Treatment with Human Mesenchymal Stem Cells (Remestemcel-L) Is Effective in Pediatric Patients with Refractory Acute Graft Versus Host Disease*

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Abstract

Disease in Pediatric Patients

Note: This presentation updates a previous report of results from the first 75 patients treated in this program: Kurtzberg J, Chadhuy S, Prockop S, et al. A clinical trial of a cytokine receptor supergene fusion protein (Prochymal or Prochymal L) Is Effective in Pediatric Patients with Severe Refractory Acute Graft versus Host Disease. The results of this study support the use of remestemcel-L for patients with GVHD of all grades.

Study Design, Eligibility, and Treatment

Eligibility Criteria:

- Signed or witnessed consent by parent or legal guardian
- Age greater than 17 years of age
- Exclusion of patients in treatment for Grade A (≤ 10% death) GVHD
- Participant has received at least 3 months of standard methotrexate before remestemcel-L treatment
- No known evidence of a pulmonary infiltrate or diffuse alveolar hemorrhage and must have been unlikely to have received treatment within the last 12 months

Study Design:

- Expanded Access Program (EAP) Protocol 275 sponsored by Mesoblast, Inc.; Participating institutions: Duke University Hospital, Durham, NC; Al fonso Children’s Hospital, Columbus, OH; University of Alabama Hospitals/Case Western Reserve University, Cleveland, OH; Texas Children’s Hospital, Houston, TX; Children’s Hospital of Philadelphia, Philadelphia, PA.

Effect of Additional Remestemcel-L Treatment

- Approximately 60% of patients showed improvement in all organs with additional treatment
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Safety

- No known evidence of a pulmonary infiltrate or diffuse alveolar hemorrhage and must have been unlikely to have received treatment within the last 12 months
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Participating Institutions

- Duke University Hospital, Durham, NC; Al fonso Children’s Hospital, Columbus, OH; University of Alabama Hospitals/Case Western Reserve University, Cleveland, OH; Texas Children’s Hospital, Houston, TX; Children’s Hospital of Philadelphia, Philadelphia, PA.

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