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CYTOPEUTICS® UMBILICAL CORD MESENCHYMAL STROMAL CELLS (CYTO-MSC) FOR PATIENTS WITH GRADE II–IV ACUTE GRAFT-VERSUS-HOST DISEASE: A PHASE I/II CLINICAL STUDY – PROTOCOL OVERVIEW

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Background: Graft-versus-host disease (GVHD) is a devastating complication following allogeneic hematopoietic stem cell transplantation (HSCT) mediated by stimulation of antigen presenting cells (APCs) which leads to donor T-lymphocytes activation and target tissue destruction, particularly affecting the skin, gastrointestinal tract, and liver in acute setting. In recent years, researchers have discovered that the application of mesenchymal stromal cells (MSCs) as salvage treatment among steroid refractory GVHD patients improves outcomes without long-term risk association. On the other hand, the use of MSCs concurrently with steroids as front-line treatment for acute GVHD has yet to be researched on. We hypothesize that this approach, as the MSCs will be administered at earlier stage of the disease, will increase survival rate and reduce mortality among aGVHD patients.

Objective: In this study, we aim to determine the efficacy and safety of allogeneic infusion of Cytopeutics umbilical cord-derived mesenchymal stromal cells (Cyto-MSC) in combination of standard corticosteroid therapy as front-line approach for treatment of grade II-IV acute GVHD patients.

Methodology : This is a phase I/II clinical study involving patients who underwent an allogeneic HSCT for malignant or non malignant haematological disorders and developed grade II-IV acute GVHD. A total of 40 eligible patients will be recruited in this study. For Phase I open labelled study, 5 eligible patients will be recruited to receive Cyto-MSC (5x10 MSC per kg bodyweight) and standard treatment. Meanwhile, for Phase II double blinded placebo controlled study, another 35 eligible patients will be recruited and randomized into 2 study groups where 15 patients will be assigned into Group A to receive Cyto-MSC (5x10 MSC per kg bodyweight) and standard treatment, meanwhile another 20 patients will be assigned into Group B to receive Placebo and standard treatment. Cyto-MSC or Placebo will be administered at Day 1 and Day 4. Another infusion of Cyto-MSC or Placebo will be given at Day 7 if the patient shows no or partial response based on GVHD grading criteria. All patients will be assessed up until 6 months follow-up which include medical history, clinical and physical evaluations, pathology investigations, biomarkers and immune cell subsets analysis, as well as quality of life questionnaires.