Biotech Daily

Monday February 16, 2015

Daily news on ASX-listed biotechnology companies

*** ASX, BIOTECH UP: GENETIC TECHNOLOGIES UP 36%, PRANA DOWN 12%**

*** MESOBLAST 160-PATIENT DATA BACKS MSC-100-IV FOR GVHD**

*** FDA ORPHAN DRUG STATUS FOR NEUREN'S TROFINETIDE (NNZ-2566)**

*** CYNATA HIRES US CRO FOR STEM CELL STUDY**

*** ACTINOGEN READY FOR XANAMEM ALZHEIMER'S PHASE I TRIALS**

*** REPRODUCTIVE HEALTH APPOINTS ISRAEL, STH AFRICA DISTRIBUTORS**

*** SAFETY MEDICAL RETURNS AS 3D MEDICAL ON WEDNESDAY**

MARKET REPORT

The Australian stock market was up 0.19 percent on Monday February 16, 2015 with the S&P ASX 200 up 11.2 points to 5,888.7 points. Seventeen of the Biotech Daily Top 40 stocks were up, 12 fell, 11 traded unchanged and none were untraded.

Genetic Technologies was the best for the second trading day in a row, up 1.8 cents or 36 percent to 6.8 cents with 57.0 million shares traded, followed by Optiscan for the second trading day in a row, up 12.9 percent to 10.5 cents with 13.1 million shares traded and Nanosonics up 10.3 percent to $1.77 with 1.7 million shares traded.

Compumedics and Starpharma climbed more than seven percent; Universal Biosensors rose 6.7 percent; Alchemia and GI Dynamics were up more than four percent; Atcor, Bionomics, Living Cell, Medical Developments and Osprey were up more than three percent; Benitec and Impedimed rose more than two percent; with CSL, Psivida and Viralytics up one percent or more.

Prana led the falls for the second trading day in a row, down two cents or 11.4 percent to 15.5 cents with 703,395 shares traded.

Patrys lost 9.1 percent; Oncosil fell 8.8 percent; Circadian was down 5.6 percent; Analytica fell four percent; Avita was down 3.4 percent; Acrux, Anteo and Mesoblast shed two percent or more; Phosphagenics and Sirtex were down more than one percent; with Clinuvel, Cochlear and Resmed down by less than one percent.

MESOBLAST

Mesoblast says that data from 160 children treated with MSC-100-IV (formerly named Prochymal) for steroid-resistant acute graft versus host disease supports the treatment.

Mesoblast said that the lead investigator, Durham, North Carolina-based Duke University Medical Center's Prof Joanne Kurtzberg, said presented the data from its expanded access program at the American Society for Blood and Marrow Transplantation meeting in San Diego on February, 14, 2015.

The company said that the study showed "a clear and meaningful survival benefit among responding pediatric bone marrow transplant recipients".

Mesoblast said that 81 percent of the patients were severe, with 28 percent having grade C and 53 percent with grade D graft versus host disease, with reported survival probabilities of five to 20 percent.

The company said that overall response, defined as partial and complete response, at day-28 was 64 percent and this was correlated with statistically significant improved survival compared to non-responders at day-100 after an MSC-100-IV infusion of 81 percent for responders compared to 39 percent for non-responders ($p = 0.0001$).

Mesoblast said that the day-28 overall responses were 74 percent for grade B, 66 percent for grade C and 59 percent for grade D and overall responses by organ involvement at day-28 were 62 percent for gastro-intestinal response, 77 percent for skin and 53 percent for liver.

The company said that about 80 percent of grade B and C patients and more than 50 percent of grade D patients survived to day-100.

In 2013, research published in 'Biology of Blood and Marrow Transplantation' showed that use of Prochymal in 75 children, with a median age of eight years, with acute graft versus host disease (GvHD) gave an overall survival to 100 days of 47.9 percent compared to an average of 30 percent for grade C and D (BD: Nov 14, 2013).

