CiRA Foundation

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CiRA Foundation Activity Report

vol. 3
December2021

Message

Aiming to make iPS cell therapy a common treatment option

How was 2021 for you, with all of the uncertainty due to COVID-19? In July, we decided to set up a facility in Osaka for the my iPS project to create iPS cells from patients' own cells. We hope to start providing the iPS cells to medical institutions from 2025. In August, the project members moved to the Kyoto Research Park in order to secure research space and to accelerate research and development for more efficient cell production. In addition, we have also started providing to universities and companies overseas iPS cell stock made from the blood of donors whose cell types are less likely to cause immune rejection in many people when transplanted. The production of iPS cells for medical use is completed only through strict adherence to the prescribed process and detailed quality testing. Cell manufacturing is a continuous process that requires steady and patient work, and we work hard every day while dreaming of a future in which medical treatment using iPS cell technology reaches patients.

For us, 2021 was a year where we felt more than ever that we were with all of you in our activities: donors who cooperate with blood collection, companies, medical institutions and research institutes that use our iPS cell stock, and donors who support our activities. May 2022 be a good year for you all.

Representative Director
Shinya YAMANAKA

Contribution

Contribution to the research on COVID-19

We are making the following efforts to quickly clarify the onset mechanism and the causes of the different severities of COVID-19.

- Providing iPS cells derived from COVID-19 recovering patients.

We performed the quality testing for the COVID-19 recovery patient-derived iPS cells established at the Center for iPS Cell Research and Application, (CiRA) Kyoto University, and have been providing them free of charge to domestic and foreign research institutes (including commercial companies) since March 2021. This is a project to promote the research on COVID-19 with the cooperation of CiRA, Rinku General Medical Center, Kyoto University Hospital. Through this project, we aim to contribute to the establishment of appropriate diagnostic, preventive, and therapeutic methods for COVID-19.

- Genome analysis of COVID-19

We perform the genomic analyses of SARS-CoV-2 and the iPSC-derived airway cells and lung cells infected with SARS-CoV-2, commissioned by Kyoto University Graduate School of Medicine, Kyoto University Hospital, and research institutes such as the National Institute of Infectious Diseases. Through these analyses, we will contribute to the elucidation of the mechanism of virus infection, the rapid monitoring of virus mutant strains, and the study of measures for controlling epidemics, and we will utilize the test methods for our research and quality evaluation of iPS cells.

Project1

Accelerate my iPS project
New base in Kyoto Research Park

iPS cells differentiate into cells of various tissues and organs in the body. Patients can minimize the risk of rejection and other problems when transplanted with iPS cells made from their own cells. But on the other hand, custom-made treatment for each patient may be expensive. Our my iPS project (hereafter referred to as "the project") aims to efficiently manufacture iPS cells so that we can provide patients in need with self-derived iPS cells at an affordable price. We have already decided to set up the manufacturing facility for the project at the International Hub for Healthcare Innovation in Nakanoshima, Osaka.

In July, the department responsible for the project branched off from the Facility for iPS Cell Therapy (FiT) as the Research and Development Center, with the aim of providing cells from 2025. In August, the center relocated from the Kyoto University campus to the Kyoto Research Park in Tambaguchi to provide sufficient space for its research and development activities, which are expected to progress in the future. We have already started joint research with various organizations related to the project, which will further accelerate our research and development.

The practical application of transplantation medicine using one's own iPS cells has great potential to overcome diseases that have been difficult to treat in the past. Nearly 20 researchers are working on research and development every day to put iPS cell-based medical care to practical use as soon as possible. There are still many technical challenges to overcome in order to achieve both the safety and cost reduction of this medical treatment and make it available to everyone. Innovative ideas and steady research are essential. There are several issues that we cannot solve on our own, such as the design of automated equipment and the introduction of AI-based image processing technology. Our mission is to overcome these difficulties with the tremendous support and cooperation of corporations, universities, hospitals, and other organizations, both domestic and international, and this is the most "rewarding" challenge for us.

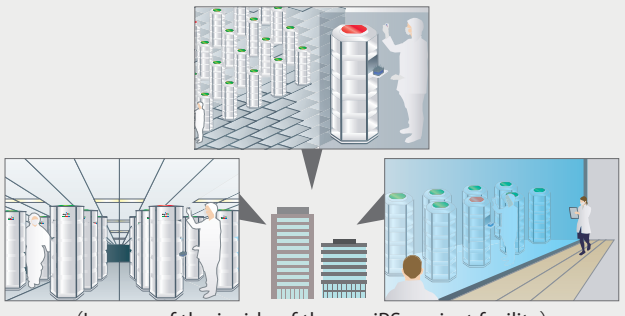
Masayoshi TSUKAHARA,
Head of Research and Development Center

my iPS Project Practical Application Model

myiPS

Establishment of a technology platform
Development of a series of infrastructure

The my iPS project facility
(International Hub for Healthcare Innovation※)



(Images of the inside of the my iPS project facility)

Consultation Desk

- Reception and consultation

Medical institutions

Medical Examination

- Medical interview
- Tests for infectious diseases, etc

Blood sampling

Cell Reprogramming / Expansion culture of iPS cells

Induction of differentiation from iPS cells

Frozen storage of cells

Induction of differentiation from iPS cells

External CPC

Medical institutions

Patients

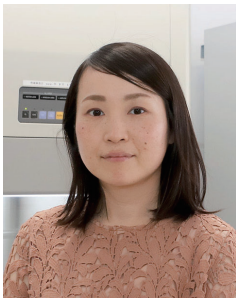
Treatment with differentiated cells from iPS cells

※The International Hub for Healthcare Innovation

A unique center in Japan that features a concentration of medical institutions, companies, start-ups, and support organizations under one roof. The Organization for Advanced Healthcare Innovation, a general incorporated foundation established by private companies and Osaka Prefecture, will be responsible for the operation of the center.

