Aging Science Seminar

" Using stem cell and gene editing techniques to study and treat aging-associated disorders "

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Hutchinson-Gilford progeria syndrome(HGPS) and Werner syndrome (WS) are two human premature aging disorders with features that closely recapitulate the Characteristics of human aging. Mutations in LMNA and WRN genes lead to aberrant splicing product progerin and protein loss in HGPS and WS, respectively. Study on how genetic alteration leads to the cellular and organismal phenotypes of premature aging will provide clues to the molecular mechanisms underlying physiological aging and facilitate our understanding of the molecular pathways contributing to healthy aging. We have generated induced pluripotent stem cells(iPSCs) from fibroblasts obtained from patients with HGPS, Parkinson's disease(PD), Amyotrophic lateral sclerosis(ALS), Fanconi Anemia(FA), and Xeroderma pigmentosum(XP). Further, using targeted gene correction technique, we successfully corrected the mutated LMNA in HGPS-iPSCS, mutated LRRK2 in PD-iPSCs, mutated FANCA in FA-iPSCs, and mutated SOD1 and FUS in ALS-iPSCs. Finally, by using targeted "Knock-out" and "Knock-in" techniques, we generated WS-,FA-,PD-, and Glioblastoma multiforme(GBM)-specific human stem cells with relevant pathogenic mutations. Upon differentiation of these disease-specific pluripotent stem cells to specific somatic cell types, the latter recapitulated aging/diseaseassociated and tissue-specific phenotypic defects. Altogether, these studies provide important platforms for studying aging/disease mechanisms and developing new therapies.

Date: Tuesday, February 20th, 2018: Time: 11:00~12:00

Location: Biken Hall, 1st floor, main building, Research Institute for Microbial Diseases (微生物病研究所 本館1階 微研ホール)

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