asx announcement

mesoblast
the regenerative medicine company

MESOBLAST OPERATIONAL HIGHLIGHTS AND FINANCIAL RESULTS FOR THE PERIOD ENDED MARCH 31, 2017

Melbourne, Australia; May 25, 2017; and New York, USA, May 24, 2017: Mesoblast Limited (ASX: MSB; Nasdaq: MESO) today provided the market with operational highlights and financial results for the three and nine month reporting periods ended March 31, 2017. During the reporting period, the Company achieved a major milestone in its valuable heart failure Phase 3 program, maintained momentum in its additional Phase 3 trials, and continued to reduce spend.

During the first nine months of FY2017, the Company executed its planned operational streamlining and re-prioritization of projects to successfully absorb the incremental costs of the MPC-150-IM Phase 3 program in advanced chronic heart failure (CHF). Due to these measures, cash outflows for R&D product support costs, manufacturing, and management & administration were reduced for the nine months of FY2017 by US$16.4 million (24%), compared with the nine months of FY2016. For the third quarter of FY2017, cash outflows for the same operational activities were reduced by US$5.1 million (23%) compared with the third quarter of FY2016.

These savings enabled the Company to allocate sufficient funds for the CHF Phase 3 trial through to the successful interim futility analysis of the trial's efficacy endpoint in early April 2017.

After absorbing the incremental R&D costs associated with the CHF Phase 3 trial, together with increased spend on advancing the other Tier 1 product candidates in Phase 3 trials, total operating cash outflows were reduced by US$0.8 million as compared to the first nine months of FY2016.

As of March 31, 2017, the Company had cash reserves of US$69.1 million following a capital raising of approximately US$40 million. As previously announced, Mesoblast has established an equity facility for up to A$120 million/US$90 million, to be used at its discretion over the next two years to provide additional funds as required.

The Company intends to partner one or more of its four Tier 1 product candidates in order to increase cash reserves and further reduce cash burn. As previously announced, the Company is in exclusive negotiations with Mallinckrodt Pharmaceuticals in regard to a potential commercial and development partnership for two of its lead product candidates.

Key operational highlights for the quarter with respect to the Company's four Tier 1 product candidates were:

- Mesoblast's Phase 3 CHF trial of MPC-150-IM achieved a successful pre-specified interim futility analysis of the efficacy endpoint in the first 270 patients. After notifying the Company of the interim analysis results, the trial's Independent Data Monitoring Committee formally recommended the trial be continued as planned.
- A Phase 2 trial evaluating MPC-150-IM in children under the age of 5 with hypoplastic left heart syndrome (HLHS) undergoing corrective surgery was cleared by the United States Food and Drug Administration (FDA) to commence at Boston Children's Hospital.
- Results from Mesoblast's Phase 2 trial in patients with biologic refractory rheumatoid arthritis (RA) showed that a single 2m/kg injection of MPC-300-IV resulted in early and sustained responses through 39 weeks.
- Results from Mesoblast's Phase 2 trial in patients with chronic low back pain due to disc degeneration (CLBP) showed that a single injection of MPC-06-ID resulted in meaningful improvements in both pain and function that were durable for at least 36 months.
- Fast Track designation was granted by the FDA for the use of MSC-100-IV in children with acute graft versus host disease (aGVHD).

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Operational Update

MPC-150-IM is being developed for advanced and end-stage chronic heart failure (CHF) in New York Heart Association (NYHA) Class II/III and Class IV patients:

- Intramyocardial administration of MPCs in animal models of heart failure has resulted in improved cardiac function and attenuated pathological ventricular remodelling. These effects were attributable, at least in part, to MPC secretion of biomolecules that stimulate reparative processes in the failing heart including new blood vessel formation, cardiac muscle cell survival, and reduction in tissue fibrosis.

