平成 26 年度 医学・生命科学セミナー ／ D1"Medical & Life Science Seminar, 2014"

Embryonic stem cell-based therapy

Title has been changed.
演題名が変更となりました。

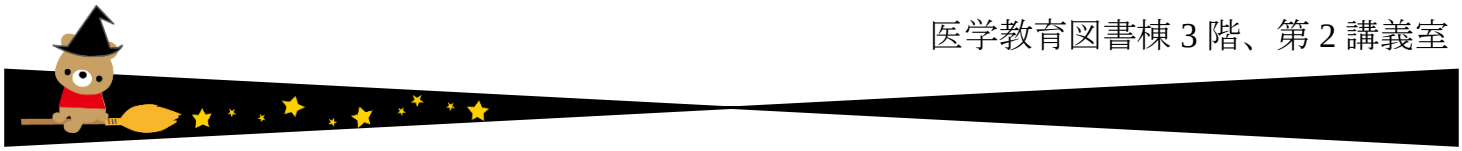
★**Lecturer: Dr. Akihiro Umezawa** 梅澤　明弘 先生

(National Center for Child Health and Development Research Institute / 国立成育医療研究センター)

★**Date: October 1st (WED), 2014 from 17:30.**　　平成 26 年 10 月 1 日（水）17:30.

★**Place: Lecture room 2, Medical Education & Library Building 2F.**

医学教育図書棟 3 階、第 2 講義室



Hyperammonemia is a metabolic disturbance characterized by an excess of ammonia in the blood, and is a dangerous condition that leads to mental retardation. Primary hyperammonemia is caused by inborn errors of metabolism such as ornithine transcarbamylase (OTC) and carbamoyl phosphate synthetase I in the urea cycle. OTC deficiency (OTCD) is an X-chromosome-linked disorder and is severe in hemizygous boys. Hemizygous boys often develop this disease during the neonatal period, and the patient often dies during this period. Living donor liver transplantation has been indicated for neonatal-onset type OTCD, and outcomes are favorable. However, neurological impairment associated with hyperammnonemic episode of OTCD often occurs before liver transplantation that is usually performed at an age of 5 months. For bridging to liver transplantation, we aim to perform embryonic stem cell-based therapy to a patient with OTCD to prevent hyperammonemia. To this end, we prepared hepatocytes derived from human embryonic stem cells as an investigational new drug. Sequencing analysis revealed that the embryonic stem cells as a raw material have intact the urea cycle-associated enzymes. The products expressed enzymes and exhibited metabolic activity of ammonia in vitro. To perform proof of concept (POC) studies in a disease model, we generated immunodeficient mice with OTCD, which can receive human embryonic stem cell-derived products. In addition, we generated OTCD pig by the nuclear transfer technique to establish treatment protocol and surgical procedure. Drug (cell) disposition can be determined by using these animals. In this study, we introduce results of our preclinical POC data and non-clinical pharmacology and toxicology.

★**Inviter: Prof. Inomata (Dept. of Pediatric Surgery & Transplantation)**

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