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Stem Cell Product HLCM051 for ARDS Orphan Regenerative Medicine Designation

HEALIOS K.K. ("Healios") is currently conducting a clinical trial using stem cell product HLCM051 for the patients of ischemic stroke and Acute Respiratory Distress Syndrome (ARDS) in Japan.

Today it announces that the Pharmaceutical Affairs and Food Sanitation Council (PAFSC) acknowledged to designate HLCM051 as an orphan regenerative medicine for ARDS. It will be officially designated by the Ministry of Health, Labour and Welfare in the next few weeks.

The orphan regenerative medicine designation system is based on the Article 77-2 of the Act on Securing Quality, Efficacy and Safety of Pharmaceuticals, Medical Devices, Regenerative and Cellular Therapy Products, Gene Therapy Products, and Cosmetics if they are intended for use in less than 50,000 patients in Japan and for which there is a high medical need. Orphan designation is determined by the Ministry of Health, Labour and Welfare based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council (PAFSC).

Receiving orphan regenerative medicine designation provides support in various ways including subsidies to reduce the financial burden of product development, priority consultation and review, preferential tax treatment and extension of re-examination period.

ARDS is a general term for the symptoms of acute respiratory failure suddenly occurring in seriously ill patients. The major causes are severe pneumonia, septicemia, trauma etc. Inflammatory cells are activated in response to these diseases or injuries, causing damage to the tissue of the lungs. As a result, water accumulates in the lungs, leading to acute respiratory failure. According to the ARDS treatment guideline 2016, the mortality rate is approximately 30 to 58%. Artificial respiration using an endotracheal tube or mask is used to treat respiratory failure in an intensive care unit. However, it is known that prolonged use of a ventilator worsens a patient's prognosis. There is demand for a new treatment for ARDS that will lead to improvement in patients' symptoms and prognosis.

Healios commenced its development of somatic stem cell products by signing an exclusive licensing agreement with the United States based Athersys, Inc. ("Athersys") in January 2016, whereby Healios acquired rights to develop and distribute Athersys' proprietary stem cell product MultiStem® (HLCM051) to treat ischemic stroke in Japan. In June 2018 Healios and Athersys further expanded their collaboration broadly, and as part of this expansion Healios acquired the development and distribution licenses to use MultiStem (HLCM051) to treat ARDS in Japan.

Athersys conducted the Phase 1/2 exploratory clinical trial for ARDS patients in the United States and the United Kingdom. The analysis of the double blinded, placebo-controlled and randomized

phase (Phase 2a portion) shows positive results:

-Lower mortality in the MultiStem treatment group than in the placebo group through 28 days after the administration
-Higher ventilator-free days in the MultiStem treatment group than in the placebo group in 28 days after the administration

Healios is currently conducting a Phase 2 clinical trial (ONE-BRIDGE study) for pneumonia induced ARDS patients using HLCM051 at over 20 clinical sites in Japan. This trial is being conducted as an open label, standard therapy controlled study and is seeking to enroll 30 patients. Its primary endpoint is the number of days out of 28 in which a ventilator was not used for the patient (Ventilator-Free Days).

Healios will promptly announce if any matter occurs that must be disclosed.