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Full Enrolment of TREASURE Study for Patients with Ischemic Stroke

HEALIOS K.K. (“Healios”) announces that it has fully enrolled the planned patients in its TREASURE study for ischemic stroke, the clinical trial to test the efficacy and safety of HLCM051*¹ in Japan. (Lead Investigator: Kiyohiro Houkin, MD, President, Hokkaido University)

The TREASURE study is a placebo-controlled, double-blind, phase 2/3 trial designed to confirm the efficacy and safety of HLCM051 in treating patients with ischemic stroke. Patients were randomized 1:1 to receive either a single intravenous infusion of HLCM051 or placebo within 18–36 hours of the onset of stroke. The primary efficacy outcome is the proportion of subjects achieving an Excellent Outcome*² at day 90. The study was planned for 220 patients and Healios determined that enrolment was completed after a period of follow up to ensure any dropouts would not have an impact on data calculations.

Going forward, we plan to analyze and evaluate the data after an ongoing follow-up period.

This action has no impact on our company’s consolidated financial results for the current fiscal year. We will promptly make the necessary announcements if any matter requiring disclosure arises in the future.

*¹ HLCM051

HLCM051 is a somatic stem cell regenerative medicine product. Healios added this product to its pipeline 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[®] to treat ischemic stroke in Japan. Further, in June 2018, Healios and Athersys expanded their collaboration broadly, and as part of this expansion, Healios acquired the development and distribution licenses to use MultiStem to treat Acute Respiratory Distress Syndrome (ARDS) in Japan.

*² Excellent Outcome

Functional and neurological deficit and recovery following ischemic stroke are evaluated using three standard methods: the modified Rankin scale (mRS), the NIH stroke scale (NIHSS), and the Barthel Index (BI). “Excellent Outcome” is defined as achieving scores ≤ 1 on the mRS and on the NIHSS and a score ≥ 95 on the BI.

mRS

The mRS measures the degree of disability or dependence in the activities of daily living of people who have had a stroke or have a neurological disability due to other reasons. It is used

to categorize the level of functional independence with reference to pre-stroke activities. The scale includes scores from 0 to 6, ranging from perfect health without symptoms of disability (i.e., a score of 0) to death (a score of 6). A lower score indicates a lower degree of disability.

NIHSS

The NIHSS is a systematic assessment tool that provides a quantitative measure of stroke-related neurologic deficit in the following areas: level of consciousness, facial paralysis, visual acuity and function, arm and leg motor function, limb coordination, language and speech, sensory loss, and other parameters. A higher score on the NIHSS indicates a higher degree of neurological impairment in a stroke patient. The score for each function ranges from 0 to 4, with 0 indicating normal function (i.e., no deficit) and 4 indicating complete impairment (Note that some functional assessments use a scale of 0–2, or 0–3). The total NIHSS score of the patient is calculated by adding the score for each element on the scale, based on the individual assessments; 42 is the highest possible score, which reflects the maximum disability of the patient in each category.

BI

The BI is a 100-point scale that is used to assess the ability of the patient to independently perform activities of daily living and to evaluate a range of different functions. These include the ability of the patient to walk, dress, feed, bathe, climb stairs, use a toilet, self-groom, and certain other metrics. The patient is evaluated for each activity to assess for independence, partial dependence, or complete dependence, and then, a score between 0 and 10 is assigned (10 points = independence, 5 points = partially dependent, and 0 points = completely dependent). The BI score ranges from 0 to 100; a score of 100 indicates no dependence on any activity, and a lower score indicates a greater need for assistance.

(Source) Prepared by Healios on the basis of materials provided by The Japan Stroke Society.