- In Phase 2 results, a single injection of MPC-150-IM by catheter into the endo-myocardium of patients with moderate to advanced chronic heart failure prevented any HF-related hospitalizations or cardiac deaths over three years of follow-up.

- MPC-150-IM, injected by catheter into the endo-myocardium, is being evaluated in a 600-patient Phase 3 trial of NYHA Class II-III advanced CHF patients.

    - In April 2017, the pre-specified interim futility analysis of the efficacy endpoint was successful in the trial's first 270 patients.

        - The trial's efficacy endpoint is a comparison of recurrent non-fatal heart failure-related major adverse cardiac events (HF-MACE) in moderate to advanced CHF patients receiving either MPC-150-IM by catheter injection into the damaged left ventricular heart muscle or sham control.

        - The statistical method uses the Joint Frailty Model to evaluate the trial's efficacy endpoint while accounting for increased likelihood of a terminal cardiac event (such as death, implantation of a mechanical heart assist device or a heart transplant) for patients with multiple HF-MACE.

        - After notifying the Company of the interim analysis results, the trial's Independent Data Monitoring Committee (IDMC) formally recommended the trial be continued as planned.

        - In line with best practice for blinded Phase 3 clinical trials, the interim analysis data were only reviewed by the IDMC. Mesoblast, the FDA, and trial investigators remain blinded to grouped safety and efficacy data for the ongoing trial as well as the numerical results of the interim analysis.

- MPC-150-IM, injected directly into the epicardium, is being evaluated in a Phase 2b trial in patients with NYHA Class IV/end-stage heart failure who have received a left ventricular assist device (LVAD).

    - The 159-patient, 2:1 randomized, placebo-controlled trial is being funded by the United States National Institutes of Health (NIH) and is being conducted by a multi-center team of researchers within the NIH-funded Cardiothoracic Surgical Trials Network (CTSN).

    - Enrollment of this trial is expected to be completed during 1H CY2017 with a data read-out expected in 2H CY2017.

- During the reporting period, the FDA cleared the commencement of a 24-patient trial combining MPCs with corrective heart surgery in children under the age of 5 with HLHS. The trial is sponsored and funded by the Boston Children's Hospital, the pediatric teaching hospital of Harvard University, with support from Bulens and Capozzi Foundation and the Ethan Lindberg Foundation.

- Under the United States 21st Century Cures Act, MPC-150-IM may be eligible for regenerative medicine advanced therapy (RMAT) designation for treatment of advanced and/or end-stage CHF in adults and children. Such designation may facilitate accelerated approval pathways for this product candidate.

MPC-300-IV is being developed for biologic refractory rheumatoid arthritis (RA):

- Results of a study were published in the peer-reviewed journal *Stem Cell Research & Therapy* in February 2017, showing that a single intravenous infusion of 150 million of the Company's proprietary allogeneic "off-the-shelf" STRO-3 immunoselected MPCs significantly improved clinical disease severity, reduced joint cartilage erosions, and improved synovial inflammation and histopathology in a large animal model of early RA.

- This study provides mechanistic and translational support for the clinical outcomes reported in the ongoing Phase 2 trial of MPC-300-IV for biologic refractory RA.

- Results from this 48-patient placebo-controlled, randomized Phase 2 trial evaluating two dosing regimens against placebo in RA patients resistant to anti-Tumor Necrosis Factor (TNF) agents showed that single intravenous infusion of MPC-300-IV resulted in durable responses through nine months (39 weeks). All three cohorts (2m MPCs/KG; 1m/MPCs/KG and placebo) were well matched for disease activity and other demographics at baseline. The results showed that:

    - The safety profile over 39 weeks was comparable among the placebo and both MPC treatment groups, with no cell-related serious adverse events reported.

    - Both MPC doses outperformed placebo at the week 39 follow-up in each of ACR20/50/70 responses, as well as by median ACR-N analysis.

    - The 2 million MPC/kg dose showed the earliest and most sustained treatment responses in this Phase 2 trial in the period assessed.

