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Stellingen behorend bij het proefschrift

Clinical and Immunological Outcome after Paediatric Stem Cell Transplantation in Inborn Errors of Immunity

Su Han Lum

- I. HLA-mismatched donor transplantation with CD3+TCR $\alpha\beta$ /CD19 depletion results in excellent survival in children with inborn errors of immunity (IEI) or primary immunodeficiencies (PID) (this thesis).
- II. HLA-mismatched donor transplantation using CD3+TCR $\alpha\beta$ /CD19 depletion and unmanipulated HLA-matched donor transplantation result in comparable outcomes in young children with IEI (this thesis).
- III. Haematopoietic cell transplantation provides 100% survival in young children (< 5 years of age) with chronic granulomatous disease (this thesis).
- IV. Persistent CD4+ lymphocytopenia was observed in transplant survivors of MHC class II deficiency but immune function was sufficient to confer protective immunity, produce good immunoglobulin levels and vaccine responses (this thesis).
- V. Five-year cumulative incidence of post-transplant autoimmune cytopenia in children with IEI is 9.4% and its occurrence is associated with the presence of graft versus host disease and the use of alemtuzumab (this thesis).
- VI. Cumulative incidence of post-transplant non-haematological autoimmunity in children with IEI is 11% at 8 years post-transplant and none is reported after *ex vivo* T-cell depleted recipients (this thesis).
- VII. Children are not just little adults; just ask their haematopoietic stem cells (Williams et al, J Clin Invest; 2006; 116 (10): 2593-6).
- VIII. Advanced graft depletion of CD45RA+ naïve T cells can preserve memory-immunity in the allografts and confer protection against important viral infections in the early post-transplant period (Wing Leung, BMT, 2015; 968-977; Transpl Infect Dis, 2018; 20 (1)).
- IX. Multicentre collaboration, partnership with patient organizations, training opportunities and leveraging existing resources are keys to success for research in rare diseases (Augustine et al, J Child Neuro, 2013; 28(9): 1142-1150).
- X. Children's health is our society's ultimate treasure and therefore patient reported outcome measures should be included for assessment of intervention in children (Huang et al, Qual Life Res, 2015; 23(3): 747-750).
- XI. Holding faithfulness and sincerity as first principles is always right (Confucius).
- XII. Research collaboration is like a symphony orchestra which is greater than the sum of the individuals involved for the benefit of our patients.