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The Status of a Clinical Trial of Adult Stem Cell Regenerative Medicine HLCM051 in Patients with Ischemic Stroke in Japan (TREASURE study)

HEALIOS K.K. (“Healios”) today announces that the patient enrollment of the clinical trial of adult stem cell regenerative medicine HLCM051 (MultiStem®) ^{*1} in patients with acute ischemic stroke in Japan, named the TREASURE study, ^{*2} has been temporarily suspended. Placebo was found to be out-of-specification and is being replaced, which is expected to take approximately one month. The placebo material, which had an incorrect concentration of a media component, had not been administered to subjects and did not present a safety issue.

Athersys Inc. (“Athersys”), the supplier of the investigational product, identified the issue and promptly notified Healios, which informed the open sites of the temporary suspension of patient enrollment on the same day, after confirming that none of HLCM051 nor placebo product had been administered. Healios received the report from Athersys that incorrect placebo manufacturing instructions were utilized by its contract manufacturer. These instructions have been corrected, and replacement placebo material is being manufactured and is expected to be ready for release in about one month following testing and inspection. The placebo will be distributed to the clinical trial sites in accordance with our quality control process in Japan, and then the patient enrollment will be restarted after confirming that the placebo meets required specifications.

The new placebo will be distributed to the sites which already completed the preparations for patient enrollment. In addition, there are more than 20 medical institutions where Institutional Review Boards have granted approval to conduct the trial, and the number of the sites to initiate enrollment will be increased sequentially. Healios plans to conduct the trial at over 30 sites throughout Japan.

In order to prevent the recurrence of defect or other manufacturing issues, Healios will work closely with Athersys to ensure the thorough management of manufacturing and quality control and oversight of Athersys’ contract manufacturer, and to facilitate the TREASURE study.

If matters to be disclosed arise in the future regarding the effect on fiscal year 2017 financial performance, Healios will make an announcement without delay.

*1: HLCM051

Healios holds a development pipeline for treating ischemic stroke using the stem cell product HLCM051 (MultiStem) in Japan. Ischemic stroke is a condition in which a blockade in blood vessels in the brain precludes the delivery of oxygen and nutrients beyond the blockade, causing necrosis of nerve cells over time. Currently, ischemic stroke is treated with t-PA (a thrombolytic agent) that dissolves clots lodged in a blood vessel in the brain, mechanical reperfusion therapy, or other treatment options; however, there is a need for a new drug that can be used during a longer period of time after the onset of ischemic stroke and that also provides the potential for improved outcomes for patients.

Healios has introduced HLCM051 by signing an exclusive licensing agreement with a biopharmaceutical company, Athersys, Inc., in the United States in January 2016 on the domestic development and distribution of regenerative medicine products in Japan for ischemic stroke using the Athersys' proprietary stem cell product, MultiStem.

*2: TREASURE study

TREASURE is an abbreviation for "Treatment Evaluation of Acute Stroke Using Regenerative Cell Elements".