
Preface

Recent advances in stem cell biology have enabled us to examine the regeneration of various tissues and organs. One of the biggest advances in this field is the induced pluripotent stem cell (iPS cell), which was developed by Shinya Yamanaka in 2006. The iPS cell was a tailor-made multipotent stem cell, and was generated by transfection of the combination of several embryonic stem (ES) cell-specific transcription factors such as Oct3/4, Sox2, and Klf4. It has pluripotency, and can differentiate into various types of cells such as ES cells. Because the iPS cells maintained all the genome information including HLA, it cannot be immune-rejected when its derived cells are transplanted to the host. In 2014, the first clinical trial was performed by Masayo Takahashi in patients with senile macular degeneration using autologous iPS cell-derived retinal pigmented cells. Other clinical trials are now being conducted in diverse ways including the cornea, Parkinson's Disease, spinal cord injury, platelet production, and severe congestive heart failure. Realization of these projects has been eagerly awaited by patients with severe intractable diseases.

It was also expected that iPS cells could be used in another field: disease modelling. Hereditary diseases are caused by genome mutations, but their clinical phenotypes, severity, onset, and treatment show wide variation. Mouse models of human disease have been generated by gene targeting and transgenic animals during the past 25 years. Nevertheless, these animal models cannot always help us to simulate human disease and screening of drugs. Genes and proteins are different between mice and humans. Because human tissues and cells were not usually available in the in vitro experiments except for small amounts of tissue obtained by biopsy or autopsy, this became a big hurdle for in vitro phenotype analysis and drug development. Moreover, disease modelling of human cells was not available in a routine clinical and experimental setting. Generation of patient-derived iPS cells and induction of in vitro differentiation into the targeted cells and tissues greatly changed the situation. Use of iPS cells for the investigation of disease modelling and drug screening is certain to change the future direction of research and industry.

In this book, we asked the top scientists in the field to write about human iPS cells for disease modelling. This will greatly help readers to understand what an iPS cell is, how to make iPS cells from blood cells, how to apply these techniques to approaching a pathophysiological analysis, and how to perform drug development

for patients with hereditary diseases. I strongly hope that readers can easily understand this field and will attempt disease modelling and tailor-made drug development for patients around the world.

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