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HEALIOS K.K. and Universal Cells Inc. enter Collaborative Research Agreement with Exclusive Option to Produce Universal Donor iPSCs

HEALIOS K.K. (“Healios”) (Head Office: Tokyo, Japan; President, Representative Director and CEO: Hardy TS Kagimoto) decided to enter a collaborative research and exclusive option agreement with Universal Cells, Inc. (“Universal Cells”) (Head Office: Washington, USA; CEO and co-founder: Claudia Mitchell Ph.D.). regarding application of their genome editing technology to process iPS cells. With this technology, expression of HLA (cell surface molecules that identify self and non-self) can be controlled, and pluripotent cells can be manufactured to avoid transplant rejection.

Universal Cells is a private biotechnology company, and is developing a genome editing technology for cell and gene therapies. Genome editing is a technology to modify nucleotide sequence at a specific site on genome, and is now attracting attention worldwide.

Universal Cells’ technology is based on discoveries by Dr. David Russell, the co-founder and Chief Scientific Officer of Universal Cells, and a world-renowned leader in gene editing. Universal Cells has licensed patents on recombinant adeno-associated viral (rAAV)-mediated gene editing.

Universal Cells is developing and commercializing Universal Donor Stem Cells by using their gene editing technology to remove and replace the genes that are required for transplant rejection. When the HLA molecules are modified, patients can receive cell transplantation with less possibility of rejection. A "suicide gene" is also incorporate in the cells as a safety mechanism. Even if the regenerative medicine manufactured from Universal Donor Stem Cells circumvents immune response of the recipient and shows excessive proliferation, this suicide gene can be activated by administration of a drug, and the regenerative medicine can be excluded before triggering serious adverse effect.

Healios decided to execute a collaborative research agreement to investigate whether Universal Donor Stem Cells technology developed by Universal Cells can be applied to iPSC regenerative medicine. When iPSC cells with reduced transplant rejection are developed, the door will open to the next stage of regenerative medicines with less transplant rejection based on cells differentiated from the iPSC cells. For this purpose, the exclusive option is for the treatment of dry/wet AMD, an intractable eye disease, and for use with Healios' proprietary Organ Bud technology in liver and kidney clinical fields. The cell line developed under this agreement will be proprietary to Healios.

Healios considers development of iPSC regenerative medicine as its core business. iPSC regenerative medicine aims at recovery of function by replacing dysfunctional cells due to disease or aging, with transplanted healthy cells manufactured by differentiation of iPSC cells.

Addressing the underlying cause of transplant rejection in iPSCs will allow for commercial-scale manufacturing and quality control so iPSC regenerative medicine can be used to treat a variety of diseases safely and predictably. However, risk of transplant rejection by immune system is yet to be excluded, in some tissues surrounding the transplanted cells. This technology therefore greatly enhances clinical application of iPSC cell technology.

<Implication for our budget>

The total expenses related to the cost of the collaborative research and of the exclusive option rights associated with this agreement will be 260 million yen, and will be earmarked for selling and general administrative expenses in financial settlements from FY2016 onward.

(Forecast of financial results of this term)

(Figures are rounded down to the nearest million yen)

	Net sales	Operating income	Ordinary income	Net income
F Y 2 0 1 6	—	—	—	—
F Y 2 0 1 5	98	Δ1,060	Δ987	Δ958

We consider that proper and reasonable forecast is difficult because we have several pending plans that may influence on our financial condition such as business collaboration or acquisition of new seeds. We do not disclose our forecast for whole financial year. Instead, we will disclose financial settlement and summary of our business each quarter in a timely manner.

<About Universal Cells>

Universal Cells is a private biotechnology company developing proprietary nuclease-free genome editing technologies that allow efficient and accurate editing of any gene without cutting the genome. The company is using this platform to create cell therapies that are rejection-free, off-the-shelf products that can be administered to any recipient without the need for immunosuppressive drugs. For more information: <http://www.universalcells.com>