Megakaryon Corporation
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News Release

June 2, 2022

Megakaryon Corporation initiates first-in-human clinical trial of allogenic human iPSC-derived HLA homozygous platelets (MEG-002).

Kyoto, Japan –Megakaryon Corporation (hereinafter, Megakaryon) today announces its clinical trial of allogenic human iPSC-derived HLA homozygous platelets (Development Code: MEG-002) in collaboration with Kyoto University Hospital, Center for iPS Cell Research and Application, Kyoto University (CiRA) and CiRA Foundation (CiRA_F).
Initial administration to the first subject was successful and with no reported adverse events.

Background

The Department of Hematology, Kyoto University Hospital (Professor Akifumi Takaori-Kondo, M.D., Ph.D.) and CiRA (Professor Koji Eto M.D., Ph.D.) have conducted clinical research for the transfer of autologous iPSC-derived platelets to a thrombocytopenia patient to verify the safety of iPSC-derived platelet preparations. The clinical trial announced today is for a study using allogenic human iPSC-derived HLA homozygous platelets developed by Megakaryon which will enable industrialization of iPSC-derived platelets and their subsequent availability to a large number of patients.

March 26, 2021	Submitted clinical trial notification to Japan's Pharmaceutical and Medical Devices Agency (PMDA)
April 26, 2021	Completion of 30-day review by PMDA
June 7, 2021	Submitted application to IRB of Kyoto University Hospital
June 21, 2021	Approval from IRB of Kyoto University Hospital
August 19, 2021	Clinical trial information published on Japan Registry of Clinical Trials (jRCT)
April 2022	Administration to the first subject

About the first administration of investigational product

Hospital:	Kyoto University Hospital
Dosing date:	April 2022
Coordinating investigator:	Akifumi Takaori-Kondo, M.D., Ph.D., Professor of Hematology and Oncology
Principal investigator:	Junya Kanda, M.D., Ph.D., Associate Professor of Hematology and Oncology
Clinical trial products:	Allogenic human iPSC-derived HLA homozygous platelets (MEG-002)
Dose:	3 units ($0.6x10^{\wedge 11}$ platelets)
Manufacturing location:	Facility for iPS Cell Therapy (FiT) of CiRA_F

Summary of the clinical trial

During the clinical trial, Megakaryon will assess the safety and estimate the efficacy of MEG-002 for patients with thrombocytopenia. MEG-002 is prepared from an iPSC provided by CiRA (currently CiRA_F) and consists of platelets with the most common HLA haplotype among the Japanese population. The technology for producing platelets from iPSCs invented by Professor Koji Eto is used for the development of MEG-002. The clinical trial will be conducted at multiple medical institutions, including the Department of Hematology and Oncology, Kyoto University Hospital using a product manufactured by CiRA_F.

Title of the clinical trial

Exploratory clinical study on the tolerability, safety and efficacy of iPS cell-derived platelets (MEG-002) in patients with thrombocytopenia

Purpose of the clinical trial

Tolerability, safety and efficacy of a single dose of MEG-002 in thrombocytopenia patients.

Design of the clinical trial

Phase 1/2 study:	Open-label, uncontrolled study / single-dose study
Dose (1):	3 units ($0.6x10^{\wedge 11}$ platelets)
Dose (2):	10 units ($2x10^{\wedge 11}$ platelets)

Primary endpoint

Safety: Incidence of adverse events and side effects
Efficacy: Corrected count increment (CCI)

Period of the clinical trial

July 2021 – August 2022 (Planned to be extended)

Inclusion Criteria

1) Thrombocytopenic patients under stable medical conditions who have been diagnosed with any of the following diseases;
   - Aplastic anemia
   - Myelodysplastic syndrome
   - Leukemia (excluding induction therapy, early cytopenia after consolidation therapy and acute promyelocytic leukemia)
   - Solid cancer after chemotherapy (prolonged thrombocytopenia)
2) Patients whose platelet count is 20,000 microL or less, or expected to be 20,000 microL or less without blood transfusion, or patients who are at a risk of bleeding at 30,000 microL or less
3) Patients who can wash out for 3 days or more after blood donation platelet transfusion before administration of MEG-002
4) Aged 18 years or older at the time of the informed consent
5) Gender: Not specified
6) Patients with written informed consent (and a surrogate in the case of under 20)

For more information at Japan Registry of Clinical Trials (jRCT).
https://jrct.niph.go.jp/en-latest-detail/jRCT2053210068

Megakaryon Corporation

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Megakaryon Corporation was established in 2011 with the aim of utilizing technologies invented by Professor Koji Eto and others for producing platelets from human iPSCs for clinical application. By developing large-scale manufacturing of human iPSC-derived platelets with no risk of infection, we aim to supply platelets to medical facilities around the world.

Media Contact:

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Notes:

Human iPSC-derived Platelets

Human iPSC-derived platelets are produced by maturing megakaryocytes cultured from a master cell bank (MCB). MCB is made from cryopreservable immortalized megakaryocyte precursor cells obtained by introducing three genes into hematopoietic precursor cells differentiated from human iPSCs.

Platelets, Thrombocytopenia

Platelets, also known as thrombocytes, are major blood components that play a crucial role in hemostasis. Upon endothelial damage, platelets are activated leading to adhesion and aggregation at the wound site, thereby stopping bleeding.

Thrombocytopenia is a condition in which the number of platelets in the blood is low. If the platelet count drops below a certain level or if there is a high risk of bleeding, treatment with blood transfusion platelet preparations is given.

Corrected Count Increment (CCI)

A measure of the expected increase in platelets following a platelet transfusion. The “count increment” refers to the increase in platelets following a transfusion. The “correction” is based on the patient's size and the number of platelets transfused.