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Announcement of Changes in Joint Development Framework in Japan for the Treatment using iPSC-derived RPE cells

HEALIOS K.K. ("Healios") is currently developing in both the iPSC regenerative medicine and somatic stem cell regenerative medicine fields. In iPSC regenerative medicine, Healios and Sumitomo Dainippon Pharma Co., Ltd. ("Sumitomo Dainippon Pharma"), its joint development partner in Japan for the treatment using iPSC derived retinal pigment epithelium ("RPE") cells, are currently preparing for a clinical trial. In somatic stem cell regenerative medicine, Healios is currently conducting two clinical trials for ischemic stroke and acute respiratory distress syndrome (ARDS).

Under these circumstances, to deliver new therapies to patients as quickly as possible and to streamline our sales and manufacturing capabilities as a pharmaceutical company, Healios has decided to focus its near term clinical development resources on its two ongoing trials in the field of somatic regenerative medicine and revise its joint development agreement in relation to iPSC-derived RPE cell therapies with Sumitomo Dainippon Pharma. Today Healios and Sumitomo Dainippon Pharma announce that an agreement has been reached regarding these changes.

<Summary of Changes and Reasoning>
In February 2013, Healios signed a patent licensing agreement with iPS Academia Japan, Inc., providing it with rights to the fundamental technology required to create iPS cells for the purposes of producing and commercializing on worldwide basis products containing RPE cells as an active component. Likewise, in March 2013, Healios signed a patent licensing agreement with RIKEN providing it with exclusive worldwide rights to commercialize regenerative medicine products containing RPE cells derived from pluripotent stem cells, including iPS cells, as an active component.

Furthermore, in December 2013, to conduct development based on these patent licenses of therapies using RPE cells derived from iPS cells, Healios agreed to joint development with Sumitomo Dainippon Pharma, limited to Japan, signing (1) a License Agreement regarding the licensing of the IP rights owned by Healios, (2) a Joint Development Agreement establishing the division of roles and allocation of costs in connection with joint development, and (3) a Joint Venture Agreement establishing a joint venture to undertake outsourced manufacturing and sales promotion of such products, as well as stipulating the outsourcing fees to be paid to the joint venture.

Under this joint development framework, Healios conducted R&D for commercialization using the technologies and know-how to induce differentiation of RPE cells from iPS cells and transplant them based on methods devised by RIKEN in combination with Healios' proprietary technologies and know-how concerning mass production and consistent quality, etc. However, as the practical application of the new therapy using the iPSC technology will require a long-term development approach involving various stakeholders in addition to Healios and Sumitomo Dainippon Pharma, having considered the optimal allocation of resources, we determined that it was appropriate to alter the current joint development framework.

The major changes are as follows:
1) Changes in Development Roles
Under the Joint Development Agreement, it was agreed that Healios would lead clinical trials and apply for manufacturing and sales approval, etc., but we have determined that it will be more efficient for Sumitomo Dainippon Pharma to lead clinical trials going forward. Accordingly, we will transition to a framework in which both companies will apply for sales and manufacturing approval based on the results of the clinical trial.

2) Changes in the License Agreement
Under the License Agreement, it was agreed that Healios would receive milestone payments from Sumitomo Dainippon Pharma in accordance with the progress of development of ¥1.6 billion in total (of which ¥0.7 billion has been received), but with these changes to the joint development framework, the total amount of milestone payments Healios will receive from Sumitomo Dainippon Pharma has been revised to ¥1.0 billion. Details such as the timing and amount of payments received have not been made public.

Regarding development costs, it was originally agreed that Sumitomo Dainippon Pharma would bear a maximum of ¥5.2 billion based on the assumption that Healios would lead development, but in accordance with the transition to a new framework in which Sumitomo Dainippon Pharma will lead the clinical trial as well as future revisions in relation to sales and manufacturing activities, we have made adjustments to provide greater flexibility in cost allocation. As the particulars involve the development strategies of the two companies, they will be kept confidential.

The patent license Healios granted Sumitomo Dainippon Pharma regarding RPE cell products now includes a non-exclusive overseas license in addition to the exclusive license in Japan.

3) Changes to the Role of the Joint Venture
Based on the Joint Venture Agreement, Healios and Sumitomo Dainippon Pharma each contributed 50% of the capital to establish Sighregen Co. Ltd., to be exclusively entrusted with manufacturing and sales promotion for RPE cell products. However, in the event that both Healios and Sumitomo Dainippon Pharma apply for sales and manufacturing approval in the future, only the manufacturing of cell products will be outsourced to Sighregen. Currently, Sighregen is making steady progress in building manufacturing capability with a team of about 20 staff.

“These changes to the joint development framework are supportive of our efforts in the ophthalmology field. They are a step toward realizing our long-term development strategy to deliver innovative therapies to patients around the world.”, commented Hardy TS Kagimoto, MD, Chairman and CEO of Healios. “To achieve our founding ambition of eliminating blindness, we at Healios continue to evaluate several cutting-edge technologies and partner candidates. Additionally, we are also making progress in creating next generation iPS cells which will require the use of minimal or no immunosuppressants via gene editing technologies. In the short term, we will focus our resources on ongoing trials, while in the long term we aim to consolidate these core technologies to realize our original goal,” concluded Kagimoto.

The matters that may affect results of the fiscal year of 2019 will be disclosed promptly if necessary.