MPC-06-ID is being developed for chronic low back pain (CLBP) due to disc degeneration:

- The ongoing 360-patient Phase 3 trial for MPC-06-ID in patients with CLBP due to intervertebral disc degeneration is actively recruiting across U.S. and Australian sites with enrollment targeted to complete this year. The primary endpoint composite is 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 FDA guidance, the Phase 3 trial's 24-month primary endpoint composite is being analyzed using an intent to treat (ITT) population.

- The 36-month analysis from March 2017 of the randomized, placebo-controlled, 100-patient Phase 2 trial of MPC-06-ID aimed to determine the proportion of patients who maintained treatment success beyond the 24-month primary evaluation. Key trial results using the ITT analysis were:

    - 38% of the 6 million MPC group achieved the primary endpoint composite over 24 months 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
        - 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

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- 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 CLBP due to disc degeneration.

MSC-100-IV is being developed for Acute Graft Versus Host Disease (aGVHD):

- The pre-specified interim futility analysis of the primary endpoint of the ongoing 60-patient open label Phase 3 trial was successful in November 2016.

- This Phase 3 trial is expected to read out top-line results in 2H CY 2017.

- During the reporting period, the FDA granted a Fast Track designation for the use of MSC-100-IV to improve overall response rate in children with steroid refractory aGVHD.

    - Fast Track designation has the potential to shorten the time to FDA approval of MSC-100-IV for this indication through priority review (shortened FDA review process from 10 to 6 months) and a streamlined rolling review process (completed sections of the Biologics License Application, BLA, can be submitted for FDA review as they become available, instead of waiting for all to be completed).

    - The product candidate's existing Orphan Indication designation may additionally lead to potential commercial benefits following FDA approval.

- Based on guidance from the FDA, Mesoblast believes that data from this Phase 3 trial may be sufficient for filing for accelerated conditional approval of MSC-100-IV in the United States.

- Mesoblast plans to broaden the use of its technology platform with studies in adult patients with high-risk steroid-refractory acute GVHD.

- In December 2016, Mesoblast and Mallinckrodt Pharmaceuticals entered into an agreement to exclusively negotiate a commercial and development partnership for MSC-100-IV in the treatment of aGVHD.

Financial Results for the Three Months Ended March 31, 2017 (third quarter) (in U.S. Dollars)

The Company continued to execute its planned operational streamlining and re-prioritization of projects to successfully absorb the incremental costs of the MPC-150-IM Phase 3 program in CHF. Due to these measures, cash outflows for R&D product support costs, manufacturing, and management & administration were reduced by $5.1 million (23%) for the third quarter of FY2017, compared with the third quarter of FY2016. These reductions comprised: $3.9 million within manufacturing and $1.2 million within R&D product support costs.

There was an improvement of $8.2 million (39%) in the loss before income tax for the third quarter of FY2017, compared with the third quarter of FY2016. This was primarily due to non-cash items that do not affect our cash reserves, such as remeasurement of contingent consideration, finance costs and foreign exchange movements within other operating income and expenses. Additional items which impacted the loss before income tax movement were:

- **Manufacturing** expenses were $3.8 million for the third quarter of FY2017, compared with $7.7 million for the third quarter of FY2016, a decrease of $3.9 million due to sufficient quantities of clinical grade product previously manufactured for all ongoing clinical trials.

- **Research and Development:** After absorbing the incremental R&D costs associated with the CHF program, total R&D costs were $13.9 million, an increase of $1.9 million versus the comparative quarter in FY2016.

- **Management and Administration** expenses were relatively stable at $5.5 million for the third quarter of FY2017, compared with $5.4 million for the third quarter of FY2016.