Mesoblast acquired Prochymal along with the mesenchymal stem cell assets of Osiris one month earlier (BD: Oct 11, 2013).

The 2013 study, entitled 'Allogeneic Human Mesenchymal Stem Cell Therapy (remestemcel-L, Prochymal) as a Rescue Agent for Severe Refractory Acute GvHD in Pediatric Patients', was led by Prof Kurtzberg and co-authored by Osiris chief executive officer Dr Charles Randall Mills.

The 2013 study concluded that overall response at day-28 for patients treated for severe refractory acute graft versus host disease "was 61.3 percent and this response correlated with statistically significant improved survival 100 days post infusion of [human mesenchymal stem cells]".

"Patients who responded to therapy by day 28 had a higher … estimated probability of 100-day survival than patients who did not respond (78.1% vs 31.0%, $p < 0.001$) [and] prochymal infusions were generally well tolerated without any evidence of ectopic tissue formation," the abstract concluded .

Today, Mesoblast said that MSC-100-IV continued to be well tolerated with minimal clinically significant toxicities and the large expanded access program data set supported the benefit of MSC 100-IV for the treatment of acute graft versus host disease in children.

Mesoblast said that a 60-patient, single arm, open-label US phase III registration trial was underway progress.

"The enrolled patients represent a very challenging population with severe graft versus host disease that was non-responsive to treatments, including steroids and for many of these children, multiple immunosuppressive agents, so we believe these results are very promising," Prof Kurtzberg said.

Mesoblast fell 12 cents or 2.9 percent to $4.04 with 494,731 shares traded.

NEUREN PHARMACEUTICALS

Neuren says the US Food and Drug Administration has granted orphan drug designation for its trofinetide (NNZ-2566) for Rett syndrome.

Neuren said that orphan drug designation qualified the sponsor of the drug for seven years of market exclusivity following authorization.

Neuren has also applied for FDA breakthrough therapy status and executive chairman Dr Richard Treagus told Biotech Daily that the 60-day review period was due to conclude "by the end of this month"

The company said that the FDA granted orphan drug designation for trofinetide in Fragile X syndrome in October 2013.

Neuren said it had begun the process of orphan drug applications to the European Medicines Agency for trofinetide in both Rett syndrome and Fragile X syndrome.

The company said that European Union orphan drug designation qualified the sponsor for 10 years of marketing exclusivity following authorization.

"This further validates a key part of our strategy and the grant of orphan drug designation for Rett syndrome is another important commercial milestone for Neuren," Dr Treagus said. "The marketing exclusivity periods that apply to orphan drugs in the United States and Europe are extremely valuable in relation to the potential commercialization of trofinetide."

The company said that in November 2014, it announced top-line results from its phase II Rett syndrome trial, which demonstrated clinical benefit from treatment with trofinetide, encompassing many of the core symptoms of Rett syndrome and observed in both clinician and caregiver assessments.

The company said that it expected to meet the FDA by July 2015 to discuss the remaining requirements for the development of trofinetide in Rett syndrome.

Neuren said that there had been "growing interest in its key clinical programs" and it had posted a video describing trofinetide's mechanism of action on its website at: www.neurenpharma.com.

The company said that trofinetide was a synthetic analogue of a naturally occurring neuro-trophic peptide derived from insulin-like growth factor -1 (IGF-1), a growth factor produced by brain cells.

Neuren said that in animal models, trofinetide exhibited a wide range of important effects including inhibiting neuro-inflammation, normalizing the role of microglia and correcting deficits in synaptic function.

Neuren was unchanged at 17 cents with 5.8 million shares traded.

CYNATA THERAPEUTICS

Cynata says it has hired the Wilmington, North Carolina-based Pharmaceutical Product Development to begin planning and logistics development for a phase I stem cell study.