Topics

A day in the life of a researcher at the R&D Center



Yuko KITANO

Responsible for process development for my iPS project

9AM Going to work

I took my child to a preschool and went to work at 9:00. I usually listen to music on my way to work to boost my motivation.

Morning work

I checked my email to see if there was any urgent work to be done. After that, I had a meeting with other staff members to ensure that the day's work goes smoothly and prepare to start my research.
We are examining what the best facilities for iPS cell production look like and how they should be designed to reduce costs. Today, we also compared the equipment that might be useful for our project.

12PM Lunch time

I bought a lunch nearby and ate it at my desk to protect myself from COVID-19.

Afternoon work

We discussed how to extract the source of iPS cells from blood with the company. When creating iPS cells, we can use fresh blood that has not been stored for a long time, rather than the commonly used commercially available somatic cells. This is only possible thanks to the cooperation of our blood collection volunteers. The project's goal is to provide safe and effective medical care for all patients who wish to receive it. To achieve this, it is necessary to effectively treat a wide range of blood types, with different conditions for different people. I am grateful every day to all the volunteers. In addition to the meeting, I also wrote papers and prepared materials for conferences.

5PM End of work

I am currently working shorter hours to raise my child. But thanks to the support of my colleagues, I have managed to get my work done. Every time I ask them to take over a task on a day off or after hours, I feel sorry for myself and renew my resolve that I will support the next generation of colleagues as they raise their children. Although I am inevitably pressed for time, my supervisor leaves it to me to manage my time, which makes it easy for me to work.

In closing

When I was a student, I studied embryology in the hope that my research would lead to helping people, and now I feel that desire again. There is still a lot of research and development to be done before this project becomes a medical system that can actually be provided, but I will continue to do my best while maintaining a good balance between work and family.

Project2

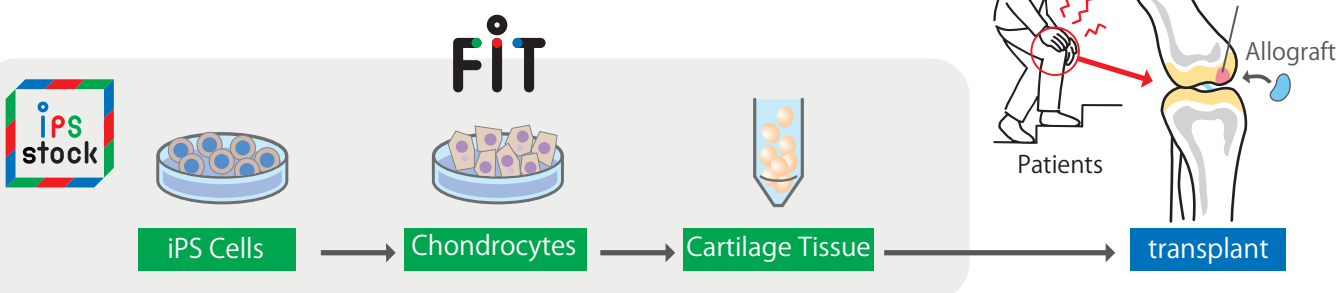
Toward the realization of therapies to regenerate cartilage

It is very difficult for articular cartilage tissue to self-repair once it is damaged. One of the effective treatment approaches is to take out the patient's own cartilage tissue, culture it, and transplant it into the damaged area. But with this approach, the patient has to undergo surgery twice. Professor Tsumaki has been conducting clinical research to treat patients who have damaged cartilage in their knee joints using cartilage made from iPS cells.

In 2019, he submitted a plan for the provision of regenerative medicine to the Japanese government. We have been assisting him in many ways to ensure that his project proceeds efficiently, including regulatory compliance and coordination with Kyoto University Hospital, with which he is collaborating. The cartilage for transplantation was also manufactured at FiT using our iPS cell stock. Even though we have extensive experience in manufacturing and quality testing iPS cell-derived processed products, we are nervous about cooperating in such clinical research. Working towards the same goal as the researchers is very rewarding for us. We will continue to play our role in realizing clinical applications using iPS cell technology while staying close to the researchers.

Based on his own experience as an orthopedic surgeon, Professor Tsumaki hopes to cure patients' diseases and disorders as much as possible. He has been engaged in a wide range of research, including basic research on the function of genes that are important for cartilage formation and differentiation, drug discovery research to find therapeutic agents for bone system diseases, and regenerative therapy research for iPS cell-derived cartilage transplantation.

Graduate School of Medicine /Flontier Biosciences, Osaka University
Center for iPS Cell Research and Application（CiRA）, Kyoto University
Noriyuki Tsumaki M.D., Ph.D.



Thanks

Thank you for your support of our activities

We are engaged in "translational" activities to support the transfer of university-originated technology to companies toward the practical application of new medical care using iPS cells. It is important that medical institutions, pharmaceutical companies, venture companies, etc. succeed in commercializing treatments and medical technologies using iPS cells, and that they eventually reach the people who need them. By producing quality-assured iPS cells for regenerative medicine and providing them free of charge to non-profit institutions and at a low price to for-profit institutions, we hope to promote the entry of research institutes and companies into this field, shorten the time required for the practical application of iPS cell-based medicine, and reduce R&D costs. Our activities are supported by public research funds and project income, as well as by your donations. We would like to thank you again for your donations, which are helping us to build bridges with industry and deliver iPS cell technology to patients.

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