- **Revenues** from sales of TEMCELL HS Inj.® (TEMCELL), a registered trademark of JCR Pharmaceuticals Ltd., increased from $0.1 million in the third quarter of FY2016 to $0.8 million in the third quarter of FY2017. There was a decrease of $3.2 million in total revenues for the third quarter of FY2017 compared with the third quarter of FY2016, primarily due to a non-cash deferred revenue item recognized in FY2016 related to our MPC-150-IM product.

Our net loss attributable to ordinary shareholders was $9.8 million, or 2.46 cents per share, for the third quarter of FY2017, compared with $16.9 million, or 4.49 cents per share, for the third quarter of FY2016.

Financial Results for the Nine Months Ended March 31, 2017 (the nine months) (in U.S. Dollars)

The Company continued to execute its planned operational streamlining and re-prioritization of projects to successfully absorb the incremental costs of the MPC-150-IM Phase 3 program in CHF. Due to these measures, cash outflows for R&D product support costs, manufacturing, and management & administration were reduced by $16.4 million (24%), compared with the nine months of FY2016. These reductions comprised: $11.1 million in manufacturing, $4.5 million within R&D product support costs and $0.8 million within management & administration.

There was an increase of $2.3 million (4%) in the loss before income tax for the nine months of FY2017, compared with the nine months of FY2016. This was primarily due to non-cash items that do not affect our cash reserves, such as remeasurement of contingent consideration, finance costs and foreign exchange movements within other operating income and expenses. Additional items which impacted the loss before income tax movement were:

- **Manufacturing** expenses were $10.9 million for the nine months of FY2017, compared with $22.0 million for the nine months of FY2016, a decrease of $11.1 million due to sufficient quantities of clinical grade product previously manufactured for all ongoing clinical trials.

- **Research and Development:** After absorbing the incremental R&D costs associated with the CHF program, total R&D costs were $43.0 million, an increase of $7.4 million versus the comparative period in FY2016.

- **Management and Administration** expenses were $15.9 million for the nine months of FY2017, compared with $16.7 million for the nine months of FY2016, a decrease of $0.8 million. This decrease was primarily due to planned operational streamlining.

- **Revenues** from royalties on sales of TEMCELL increased by $0.9 million in the nine months of FY2017, compared with the nine months of FY2016. There was a decrease of $13.8 million in total revenues in the nine months of FY2017, compared with the nine months of FY2016, primarily due to a non-cash deferred revenue item recognized in FY2016 related to our MPC-150-IM product.

Our net loss attributable to ordinary shareholders was $49.6 million, or 12.87 cents per share, for the nine months of FY2017, compared with $52.4 million, or 14.76 cents per share, for the nine months of FY2016.

Conference Call Details

Mesoblast will be hosting a conference call beginning at 8am AEST on May 25, 2017 / 6pm EST on May 24, 2017. The conference identification code is 528910.

The live webcast can be accessed via:
http://webcasting.boardroom.media/broadcast/592263cc45e9d25707f89993

To access the call, please dial:

Australia Toll Free	1 800 558 698
Australia Alternate	1 800 809 971
United States	1 855 881 1339
United Kingdom	0800 051 8245
Japan	0053 116 1281
Singapore	800 101 2785
Hong Kong	800 966 806
International	+61 2 9007 3187

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.

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Consolidated Income Statement

(in U.S. dollars, in thousands, except per share amount)	Three Months Ended March 31, 2017	Three Months Ended March 31, 2016	Nine Months Ended March 31, 2017	Nine Months Ended March 31, 2016
Revenue	901	4,142	1,846	15,669
Research & development	(13,928)	(12,015)	(42,975)	(35,618)
Manufacturing commercialization	(3,830)	(7,721)	(10,915)	(22,042)
Management and administration	(5,521)	(5,413)	(15,859)	(16,666)
Fair value remeasurement of contingent consideration	10,381	1,826	10,693	6,097
Other operating income and expenses	384	547	1,168	2,891
Finance costs	(1,264)	(2,489)	(2,915)	(6,939)
Loss before income tax	**(12,877)**	**(21,123)**	**(58,957)**	**(56,608)**
Income tax benefit/(expense)	3,093	4,190	9,324	4,190
Loss attributable to the owners of Mesoblast Limited	**(9,784)**	**(16,933)**	**(49,633)**	**(52,418)**
Losses per share from continuing operations attributable to the ordinary equity holders of the Group:	**Cents**	**Cents**	**Cents**	**Cents**
Basic - losses per share	(2.46)	(4.49)	(12.87)	(14.76)
Diluted - losses per share	(2.46)	(4.49)	(12.87)	(14.76)

