ASHBi SEMINAR

A genome editing technology CRISPR-Cas3 in gene therapy

Lecturer: Tomoji Mashimo Ph.D.

Professor, Institute of Medical Science,
The University of Tokyo

Date Tuesday, 15 February 2022

Time 16:00 – 17:00

Venue Zoom Online Meeting*

*Register via the right QR code

Abstract

Although single-component Class 2 CRISPR systems, such as type II Cas9 or type V Cas12a, are widely used for genome editing in eukaryotic cells, the application of multi-component Class 1 CRISPR has yet to be developed. Recently we demonstrated that type I-E CRISPR, which is composed of Escherichia coli Cascade, Cas3, and programmable pre-crRNA, mediates distinct DNA cleavage activity in human cells. Notably, Cas3, which possesses helicase and nuclease activity, predominantly triggered several thousand base pair deletions upstream of the 5-ARG PAM, without prominent off-target activity. This Cas3-mediated directional and broad DNA degradation can be used to introduce functional gene knockouts and knock-ins. As an example of potential therapeutic applications, we show Cas3-mediated exon-skipping of the DMD gene in patient-iPSCs. We also highlight potential use for Cas3-mediated rapid, low-cost, instrument-free detection method for SARS-CoV2. This Cas3-based assay is comparable with Cas12- and RT-PCR-based assays in its speed and sensitivity but offers greater specificity for single-base-pair discrimination while negating the need for highly trained operators.

Organizer：Institute for the Advanced Study of Human Biology (WPI-ASHBi)

Contact: Associate Prof. Tomoyuki Tsukiyama

[E-mail] ttsuki@belle.shiga-med.ac.jp