**

*** DORSAVI H1 REVENUE UP 46% to $2m, LOSS DOWN 59% TO $1.3m**

*** SUDA RECEIVES $856k FEDERAL R&D TAX INCENTIVE**

*** ATCOR SPHYGMOCOR IDENTIFIES HEART FAILURE TREATMENT**

*** MAYNE LAUNCHES MIKART'S GENERIC BUPAP IN US**

*** ANTISENSE PLANS TWO ATL1102 MS TRIALS THIS YEAR**

*** MEDIBIO APPOINTS JACK COSENTINO CEO, M-D; STARTS ON $389k**

*** RACE: 'FDA DEFINES NDA PATHWAY FOR BISANTRENE FOR AML'**

MARKET REPORT

The Australian stock market was up 0.12 percent on Thursday February 16, 2017 with the ASX200 up 7.2 points to 5,816.3 points. Seventeen of the Biotech Daily Top 40 stocks were up, 10 fell, 10 traded unchanged and three were untraded. All three Big Caps rose.

Ellex was the best, up seven cents or 6.25 percent to $1.19 with 1.3 million shares traded, followed by Starpharma up 6.21 percent to 77 cents with 286,659 shares traded.

Bionomics and Cochlear climbed more than four percent; Admedus, Cellmid, CSL and Mesoblast were up more than three percent; Atcor, Clinuvel, Osprey, Polynovo and Prima rose more than two percent; Medical Developments, Nanosonics, Oncosil and Prana were up more than one percent; with Pro Medicus, Resmed and Sirtex up by less than one percent.

Living Cell led the falls, down half a cent or 4.55 percent to 10.5 cents with 672,442 shares traded. Opthea and Orthocell lost more than three percent; Anteo, Genetic Signatures and Impedimed shed more than two percent; with Airxpanders, Factor Therapeutics, Pharmaxis and Viralytics down more than one percent.

MESOBLAST

Mesoblast says 39-week data from its 48-patient, phase II rheumatoid arthritis trial is "compelling" despite non-significance on secondary efficacy endpoints.

In a presentation and teleconference, Mesoblast chief executive Prof Silviu Itescu said that the company's MPC-300-IV mesenchymal precursor cells met the primary endpoints of safety and tolerability with no treatment-related serious adverse events at 39 weeks and equivalent rates of adverse events across the three randomized groups of 16 patients each, receiving either placebo, 1,000,000 mesenchymal precursor cells (MPCs) per kilogram of body weight, or 2,000,000 MPCs/kg in a single infusion, including 30 patients who had failed one or two prior biologic treatments, of which nine were controls, 10 received 1,000,000 MPCs/kg and 11 received the 2,000,000 MPCs/kg.

Prof Itescu said that overall the 2,000,000 MPCs/kg dose was the more effective, with the lower dose showing a greater response on most measures for the whole group, but the higher dose more effective for the sub-group of patients failing previous biologic treatments on most measures.

One of the efficacy endpoints, the American College of Rheumatology improvement criteria measured changes in swollen joints, as well as a pain scale and disability rating described as ACR20 for a 20 percent improvement, ACR50 for a 50 percent improvement and ACR70 for a 70 percent improvement.

Last year, when the company presented the 12-week data, Prof Itescu said that ACR20 was a very low bar, but ACR70 was a high bar, which was not met by any of the control patients, which was the same result in today's 39-week data for both the whole group and the sub-group (BD: Aug 9, 2017).

The company said the 39-week data showed a clear trend for the treated groups compared to the controls, but none reached significance on the three ACR measures.

Mesoblast said that on two other measures, the ACR-N "mean area under the curve" and the "mean change from baseline on the health assessment questionnaire disability index (HAQ-DI), the 2,000,000MPCs/kg dose was statistically significant for both the whole group and the sub-group.

Prof Itescu said that the data showed proof-of-concept and that given that ACR20 was sufficient for US Food and Drug Administration approval the company would seek clarity from the FDA on the requirements for a phase III pivotal trial.

Prof Itescu said that the company was involved in potential partnering discussions.

Prof Itescu said that not all patients in each group were assessed at 12 weeks and 39 weeks explaining the disparity on percentages given for improvement.

He said that in an analysis of ACR-N the 2,000,000MPCs/kg group achieved a statistically significant difference from placebo of $p = 0.004$, while equalling the 1,000,000MPCs/kg group in improvement from baseline at 39 weeks.

