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the regenerative medicine company

MESOBLAST HEART FAILURE CELL THERAPY RECEIVES ORPHAN DRUG DESIGNATION FROM FDA FOR PREVENTION OF GASTROINTESTINAL BLEEDING IN PATIENTS WITH LEFT VENTRICULAR ASSIST DEVICES

New York, USA; and Melbourne, Australia; June 24, 2019: Mesoblast Limited (ASX:MSB; Nasdaq: MESO), global leader in cellular medicines for inflammatory diseases, announced today that the United States Food and Drug Administration (FDA) has granted the company's product candidate, rexlemestrocel-L, Orphan Drug Designation for prevention of post-implantation mucosal bleeding in end-stage chronic heart failure (CHF) patients who require a left ventricular assist device (LVAD).

Mesoblast Chief Executive Dr Silviu Itescu said: "We are very pleased that the FDA has granted our heart failure cell therapy product candidate, Revascor, Orphan Drug designation for use in patients with LVADs to prevent mucosal bleeding, including from the gastrointestinal (GI) tract. We look forward to our upcoming meeting with the FDA to discuss a potential approval pathway under the product's existing Regenerative Medicine Advanced Therapy (RMAT) designation for this life-threatening condition".

About Orphan Drug Designation

The FDA's Orphan Drug Designation Program provides orphan status to drugs and biologics which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases and disorders that affect fewer than 200,000 people in the United States. Orphan designation qualifies the sponsor of the drug for various development incentives, including eligibility for seven years of market exclusivity upon regulatory approval, exemption from FDA application fees, tax credits for qualified clinical trials, and other potential assistance in the drug development process.

About RMAT Designation

The FDA's RMAT Designation Program under the 21st Century Cures Act aims to expedite the development of regenerative medicine therapies intended for the treatment of serious diseases and life-threatening conditions. RMAT designation allows for multi-disciplinary, comprehensive interactions with the FDA to support efficient development of and potential accelerated approval pathway for cell therapy candidates. The RMAT designation also offers eligibility for priority review. Once the biologics license application (BLA) for a product is approved, the FDA can require various post-approval confirmatory commitments.

About End-Stage Heart Failure, LVADs, And Major Gastrointestinal Bleeding

In the United States, over 60,000 patients annually suffer from end-stage heart failure, and despite optimal medical therapy these patients have a one-year mortality exceeding 50%.[1] The only options to increase survival in these patients are the use of left ventricular assist devices (LVADs) or heart transplants.

Due to the decline in organ donations and limited availability of healthy donor hearts, the use of LVADs in end-stage heart failure patients is gaining momentum, with approximately 5,500 LVADs implanted annually in the United States.[2,3,4] However, high rates of re-hospitalization remain a major obstacle to greater LVAD use, with up to 40% of patients being re-hospitalized as a result of major GI bleeding.[5,6]

About Revascor

Mesoblast's investigational cell therapy Revascor consists of 150 million allogeneic mesenchymal precursor cells (MPCs) and is being developed for injection into heart muscle of patients with moderate-advanced or end-stage chronic heart failure.

Revascor For End-Stage Heart Failure Patients With LVADs

In a 30-patient placebo-controlled pilot trial in end-stage heart failure patients with LVADs, conducted by the United States National Institutes of Health (NIH), a single intra-myocardial dose of Revascor

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resulted in a 70% reduction in GI bleeding and associated hospitalizations. These results supported the RMAT designation granted in December 2017 by the FDA for use of Revascor in LVAD patients.

In a subsequent meeting in 2018, the FDA advised Mesoblast that the defined endpoint of reduction in major GI bleeding and rehospitalization is an appropriate clinically meaningful endpoint and could be the basis of an approved indication for use of Revascor given the life-threatening nature of the condition, and the RMAT designation under which Revascor is being regulated.

In November 2018, NIH investigators presented results of a 159-patient randomized placebo-controlled Phase 2b clinical trial at the American Heart Association Scientific Sessions, showing that a single intra-myocardial dose of Revascor resulted in a 76% reduction in major GI bleeding events and 65% reduction in related hospitalizations in end-stage heart failure patients implanted with an LVAD.

Revascor In Patients With Moderate To Advanced Heart Failure

In addition to evaluation in LVAD patients, Revascor is also being evaluated in a placebo-controlled Phase 3 trial which has enrolled 566 patients with New York Heart Association Class II/III moderate to advanced heart failure across multiple sites in North America. The objectives of this Phase 3 events-driven trial are to evaluate the ability of Revascor to reduce the primary endpoint of recurrent heart failure-related major adverse cardiac events (HF-MACE) in patients with left ventricular dysfunction, as well as delay or prevent disease progression to terminal cardiac events, defined as death, left ventricular assist device (LVAD) implantation, or cardiac transplant. The trial has accrued approximately 85% of its required target of primary endpoint events. In April 2017, the Phase 3 trial was successful in a pre-specified futility analysis of the primary efficacy endpoint in the first 270 patients.

About Mesoblast

Mesoblast Limited (ASX: MSB; Nasdaq: MESO) is a world leader in developing allogeneic (off-the-shelf) cellular medicines. The Company has leveraged its proprietary technology platform to establish a broad portfolio of late-stage product candidates with three product candidates in Phase 3 trials – acute graft versus host disease, chronic heart failure and chronic low back pain due to degenerative disc disease. Through a proprietary process, Mesoblast selects rare mesenchymal lineage precursor and stem cells from the bone marrow of healthy adults and creates master cell banks, which can be industrially expanded to produce thousands of doses from each donor without the need for tissue matching. Mesoblast has facilities in Melbourne, New York, Singapore and Texas and is listed on the Australian Securities Exchange (MSB) and on the Nasdaq (MESO). www.mesoblast.com

References

[1] Gustafsson F, Rogers JG. Left ventricular assist device therapy in advanced heart failure: patient selection and outcomes. European Journal of Heart Failure 2017;19:595-602.

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[3]Agency for Healthcare Research and Quality – Healthcare Cost and Utilization Project – Claims Analysis ICD- 37.6.

[4]Data on file.

[5]Chatterjee A, Feldmann C, Hanke JS (2018) The momentum of HeartMate 3: a novel active magnetically levitated centrifugal left ventricular assist device (LVAD). J Thorac Dis 10 (Suppl 15): S1790-S1793.

[6]Mehra, MR Salerno C, Cleveland JC (2018) Health care resources use and cost implications in the MOMENTUM 3 long-term outcome study: a randomized controlled trial of a magnetically levitated cardiac pump in advanced heart failure.

Forward-Looking Statements

This announcement 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

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