



第 463 回つくば分子生命科学セミナー

TSUKUBA MOLECULAR LIFE SCIENCE SEMINAR

演題 : Genetically Modified Cynomolgus Monkeys for Human Disease Modeling

演者 : Prof. Masatsugu Ema

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Institute for the Advanced Study of Human Biology (WPI-ASHBi), Kyoto University

日時 : 2021 年 12 月 3 日 (金) 17:00-18:30

会場 : 臨床講義室 D

要旨 :

Nonhuman primates (NHPs) are considered one of the most valuable animal models, because NHPs are closer to humans in organ size and anatomical structure, and therefore have higher potential to recapitulate human diseases, while difficult genetic manipulation is a major issue for creation of the disease models. So far, we have established techniques to create transgenic and genome editing cynomolgus monkeys. By using these techniques, we have explored an intractable human disease, Autosomal dominant polycystic kidney disease (ADPKD) with CRISPR/Cas9 technique, and demonstrated that homozygous disruption of PKD1 allele, a causative gene for ADPKD can result in the massive renal cyst development. We also have developed a technique to modify specific allele and generated PKD1 heterozygotes selectively. Importantly, the PKD1 heterozygotes exhibited renal cysts during fetal stages, showing the recapitulation of some of features of human ADPKD pathology.

参考文献

Tsukiyama T, et al. Monkeys mutant for PKD1 recapitulate human autosomal dominant polycystic kidney disease. *Nat Commun.* 2019 Dec 11;10(1):5517. doi: 10.1038/s41467-019-13398-6.

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