For the prior biologics treatment sub-group, Prof Itescu said the 1,000,000MPCs/kg group achieved statistical significance from placebo of $p = 0.019$, while the 2,000,000MPCs/kg group achieved statistical significance from placebo of $p = 0.012$.

He said that on all efficacy measures, the MPC-treated groups out-performed the controls.

Prof Itescu said that about one-third of rheumatoid arthritis patients were resistant to anti-tumor necrosis factor (TNF) biologic agents, and they had limited treatment options.

"The nine-month outcomes generated from this study are highly encouraging," Prof Itescu said.

"The early and durable effects seen from a single infusion of 2,000,000MPC/kg support the potential of our allogeneic cell therapy to be positioned as an early treatment option for patients resistant to anti-TNF agents," Prof Itescu said.

Mesoblast was up six cents or 3.8 percent to $1.65 with 996,388 shares traded.

THE UNIVERSITY OF MELBOURNE; GARVAN, QIMR

The University of Melbourne says that genetic changes normally linked to breast, colon and ovarian cancers could also drive a rare form of pancreatic cancer.

A media release from the University of Melbourne, the Garvan Institute of Medical Research and the Queensland Berghofer Institute of Medical Research said that up to 20 percent of patients with pancreatic neuroendocrine tumors had a clear genetic predisposition for their cancers, despite no family history of the disease.

The media release said that the findings could lead to identifying people at risk of the cancers, as well as aggressive forms of the disease, and they might respond to current or new targeted therapies.

The research article, entitled, 'Whole-genome landscape of pancreatic neuroendocrine tumours' was published in Nature with an abstract available at: http://www.nature.com/nature/journal/vaop/ncurrent/full/nature21063.html.

The media release said the group collaborated with the University of Queensland and the Westmead, Sydney-based Children's Medical Research Institute, as well as the University of Verona, the University of Glasgow and the Houston, Texas-based Baylor College of Medicine.

The University of Melbourne said that the researchers carried out whole genome sequencing of tumors from 100 Australian patients recruited through the Australian Pancreatic Cancer Genome Initiative.

University of Melbourne study co-leader Prof Sean Grimmond said researchers were surprised to find striking similarities between the genetic drivers of pancreatic neuroendocrine tumors (Pannets) and other cancers.

"We found that the MUTYH and BRCA2 gene mutations, normally associated with colon and breast cancers, also appear to play an important role in Pannets," Prof Grimmond said.

"This raises exciting possibilities for how we treat this disease in the future," Prof Grimmond said.

The Queensland Berghofer Institute's Dr Nicola Waddell said that people without a family history of cancer could still carry a faulty gene that increased their risk of developing the tumor.

"The genetic variants we've identified may help to predict how aggressive each patient's tumour is and what sort of treatment they're likely to benefit from," Dr Waddell said.

"In the future, patients at risk of this rare pancreatic cancer could be identifiable through genetic screening," Dr Waddell said.

The media release said that Pannets accounted for about two percent of the 3,000 cases of pancreatic cancer diagnosed in Australia each year.

The Garvan Institute's Amber Johns said that although patients often had a better prognosis than those with more common pancreatic cancers, this group of tumors was "highly unpredictable".

"Doctors currently face the challenge of being unable to tell apart patients who would benefit from early aggressive therapy from those who might be spared harsh treatment for less invasive cancers," Ms Johns said.

Ms Johns said the study data would be freely available to cancer researchers and clinicians, to build on this work.

The media release said that the project was the latest Australian contribution to the International Cancer Genome Consortium, a global research effort focused on mapping out the genetic landscape, causal mutations and novel therapeutic opportunities in the 50 most common cancer types across the globe.

GI DYNAMICS

GI Dynamics says preliminary results from a 20-adolescent trial in Slovenia shows its Endobarrier reduced body mass index (BMI) by an average 10 percent.

GI Dynamics said that the trialof the Endobarrier duodenum insert at the Ljubljana-based University Children's Hospital led by Dr Tadej Battelino had completed enrolment of morbidly obese adolescents.

The company said that the single-arm study was designed to determine the efficacy and safety of Endobarrier treatment for up to one year and the preliminary data showed metabolic improvements and no serious adverse effects.

"The preliminary data show that treatment with Endobarrier has the potential to treat pre-diabetic, severely obese adolescents, especially those with metabolic complications," Dr Battelino said.

