

## RENAL DEVELOPMENT AND CYSTIC DISEASES

FP051 **AUTOLOGOUS BONE MARROW MESENCHYMAL STROMAL CELLS TO PREVENTING THE PROGRESS OF CHRONIC RENAL FAILURE IN PATIENTS DUE TO AUTOSOMAL DOMINANT POLYCYSTIC KIDNEY DISEASE**

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**Introduction and Aims:** In autosomal dominant polycystic kidney disease (ADPKD), cysts arise focally and disrupt normal renal tissue leading to chronic renal failure (CRF). Bone marrow mesenchymal stromal cells (BM-MSCs) are renotropic, helping to kidney regeneration and improve renal function in CRF. This study was designed to evaluation of the efficacy and safety of autologous BM-MSCs therapy in patients with

CRF due to ADPKD (ClinicalTrials.gov Identifier: NCT02166489).

**Methods:** ADPKD patients confirmed by ultrasound imaging and genetic tests enrolled with glomerular filtration rate (GFR) between 25–60 mL/min/1.73 m<sup>2</sup>. Intravenous administration of BM-MSCs was performed in five eligible ADPKD patients. The safety and efficacy potential of cells were followed during 12 months. Assessments of paraclinical and clinical findings were completed at 1, 3, 6 and 12 months after cell injection. The changes in GFR rate were evaluated by dimercaptosuccinic acid (DMSA) scan isotope.

**Results:** The results of genetic analysis, ultrasound imaging, paraclinical and clinical findings of patients demonstrated the ADPKD. The safety of autologous BM-MSCs (1×10<sup>6</sup> cells/kg) transplantation was confirmed in five patients with ADPKD and no cell related adverse events reported in renal functional indexes during 6 months follow up. The paraclinical assessment of serum creatinine, BUN and GFR revealed that BM-MSCs may prevent the progression rate of CRF in patients with ADPKD. We did not observe any mass formation or increase in size and number of cysts in ADPKD patients by sonographic follow up.

**Conclusions:** Our report shows that intravenous transplantation of autologous BM-MSCs prevents the progression rate of CRF in ADPKD patients. These data demonstrate that autologous BM-MSCs therapy in ADPKD patients is safe and exhibited recovery and protective properties. This study provides an invaluable tool to confirm safety results obtained of BM-MSCs transplantation in ADPKD patients for evaluating the efficacy of these cells in clinical trials.