

Press Release



Platelets derived using the CiRA Foundation iPS Cell Stock enter an industry-led clinical trial

Kyoto, Japan, May 18, 2021

1. Overview

The CiRA Foundation (CiRA_F; Kyoto, Japan) is participating in a corporate clinical trial plan to investigate HLA homozygous iPS cell-derived platelets (Development Code: MEG-002) in collaboration with Megakaryon Corporation, Kyoto University Hospital and the Center for iPS Cell Research and Application (CiRA), Kyoto University. (For a history of the relationship, see [CiRA Foundation has been entrusted by Megakaryon to manufacture iPS cell-derived platelets for a clinical trial](#))

Evaluation by the Pharmaceuticals and Medical Devices Agency (PMDA) of the clinical trial plan submitted by Megakaryon has been completed, and preparations for the clinical trial will be carried out at Kyoto University Hospital and several other medical institutions.

CiRA_F will be responsible for the manufacturing of human iPS cell-derived platelets, their non-clinical safety evaluation and their quality assessment.

2. Background

A group led by Professor Akifumi Takaori, Department of Hematology, Kyoto University Hospital, and Professor Koji Eto, CiRA, has been conducting clinical research using human iPS cell-derived platelet products (*1) to verify their safety (Reference: [CiRA News, 25 March 2020](#)). This clinical study investigates the products as an autologous transplant, but in order to establish a manufacturing method and launch products for wide clinical use, it is necessary to conduct a clinical trial with an allogeneic transplant, which would allow a single product to be transplanted into a large number of patients.

3. Summary of the clinical trial

We plan to confirm the safety and estimate the efficacy of MEG-002 in thrombocytopenia (*2) patients. MEG-002 is a platelet product produced from iPS cells with the most frequently expressed HLA type in the Japanese population and provided by CiRA. It was developed using technology invented by Professor Koji Eto and others at CiRA.

CiRA_F will be responsible for manufacturing MEG-002. The clinical trial is planned to be conducted at Kyoto University Hospital and other medical institutions.

4. Links for related information

- Megakaryon News Page
http://www.megakaryon.com/en/dataroom/pdf/mega_release_20210426_en.pdf
- Kyoto University Hospital News Page (written in Japanese)
<https://www.kuhp.kyoto-u.ac.jp/info/20210426.html>

5. Notes

1. Human iPS cell-derived platelets

A cellular product that resembles natural platelets. They are manufactured by maturing a master cell bank of cryopreserved immortalized megakaryocyte progenitor cells made from human iPS cells.

2. Thrombocytopenia

Thrombocytopenia is a condition in which the number of platelets in the blood is dangerously low such that there is a high risk of severe bleeding. Common treatment includes the transfusion platelet preparations.