Symposium 6: Haematopoietic Stem Cell Transplantation for Primary Immunodeficiencies

Title: **Hematopoietic cell transplantation for primary immunodeficiency diseases: Current situation and future direction**

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Hematopoietic cell transplantation (HCT) provides curative therapy for patients with severe form of primary immunodeficiency diseases (PIDs). Recent improvements in HCT have led to better outcome with less transplant related adverse effects. In this session, we show the results of HCT carried out in Japan for PIDs including severe combined immunodeficiency (SCID), Wiskott-Aldrich Syndrome (WAS), CD40L deficiency, Chronic granulomatous disease (CGD), X-linked lymphoproliferative disorder (XLP) type 2, Chronic mucocutaneous candidiasis (CMCD), and Hyper-IgE syndrome (HIE). 5 year overall survival (OS) for most of the disorders receiving HCT was around 70-80%. Similar OS was observed in the patients receiving cord blood transplantation. HCT for some of the PIDs, such as CMCD and activated PI3K delta syndrome was associated with poor engraftment, high rate of rejection, or both. HCT is one of the curative therapeutic measures, but still needs modification and optimization of conditioning regimen, monitoring system, and supportive therapy for the better outcome. Gene therapy is expected to provide safer treatment option with better outcome in some of the disorders.