Cynata said that Pharmaceutical Product Development (PPD) would undertake and oversee initial planning, including the identification of optimal sites to launch the phase I investigation of its proprietary mesenchymal stem cell product.

The company said that its Cymerus technology enabled large scale production of mesenchymal stem cells from a single, one-time donor, which was "a pivotal requirement for pharmaceutical companies moving into stem cell medicine".

Cynata product development vice-president Dr Kilian Kelly said the appointment of a contract research organization was "a key step in commercializing this novel platform technology".

Cynata was up two cents or 5.1 percent to 41 cents.

ACTINOGEN MEDICAL

Actinogen says it has ethics approval for the up to 40-patient second phase I study of Xanamem, formerly known as UE2343, for Alzheimer's disease

Actinogen said that a 24-patient, double-blinded, placebo-controlled study would be conducted at Linear Clinical Research at the Queen Elizabeth II Hospital in Perth, Western Australia.

The company said that the healthy volunteers would be given doses of 10mg, 25mg and 35mg of Xanamem in a multiple ascending dose, with eight patients in each cohort.

Actinogen said that the primary endpoint was to confirm how the body absorbed and metabolized Xanamem and the optimal dose for the drug, with results expected by mid-2015.

The company said that two follow-on phase I studies would include "a fast fed study" of Xanamem in 12 patients and a cohort of four patients to confirm the central nervous system pharmacokinetics of Xanamem.

Actinogen said that the studies would add to the evidence base enabling an investigational new drug application to the US Food and Drug Administration's for a US phase II study of Xanamem.

Actinogen chief executive officer Dr Bill Ketelbey said that he was "well aware of the significant limitations of the few therapies that are currently available".

"We aim to demonstrate that Xanamem is a valuable treatment option for Alzheimer's disease and this trial is a very important further step in that process," Dr Ketelbey said.

The company said that Xanamem worked by blocking the development of the stress hormone, cortisol, in the hippocampus and frontal cortex, the areas of the brain most affected by Alzheimer's disease.

Actinogen said that there was growing evidence that chronic stress and elevated cortisol levels led to changes in the brain affecting memory and to the development of amyloid plaques and neural death, the hallmarks of Alzheimer's disease.

Actinogen fell 0.2 cents or 3.2 percent to 6.1 cents with 1.1 million shares traded.

REPRODUCTIVE HEALTH SCIENCE

Reproductive Health says it has signed distribution agreements with Israeli and South African distributors for the sale of its Embryocellect pre-implantation genetic screening kit.

Reproductive Health said it had signed a three-year distribution agreement with Al-Rad Medical for the sale of Embryocellect in Israel and the Palestinian Authority.

The company said that Al-Rad Medical was located near Tel Aviv and supplied all 25 Israeli in-vitro fertilization (IVF) laboratories with media, equipment and devices from leading manufacturers worldwide.

Reproductive Health said that about 32,000 in-vitro fertilization cycles were undertaken in Israel and claimed that Israel "provides free, unlimited IVF procedures for women up to the age of 45 for up to two babies ... [a] policy that made Israel the biggest user of IVF per capita in the world ... [with] four percent of children ... IVF babies, compared to the approximately one percent in the US".

Reproductive Health said it had signed a three-year distribution agreement with Delfran Pharmaceuticals for the sale of Embryocellect in the South African Development Community countries, which included Angola, Botswana, Democratic Republic of Congo, Lesotho, Madagascar, Malawi, Mauritius, Mozambique, Namibia, Seychelles, South Africa, Swaziland, United Republic of Tanzania, Zambia and Zimbabwe.

Reproductive Health was up 2.5 cents or 16.1 percent to 18 cents.

3D MEDICAL (FORMERLY SAFETY MEDICAL PRODUCTS)

The ASX says that the securities of 3D Medical, previously known as Safety Medical Products will be reinstated to official quotation from 10am (AEDT) on February 18, 2015. 3D or Safety Medical last traded at a post-consolidation five cents.