Consolidated Statement of Comprehensive Income

(in U.S. dollars, in thousands)	Three Months Ended March 31, 2017	Three Months Ended March 31, 2016	Nine Months Ended March 31, 2017	Nine Months Ended March 31, 2016
(Loss)/profit for the year	**(9,784)**	**(16,933)**	**(49,633)**	**(52,418)**
Other comprehensive income				
Items that may be reclassified to profit and loss				
Changes in the fair value of available-for-sale financial Assets	(86)	36	(55)	(148)
Exchange differences on translation of foreign operations	942	1,527	368	(324)
Other comprehensive (loss)/income for the period, net of tax	**856**	**1,563**	**313**	**(472)**
Total comprehensive (loss)/income is attributable to the owners of Mesoblast Limited	**(8,928)**	**(15,370)**	**(49,320)**	**(52,890)**

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Consolidated Statement of Balance Sheet

(in U.S. dollars, in thousands)	As of March 31, 2017	As of June 30, 2016
Assets		
Current Assets		
Cash & cash equivalents	69,122	80,937
Trade & other receivables	6,522	4,054
Prepayments	10,828	3,832
Total Current Assets	**86,472**	**88,823**
Non-Current Assets		
Property, plant and equipment	2,153	3,063
Available-for-sale financial assets	1,911	1,966
Other non-current assets	1,911	2,343
Intangible assets	586,713	587,823
Total Non-Current Assets	**592,688**	**595,195**
Total Assets	**679,160**	**684,018**
Liabilities		
Current Liabilities		
Trade and other payables	26,169	27,155
Provisions	3,492	2,260
Total Current Liabilities	**29,661**	**29,415**
Non-Current Liabilities		
Deferred tax liability	53,369	62,693
Provisions	55,729	63,749
Total Non-Current Liabilities	**109,098**	**126,442**
Total Liabilities	**138,759**	**155,857**
Net Assets	**540,401**	**528,161**
Equity		
Capital	830,130	770,272
Reserves	27,991	25,976
(Accumulated losses)/retained earnings	(317,720)	(268,087)
Total Equity	**540,401**	**528,161**

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Consolidated Statement of Cash Flows

(in U.S. dollars, in thousands)	Nine months ended March 31, 2017	2016
Cash flows from operating activities		
Commercialization revenue received	1,012	—
Milestone revenue received	—	3,500
Payments to suppliers and employees (inclusive of goods and services tax)	(73,443)	(74,223)
Interest received	395	816
Net cash (outflows) in operating activities	**(72,036)**	**(69,907)**
Cash flows from investing activities		
Payments for investments	—	(805)
Payments for licenses	—	(200)
Investment in fixed assets	(315)	(680)
Rental deposits received	453	—
Net cash (outflows) in investing activities	**138**	**(1,685)**
Cash flows from financing activities		
Proceeds from issue of shares	61,784	68,549
Payments for share issue costs	(1,884)	(6,501)
Net cash (outflows) / inflows by financing activities	**59,900**	**62,048**
Net (decrease)/increase in cash and cash equivalents	(11,998)	(9,544)
Cash and cash equivalents at beginning of period	80,937	110,701
FX (losses)/gains on the translation of foreign bank accounts	183	(1,228)
Cash and cash equivalents at end of period	**69,122**	**99,929**

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