asx announcement

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the regenerative medicine company

DURABLE THREE-YEAR OUTCOMES IN DEGENERATIVE DISC DISEASE AFTER A SINGLE INJECTION OF MESOBLAST'S CELL THERAPY

Melbourne, Australia; and New York, USA; March 15, 2017: Mesoblast Limited (ASX: MSB; Nasdaq: MESO) today announced 36-month results from the randomized, placebo-controlled 100-patient Phase 2 trial of its proprietary allogeneic Mesenchymal Precursor Cells (MPCs) in patients with chronic low back pain (CLBP) due to intervertebral disc degeneration. A single intra-discal injection of 6 million MPCs resulted in meaningful improvements in both pain and function that were durable for at least 36 months.

"The sustained benefits on pain and function over three years seen with a single injection of Mesoblast's cell therapy have the potential to transform the treatment paradigm for chronic low back pain due to disc degeneration," said trial investigator Dr Hyun Bae, Professor of Surgery and Director of Education at the Cedars Sinai Spine Center, and Director of the Spine Institute in Los Angeles, CA. "Instead of replacing or fusing the disc, there is mounting compelling evidence that we can use this regenerative medicine to heal the disc. We are fast approaching this inflection point in the treatment of low back pain, which is particularly important in view of the epidemic of opioid abuse."

The durable outcomes seen from a single MPC injection in patients with degenerative disc disease who have failed conservative measures are consistent with an overarching mechanism of action that may also be evident in treatment of other chronic diseases where a single MPC dose has resulted in sustained benefits, including advanced chronic heart failure and biological-resistant rheumatoid arthritis. In each of these diseases, MPCs are thought to be activated by signals in the damaged tissues to release factors that both inhibit damaging inflammation and induce a pro-reparative state.

The Phase 2 trial compared a single intra-discal injection of 6 million or 18 million MPCs against two placebo arms, saline or hyaluronic acid, using a pre-specified Per Protocol (PP) population analysis. The primary endpoint composite was the same as is being used in the ongoing Phase 3 trial, a 50% reduction in the Visual Analog Scale (VAS) pain score and a 15-point reduction in the Oswestry disability index (ODI), with no additional intervention, at both 12 and 24 months.

In line with United States Food and Drug Administration (FDA) guidance for the ongoing Phase 3 trial, the 24-month primary endpoint composite was additionally analyzed using an intent to treat (ITT) population. The 36-month analysis aimed to determine the proportion of patients who maintained treatment success beyond the 24-month primary evaluation.

Key trial results were:

- the primary endpoint composite over 24 months was achieved by 41% of patients who received 6 million MPCs, 35% of the 18 million MPC group, 18% of the hyaluronic acid group, and 13% of the saline group, using the pre-specified PP population analysis
  - pain responder criteria (50% pain reduction with no additional intervention at both 12 and 24 months) was achieved by 52% of the 6 million MPC group compared with 13% of the saline group ($p<0.05$)
  - functional responder criteria (15-point reduction in ODI and no additional intervention at both 12 and 24 months) was achieved by 48% of the 6 million MPC group compared with 13% of the saline group ($p<0.05$)
- similar results were seen for the primary endpoint composite over 24 months using the ITT analysis, with 38% of the 6 million MPC group achieving this outcome compared with 10% of the saline group ($p<0.05$)
  - 82% of the 6 million MPC group who achieved the primary endpoint composite over 24 months maintained treatment success using this composite endpoint at 36 months

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    - 86% of the 6 million MPC group who successfully met the pain responder criteria (50% pain reduction with no additional intervention at both 12 and 24 months) remained pain responders through 36 months
    - 92% of the 6 million MPC group who met the functional responder criteria (15-point reduction in ODI and no additional intervention at both 12 and 24 months) remained functional responders through 36 months
- there were no significant differences in measurements of safety between cell-treated patients and controls over 36 months

The 36-month Phase 2 trial results support the ongoing 360-patient Phase 3 trial of Mesoblast's product candidate MPC-06-ID for CLBP by reinforcing the rationale for MPC dose selection, use of saline control, and the trial's primary endpoint composite over 24 months. If similar clinical durability is seen in the Phase 3 program, it is anticipated such data will translate into meaningful health economic benefits including increased productivity that may support attractive product reimbursement.

In December 2016, Mesoblast and Mallinckrodt Pharmaceuticals entered into an agreement to exclusively negotiate a commercial and development partnership for MPC-06-ID in the treatment of chronic low back pain due to disc degeneration.

About Chronic Low Back Pain (CLBP) Caused By Degenerative Disc Disease

Over 33 million[1] patients in the U.S. alone suffer from CLBP with approximately 22%[2] caused by degenerative disc disease. Total costs of low back pain in the U.S. are estimated to be between US$100 billion and US$200 billion annually with two thirds of these costs attributed to patients' decreased wages and productivity.

All therapies for progressive, severe and debilitating pain due to degenerating intervertebral discs treat the symptoms of the disease, but are not disease-modifying and thus do not address the underlying cause of the disease.

Limited treatment options exist for patients who have failed conservative treatment (e.g. physical therapy, anti-inflammatory agents or analgesics) or other measures including opioids and epidural steroid injections. When disc degeneration has progressed to a point that pain and loss of function can no longer be managed by conservative means, major invasive surgery is the only remaining option. Even with surgical intervention such as spinal fusion or artificial disc replacement, over 50% of patients are considered treatment failures.

Mesoblast's MPC-06-ID Phase 3 program targets a patient population with significant unmet need who have exhausted conservative treatment options. MPC-06-ID is being developed to alleviate pain and improve function, either before the use of opioids and/or epidural steroid injections or after failure of these approaches, in order to prevent invasive and costly surgical interventions.

About Mesoblast

Mesoblast Limited (ASX:MSB; Nasdaq:MESO) is a global leader in developing innovative cell-based medicines. The Company has leveraged its proprietary technology platform, which is based on specialized cells known as mesenchymal lineage adult stem cells, to establish a broad portfolio of late-stage product candidates. Mesoblast's allogeneic, 'off-the-shelf' cell product candidates target advanced stages of diseases with high, unmet medical needs including cardiovascular conditions, orthopedic disorders, immunologic and inflammatory disorders and oncologic/hematologic conditions.

Forward-Looking Statements

This press release includes forward-looking statements that relate to future events or our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements should not be read as a guarantee of future performance or results, and actual results may differ from the results anticipated in these forward-looking statements, and the differences may be material and adverse. You should read this press release together with our risk factors, in our most recently filed reports with the SEC or on our website. Uncertainties and risks that may cause Mesoblast's actual results, performance or achievements to be materially different from those which may be expressed or implied by such statements, and accordingly, you should not place undue reliance on these forward-looking statements. We do not undertake any obligations to publicly update or revise any forward-looking statements, whether as a result of new information, future developments or otherwise.

[1] Decision Resource 2015 Chronic Pain Report
[2] https://academic.oup.com/painmedicine/article-lookup/doi/10.1111/pme.12809

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