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Mesoblast CEO: We have $178 mn cash reserves

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Australia-based Mesoblast is one-of-the-world's leading developer of innovative biological products in regenerative medicine. Leveraging on its unique proprietary adult mesenchymal precursor cells technology, the company is targeting cardiovascular conditions, diabetes, inflammatory conditions of lungs and joints, cartilage degeneration and musculoskeletal conditions.

The company started the year on a positive note with positive results from the phase II clinical trial of their product NeoFuse that comprises allogeneic Mesenchymal Precursor Cells (MPCs) for lumbar spinal fusion. The company on January 30, 2013, also received US FDA's nod to commence a phase II clinical trial evaluating a single intravenous infusion of allogeneic, MPCs for the treatment of active rheumatoid arthritis.

In an interview with *BioSpectrum*, Professor Silviu Itescu, CEO, Mesoblast, speaks about the company's platform technology and its future plans for growth and commercialization of products.

Tell us in detail about Mesoblast's platform technology.
Mesoblast is developing advanced biotherapeutics based on its broad, proprietary and patented allogeneic adult Mesenchymal Precursor Cell (MPC) technology platform. This novel technology is being developed to focus on medical conditions with unmet needs such as diabetes and its complications, inflammatory and immune diseases of the joints and lungs, vertebral spine restoration, eye diseases and cardiovascular conditions.

The other businesses of the company include adult stem cell technology platform and high margin allogeneic or 'off-the-shelf' products readily available at time and place of need. Mesoblast's control of manufacturing enables the company to delineate products to support and separate partner markets, optimize reimbursement strategies and manage product life-cycles.

What is the major source of funding for the company?
Mesoblast's cash reserves ($178.6 million on December 31, 2012) enable simultaneous product development with future

revenues expected from milestone payments, distribution arrangements and direct sales. Publicly-listed on the Australian Securities Exchange (ASX) since 2004, there are approximately 288 million shares on issue, with approximately 80 percent ownership by institutional and sophisticated investors.

Share with us some of your alliances with other companies in the field.
Mesoblast has formed a strategic alliance with Teva Pharmaceutical Industries to develop and commercialize potential stem cell therapeutics for cardiovascular and central nervous system indications.

With Teva, Mesoblast is developing an adult stem cell-based therapy for the potential treatment of cardiovascular diseases, including congestive heart failure (CHF) and acute myocardial infarction (AMI), conditions that are the principal causes of hospitalization and death in the industrialized world. Mesoblast is in detailed discussions with Teva on a phase III trial design for CHF that will involve an early interim analysis to evaluate evidence of efficacy.

Additional cardiovascular indications being investigated with Teva include intracoronary injection of MPCs for prevention of heart failure after an AMI. An ongoing placebo-controlled phase II trial in 225 patients is actively recruiting. Mesoblast is also developing a stem cell therapeutic product for the potential treatment of various vascular diseases of the eye and is currently conducting a phase II trial for age-related macular degeneration (AMD) in Singapore and Australia.

A phase III clinical trial using MPCs to expand hematopoietic precursors from cord blood for transplantation in patients with hematologic malignancies is underway in the US, with plans to broaden the study to Europe. An agreement with biologics manufacturer Lonza has been formed for clinical and long-term commercial production of Mesoblast's allogeneic adult stem cell products. The alliance provides Mesoblast with significant commercial advantages, including certainty of capacity to meet long-term global supply of its proprietary MPC products.

What are the products in Mesoblast's pipeline and at what stages of clinical trials are they?
Among the intravenous administration products, Mesoblast is developing a high margin product franchise targeting a wide range of systemic diseases utilizing an intravenous formulation of allogeneic MPCs. These disorders include type 2 diabetes and its renal complications, inflammatory diseases of the joints such as rheumatoid arthritis, and inflammatory lung diseases such as asthma.

A phase II randomized, placebo-controlled dose escalation clinical trial is currently evaluating Mesoblast's MPC technology in 60 patients with type 2 diabetes at multiple trial sites in the US. In addition to monitoring safety and tolerability, the trial is evaluating glucose lowering effects of MPC treatment. This study will also explore effects on inflammatory markers including C-reactive protein (C-RP).

In January 2013, the FDA cleared a phase II trial to evaluate the effects of MPCs in patients with rheumatoid arthritis who have failed other biological therapies.

Additionally, Mesoblast is conducting preclinical studies to evaluate MPC effects in pulmonary conditions, such as asthma and idiopathic pulmonary fibrosis. In local administration products, Mesoblast is targeting degenerative diseases of the spine that represent a large and growing orthopedic market segment. These include development of a spinal fusion product, NeoFuse, for patients with advanced disc degeneration who need fusion surgery.

Encouraging phase II results suggested that NeoFuse is as effective for interbody lumbar fusion as the gold standard, bone autograft, and eliminates the need for autograft harvest and its attendant morbidity risks. Mesoblast is planning to begin a phase III trial for lumbar spinal fusion in late 2013. esoblast is also investigating a product in the much larger market of intervertebral disc damage. The results of a 100-patient phase II trial assessing MPCs for the treatment of disc repair are expected in mid-2013.

What were some of the highlights of 2012?
Clinical and preclinical development of intravenous product formulation for a wide range of systemic diseases is one highlight. The company generated positive preclinical results in primate model of type 2 diabetes; commenced phase II trial for type 2 diabetes and its complications; generated positive preclinical results in large animal model of inflammatory arthritis, showing concomitant effects on multiple pathways of joint inflammation; and plans are underway to initiate phase II clinical program in rheumatoid arthritis with additional planned applications including diabetic kidney disease, lung diseases.

Also, we completed enrollment in the phase II trials for non-surgical treatment of degenerative lumbar disc disease and for spinal fusion surgery. Phase II trial in congestive heart failure (CHF), identifying an MPC dose that prevented any hospitalization or death for a mean follow-up period approaching three years, was also completed.

The company also implemented product manufacturing strategy to facilitate scale-up, meet regulatory compliance for phase III and provide commercial supply. It reached an agreement with the US FDA on the manufacturing process to supply MPCs for use in the upcoming phase III trial for congestive heart failure, in phase III trials for other indications, and on the path forward for commercial supply.

What are the plans and strategies for 2013?
This year, we expect commencement of phase III trial for congestive heart failure involving an early interim analysis to evaluate evidence of efficacy; commencement of phase III trial for lumbar spinal fusion and clinical results in phase II trials with early type 2 diabetes and intervertebral disc repair.

Also, phase II trial for acute myocardial infarction and phase III for bone marrow transplant will continue. The company also plans to expand focus on intravenous product franchise with commencement of phase II trials for rheumatoid arthritis, diabetic kidney disease and lung diseases. We will explore additional partnering opportunities and optimal timing for growth.