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Placental stem cell transplantation for congenital metabolic disorders

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Placental stem cells are a readily available cell source for regenerative medicine, which can be procured in a non-invasive manner, and there are few ethical concerns regarding their use. The hepatic differentiation potential of human amniotic epithelial cell (hAEC) suggests usefulness in cell therapies for congenital liver metabolic disorders including ornithine transcarbamylase (OTC) deficiency. OTC deficiency is the most common urea cycle disorder, which can cause severe brain damage and death. To determine if hAEC transplantation has a therapeutic effect against OTC deficiency, we transplanted hAECs into the liver pulp of wild type (WT) and spf/ash mice and measured the following parameters: Engraftment potential, OTC enzymatic activity, levels of urine metabolite markers and behavioral response to excess ammonia. We present our findings that hAECs can engraft in immunocompetent mouse livers and provide a therapeutic effect. While OTC enzyme activity from untreated spf/ash controls produced 12.75±0.91 μM citrulline/mg protein/h, spf/ash mice treated with hAECs (spf/ash-hAEC) produced increased activity at 19.08±1.33 μM citrulline/mg protein/h ($p<0.001$). In addition, spf/ash-hAEC mice possessed levels of urine metabolites more closely to levels found in WT controls ($p<0.05$), confirming the improvement in OTC enzyme activity. Lastly, spf/ash-hAEC mice better tolerate ammonia challenge, exhibiting improved behavioral phenotypes compared to untreated controls ($p<0.01$). This growing body of evidence better suggests that hAECs can be used for cell replacement therapy to treat OTC deficiency.

Biography

Toshio Miki is an Associate Professor of Surgery at the Keck School of Medicine of USC. He has received his MD and PhD degrees from Nihon University in Tokyo, Japan. With the goal of overcoming the organ shortage faced by patients awaiting liver transplants, he started studying Xenotransplantation and Hepatocyte Transplantation. In his search for alternative cell sources for hepatocyte transplantation, he discovered stem cell-like populations in the human amnion in the year 2005. Since then, he has been best-known for his studies of placental stem cells and their clinical applications.

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