

## MEGAKARYON CORPORATION

Aiming for mass production of the first global iPSC (induced Pluripotent Stem Cell)-derived platelet product for allogeneic use

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<https://www.megakaryon.com/en/>

Founded in 2011  
Founder: MIWA Genjiro  
No. of employees: 17  
Type of Ownership: Public  
Stock Exchange: TSE/TYO Prime 6869  
Sysmex

**August 2024:** By becoming a subsidiary of Sysmex in December 2023, a world leader of in vitro diagnostics, Megakaryon accelerates the development of the iPSC-derived allogeneic platelet product and a new project involving an iMDF® (iPS-Megakaryocyte Derived Factors) growth factor cocktail.



Venture Valuation (VV) interviewed Dr. AKAMATSU Kenichi, CEO, and Mr. AYABE Tsuyoshi, COO & CFO.

**VV:** Our first interview in 2018 (see Interview Article No.2018.34<sup>1</sup>) introduced successful transfusions of iPSC-derived autologous platelets. This time, you proved the efficacy and safety of iPSC-derived allogeneic platelet products primarily targeting Japanese patients.

**Akamatsu & Ayabe:** We presented the results of the first transfusion to a thrombocytopenic patient in June 2023 at the ISTH (International Society on Thrombosis and Haemostasis) in Canada.

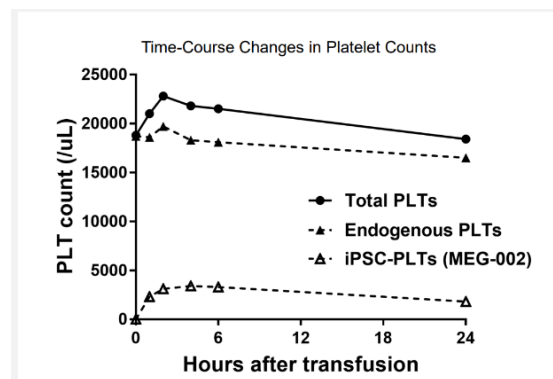
We applied an iPSC line expressing the highest human leukocyte antigen (HLA) among a Japanese population (HLA plays an important role in each person's immune response to foreign substances).

<sup>1</sup> <https://venturevaluation.com/2018/03/interviews-with-leading-life-sciences-companies-megakaryon-corporation/>

The graph shows the increase in platelet counts after the transfusion to the patient.

Analysis with flow cytometry confirmed normal circulation of iPSC-derived platelets.

Neither adverse events nor abnormalities in vital signs or electrocardiograms were observed.



Now that we have confirmed that iPSC platelets function in the body, we are working to develop HLA-null platelets that suppress HLA expression. We have already established a cell line and are preparing to conduct clinical trials in 2028.

**VV:** Among your numerous patents, the method for producing megakaryocytes that have an increased capacity to create platelets (WO/2022/265117) is a significant invention toward mass production and cost reduction of platelet transfusion.

**Akamatsu & Ayabe:** At present, the manufacturing cost of iPSC-derived platelets is expected to be higher than that of platelets from blood donation, and we recognize that reducing the manufacturing cost is very important. This patent should be essential to future cost reduction.

While we are working on reducing the cost, we would like to emphasize the fundamental advantages of the iPSC-derived platelets. First, by systematically and stably producing platelets from cell lines, we can overcome the problem of unstable platelet supply. Along with long-term supply concerns due to demographic trends, we have also experienced temporary shortages due to natural disasters and infectious disease pandemics.

Second, being produced in a sterile environment under strict manufacturing control, they are totally free from any pathogenic infection and bacterial or viral contamination: the necessary testing of donated blood is not required.

Thirdly, while the shelf life of platelets from donated blood is limited to a few days, iPSC-derived platelets can be preserved for two weeks. Their availability not only reduces the risk of shortage but also avoids the waste of valuable donated blood supplies that are not used soon enough.

Finally, iPSC-derived platelets are completely safe from tumorigenicity by using gamma-ray irradiation for sterilization in the manufacturing process.

In addition to the fundamental advantages, HLA-null platelets can be administered to people with any HLA type, including platelet transfusion refractory patients.

**VV:** Your business development strategy is to primarily focus on some specific disease areas in Japan.

**Akamatsu & Ayabe:** We are planning to treat patients with chronic hematopoietic deficiency such as aplastic anemia and myelodysplastic syndromes, or patients with thrombocytopenia due to leukemia including receiving chemotherapy.

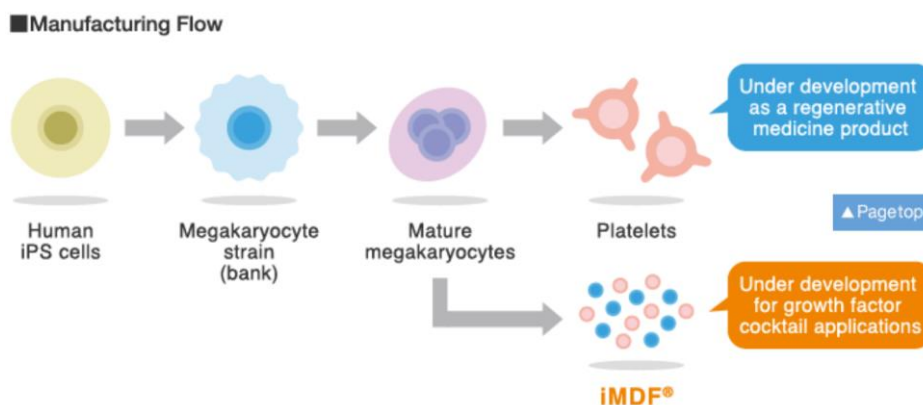
We are going to discuss in detail with the PMDA (Pharmaceutical and Medical Devices Agency) to apply for the priority review process for approval.

Our first objective is to enter Japan’s platelets market worth around 70 billion Yen (roughly USD 466 million at the time of writing) then expand overseas.

**VV:** In addition to developing the iPSC-derived platelet product for allogeneic use, the iMDF® growth factor cocktail project is in progress.

**Akamatsu & Ayabe:** The iMDF® is a growth factor cocktail like platelet-rich plasma (PRP), which is purified from mature megakaryocytes. PRP is considered to promote healing. There has been a high demand for it in diverse fields such as sports medicine, orthopedics, dermatology, cosmetics, and so on.

We are actively looking for suggestions for partnership or collaboration.



**VV Comments after the interview:**

Among competitors in the world, Megakaryon is leading in this challenging field with its proprietary technology enabling mass production of safe and quality iPSC-derived allogeneic platelets.

“If current forecasts are accurate, 2026 will be the first year in centuries when fewer babies are born than people die.<sup>2</sup>” The growing proportion of the elderly in the overall population and low reproduction will be worldwide. The older the population becomes, the fewer blood donors there are. One hopes Megakaryon’s next clinical study will be successful with Japanese patients, and its technology will be applied to patients of various ethnic/racial backgrounds.

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<sup>2</sup> Article “The old world: Shrinking populations will suppress economic growth and foster unrest” page 67 of The Economist May 25<sup>th</sup> 2024 edition