The company said the Endobarrier demonstrated therapeutic benefit in treating pre-diabetes, with blood sugar levels measured by HbA1c reduced by six percent and a 49 percent improvement in a measure of insulin resistance and beta-cell function.

GI Dynamics said that there was a clinically significant 43 percent reduction in triglycerides and a six percent reduction in systolic blood pressure, with all subjects completing treatment to the intended 12-month implant duration and no devices removed due to adverse events.

The company said there were no serious device-related adverse effects, but some patients reported abdominal discomfort, mild pain, nausea and gastrointestinal issues.

GI Dynamics chief executive officer Scott Schorer said that the study "in conjunction with many additional investigator-initiated studies around the world, is completing a picture of clinical evidence that further reinforces the broader safety and efficacy profile of Endobarrier".

GI Dynamics was up 0.7 cents or 16.7 percent to 4.9 cents with 2.3 million shares traded.

DORSAVI

Dorsavi says that revenue for the six months to December 31, 2016, was up 45.6 percent to $1,953,606 reducing net loss after tax 59.4 percent to $1,263,208.

Dorsavi said that sales revenue for its wearable body movement sensor systems increased by 33.8 percent to $1,728,724.

The company said that diluted loss per share fell 61.8 percent from 2.20 cents in the previous year to 0.84 cents for the six months to December 31, 2016, with net tangible asset backing per share constant at six cents.

Dorsavi said it had cash and cash equivalents of $8,726,634 at December 31, 2016, compared to $6,029,185 at June 30, 2016.

Dorsavi fell one cent or 2.33 percent to 42 cents.

SUDA

Suda says it has received $855,691 from the Australian Tax Office under the Federal Government Research and Development Tax Incentive program.

Suda said the rebate related to research and development expenditure for the year to June 30, 2016.

Suda chief executive officer Stephen Carter said the Research and Development Tax Incentive provided "a valuable benefit for companies such as Suda, so that we can invest more in our Oromist technology and pipeline of novel oral sprays".

Suda was untraded at 2.3 cents.

ATCOR MEDICAL

Atcor says that a study measuring central aortic waveform with its Sphygmocor device can predict which heart failure patients would respond to vasoactive drug therapy.

The research article, entitled 'Aortic Waveform Analysis To Individualize Treatment in Heart Failure' was published in Circulation: Heart Failure, with an abstract available at: http://circheartfailure.ahajournals.org/content/10/2/e003516.

Atcor said that the study, conducted at the Mayo Clinic in Rochester, Minnesota, aimed to determine whether central aortic pressure waveform analysis might guide treatment in patients with heart failure and reduced ejection fraction, when the heart did not contract effectively and less oxygen-rich blood was pumped into the arterial system.

The company said that individuals who benefited from vasoactive drug therapy were identifiable using its Sphygmocor central blood pressure diagnostic.

Atcor said that according to the study, these differences were not identifiable using brachial cuff pressures.

The article concluded that increased aortic pressure wave pulsatility and greater decrease in pulsatility on treatment were associated with functional improvement in patients with heart failure and reduced ejection fraction receiving aggressive vasodilator titration.

"These differences are not identifiable using brachial cuff pressures," the article concluded.

"Central aortic waveform analysis may enable better individualization of vasoactive therapies in chronic [heart failure] and reduced ejection fraction," the article concluded.

Atcor quoted the article saying that "in patients with heart failure and reduced ejected fraction … afterload reduction is a cornerstone in management".

"In the clinic, afterload is estimated by brachial cuff blood pressure, but the true hydraulic load … is more accurately represented by the central aortic pressure waveform, which can be assessed non-invasively using pulse waveform analysis," Atcor said.

Atcor chief executive officer Duncan Ross said the results were important because heart failure was "an increasingly common, costly and debilitating condition with the total cost to the US economy estimated at $US32 billion per annum".

"Sphygmocor was able to clearly identify the patients that would positively respond to more aggressive drug therapy while brachial cuff blood pressure could not," Mr Ross said.

"As the authors state, individualized or precision medicine has enabled major advances in delivering the right intervention to the right patient to improve outcomes; however, this has yet to be applied to most cardiovascular diseases, including heart failure," Mr Ross said.

"The findings again demonstrate that using Sphygmocor's measurements to manage patient outcomes leads to improved therapeutic outcomes," Mr Ross said.

