NEONATAL PULMONARY RESEARCH

Mesenchymal Stem Cells for Bronchopulmonary Dysplasia: Phase 1 Dose-Escalation Clinical Trial.

Background

Bronchopulmonary Dysplasia (BPD) is a major problem in preterm infants lacking an effective therapy. Previous studies have demonstrated the attenuation of hyperoxia-induced lung injuries after transplantation of mesenchymal stem cells (MSC) in animals. This is the first study describing intratracheal transplantation of MSC in humans. The authors make use of umbilical cord MSC, which are advantageous due to their high availability and proliferation capacity.

Summary of results

The primary goal of the study was to demonstrate the safety of the intratracheal MSC transplantation. It was admitted to nine very preterm infants in two different dosages.

The secondary goal was to evaluate the potential efficacy for healing BPD. The authors found reduced BPD severity in the group of transplant recipients. In addition, cytokines and growth factors in tracheal aspirat of the transplanted group was measured and shown to be significantly reduced seven days post transplantation.

Strength

The primary goal of the study was reached, since the feasibility of intratracheal transplantation of MSC in human preterm infants could be shown. No serious adverse effects were reported.

Limitations

Regarding the secondary goal, the authors found significant reduction of BPD severity despite the small case number. In contrast to the results in animal studies a higher MSC dosage was shown to be less effective. Retrospective matching of the control and a lack of blinding of the transplanted group and doctors could have influenced these results. For further studies the optimal route, dose, and timing of the transplantation should be addressed, since the authors use only animal model-based data.

Practical conclusion

The study shows the feasibility of intratracheal transplantation of MSC in preterm infants. Further studies are needed to show its effectiveness and establish the optimal protocol regarding route, dose, and timing of the transplantation

Chang YS et al., Mesenchymal stem cells for bronchopulmonary dysplasia: phase 1 dose-escalation clinical trial. Pediatr. 2014 May;164(5):966-972.e6. doi: 10.1016/j.jpeds.2013.12.011.

Written by:

Dr. med. Isabell Deinert, MD