

Session 3: Stem Cell Therapy

Cell therapy by generation of disease-corrected, patient-specific pluripotent stem cells

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Abstract

Several diseases with genetic etiologies have proved to be cured by bone marrow transplantation. However, donor availability constrains its usage. Recent technologies have made human induced pluripotent stem (iPS) cells a foreseeable and realistic source to release this limitation.

iPS cells derived from somatic cells hold promise to develop patient-specific cell therapies and provide experimental platforms to model human diseases. iPS cells are the product of somatic cell reprogramming to an embryonic-like state. Reprogramming mature somatic cells to generate iPS cells occur by the introduction of a defined and limited set of transcription factors. Very recently, iPS-cell technology has been successfully applied to human somatic cells and used for the generation of disease-corrected, patient-specific cells with potential value for stem cell therapy. The generation of patient-specific iPS cells could be used in the treatment of several human inherited diseases especially fatal diseases requiring haematopoietic stem cell transplantation. One of the most suitable candidates is a disorder called Wiskott-Aldrich syndrome (WAS). WAS is an X-linked recessive disorder characterized by immunodeficiency, thrombocytopenia and eczema. Without bone marrow transplantation, most patients die by 10 years old due to recurrent infections, haemorrhage or autoimmune diseases. We have identified a Thai patient with classic WAS with a novel and unique termination codon mutation (p.X503R) in the WAS gene. This resulted in an absence of protein with a severe phenotype in this patient. Since bone marrow transplantation cannot be performed due to a lack of a suitably matched donor, disease-corrected, patient-specific iPS cells could be an alternative source for treatment of this fatal disease.

Keywords: human induced pluripotent stem cells, iPS, patient-specific cell therapy.