

CiRA Foundation has been entrusted by Megakaryon to manufacture iPS cell-derived platelets for a clinical trial

Kyoto, Japan, 22 June 2020 – The CiRA Foundation (CiRA_F) today has signed an agreement with Megakaryon Corporation (“Megakaryon”; located in Shimogyo-ku, Kyoto) to entrust CiRA_F with the manufacturing of iPS cell-derived platelets for use in a clinical trial that Megakaryon will conduct. The iPS cells used for the derivation are provided by CiRA_F as raw material. The clinical trial will be conducted in 2021 to treat thrombocytopenic patients. Products used for the clinical trial and also pre-clinical safety assessments will be manufactured through this agreement.

Platelets are anucleate blood cells that function to prevent bleeding by aggregating at the wound when blood vessels are damaged. Platelet transfusion is the standard therapy for patients with low platelet count, and the platelets used for these transfusions come from donors. However, in rare cases, donor platelets cannot be used due to immune reactions. Moreover, from the viewpoint of a stable supply of platelet products, donor-independent systems will improve the quality of medical care.

Professor Koji Eto at the Center for iPS Cell Research and Application (CiRA), Kyoto University, and others have been researching the production of platelets from iPS cells. Since 2019, his team, with Professor Akifumi Takaori and other members of Kyoto University Hospital, have been conducting clinical research to confirm the safety of platelets produced from the autologous iPS cells of patients with aplastic anemia complicated by platelet transfusion refractory disease.

Megakaryon is a venture company working closely with such academic researchers as Prof. Eto to develop clinical-grade platelet products.

CiRA_F collaborates with academia and industry to provide iPS cell-derived regenerative medicines to patients as soon as possible.

About the CiRA Foundation

The CiRA Foundation was recognized as a public interest incorporated foundation in April 2020. Its principle purpose is to deliver high-quality iPS cells at low cost. The Foundation manages the iPS Cell Stock for Regenerative Medicine Project, which was started by the iPS Cell Research and Application (CiRA), Kyoto University, in 2013. The aim of this project is to prepare multiple iPS cell lines manufactured from healthy donors homozygous for human leukocyte antigens (HLA). These lines will expand the number

of people who can receive related therapies with minimal immune reactions and are provided to academia and industries. The Foundation contributes to the commercialization of regenerative medicine by providing services including the manufacturing of iPS cell-derived products, quality assessment, storage, and publication of SOPs for manufacturing.

About Megakaryon Corporation

Megakaryon Corporation was established in 2011 with the aim of utilizing the technologies developed by Kyoto University and the University of Tokyo for producing platelets from human iPS cells for clinical application. By developing large-scale manufacturing of human iPS-derived platelets with no risk of infection, we aim to supply platelets to medical facilities around the world both in developed countries where declining birthrate and increased ageing population cause a concern due to decreased number of blood donations, and in developing countries where there is already a shortage of donated platelets.

Since 2013, Megakaryon Corporation is supported by Innovation Network Corporation of Japan (currently INCJ, Ltd.). In 2015, Megakaryon Corporation was certified by the Japanese Prime Minister as the first application of a specific core project under the National Strategic Special Zones Law for its initiative that contributes to strengthening Japan's competitiveness in the global medical field, as well as in research and development of innovative regenerative medicine, and the promotion of its commercialization.

In February 2020, our efforts for promoting open innovation was recognized by the Cabinet Office of Japan at the 2nd Japan Open Innovation Award and Megakaryon along with its partner companies have won the Minister of State Science and Technology Policy Award. Website: <http://www.megakaryon.com/>

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