Atcor was up 0.2 cents or 2.9 percent to seven cents.

MAYNE PHARMA GROUP

Mayne Pharma says it has launched 50mg and 300mg butalbital acetaminophen tablets for tension headache, or migraine, in the US.

Mayne said it would distribute the drug on behalf of partner, the Atlanta, Georgia-based Mikart Inc, which developed and manufactured the product.

The company said that US brand sales of butalbital acetaminophen, marketed as Bupap by Valeant Pharmaceuticals, were $US27 million for the year to December 31, 2016.

Mayne chief executive officer Scott Richards said that the Mikart drug was "the first generic alternative to Bupap and will bring patients and payers improved medication affordability".

Mayne was up half a cent or 0.35 percent to $1.415 with 9.2 million shares traded.

ANTISENSE THERAPEUTICS

Antisense says it hopes to file a US investigational new drug application for a 195-patient phase IIb trial of ATL1102 for multiple sclerosis by April 2017

Antisense said that monkey plasma samples from a previous toxicology study had been assayed and the pharmacokinetic data would be available for inclusion in the application.

The company said that it was hoping to find non-dilutive funding for the trial and the US Food and Drug Administration encouraged it to apply for an appropriate grant.

Antisense said it an unnamed US neurologist who had experience in grant funding for a phase IIb multiple sclerosis trial was interested in being the trial principal investigator.

The company said that it would submit a clinical study synopsis for the phase IIb trial this month to the FDA and, pending synopsis approval and investigational new drug application approval, would lodge the grant application by July 2017.

Antisense said that it proposed to conduct a 16-patient study of ATL1102 in relapsing secondary-progressive multiple sclerosis in Germany with Cologne City Hospital's Prof Volker Limmroth.

The company said that the study would investigate the efficacy, safety, and mechanism of action of ATL1102 dosed 200mg once weekly for 24 weeks and was expected to generate important and supportive data on the use of ATL1102 in this patient population and allow for the potential early access program treatment of patients who do not adequately respond to or tolerate existing therapies.

Antisense said that in August 2016 it submitted an application to the US National Multiple Sclerosis Society for grant funding for the study, but after "feedback from the clinical expert reviewers, a suitably revised application has subsequently been resubmitted".

The company said it expected notification of grants in late June 2017, which could allow for a potential study start by December 2017.

Antisense fell 0.1 cents or 2.4 percent to four cents.

MEDIBIO

Medibio says it has appointed Jack Cosentino as chief executive officer and managing-director, effective today and starting on a base salary of $US300,000 ($A388,922).

Medibio is developing a circadian cardiac rate based diagnostic for depression and mental illness and Mr Cosentino had been appointed as it focussed on milestones of product development, clinical trials, Conformité Européenne (CE) mark and US Food and Drug Administration clearance in 2017.

The company said that the Minnesota-based Mr Cosentino had more than 20 years in executive roles in medical device and technology companies and most recently was Impedimed's chief strategy officer, before that was Diversified Medical Corp's chief executive officer and previously was an executive at Medtronic.

The company thanked former chief executive officer Kris Knauer for "his outstanding leadership" and said that Mr Knauer would be the interim chief financial officer while the company looked for a chief financial officer with medical device experience, at which time Mr Knauer would transition to the role of non-executive director.

The company said that Mr Cosentino would have a base salary of $US300,000 a year along with $US1,200 a month for a "health stipend", one annual business class return fare from the US to Australia or Europe for his spouse and use of the Medibio Melbourne apartment when in Australia, and would be entitled to a short term incentive of up to 50 percent of his base salary, pending milestones and a long term incentive of 5,000,000 options exercisable at 45 cents each within five years.

Medibio was up 1.5 cents or four percent to 39 cents.

RACE ONCOLOGY

Race came out of a trading halt to say the US Food and Drug Administration “helped clearly define key aspects of the development pathway for Bisantrene”.

Race said that the pre-investigational new drug application meeting with FDA was “collaborative, encouraging and … confirmed that the proposed development of Bisantrene qualifies for the 505(b)(2) [new drug application] pathway in the US”.

The company said the new drug application pathway allowed a sponsor to use the available preclinical and clinical data on Bisantrene, which included more than 40 clinical studies supporting the tolerability and clinical utility of Bisantrene in the treatment of cancer, notably acute myeloid leukaemia.

Race was up half a cent or 2.6 percent to 20 cents.