Multidisciplinary Centre for Advanced Research and Studies (MCARS)

JAMIA MILLIA ISLAMIA

Jamia Nagar, New Delhi – 110 025

Cordially invites you to attend Lecture on

"Targeted genome editing in patient-derived pluripotent stem cells using gene-editing nucleases"

By Dr. Sivaprakash Ramalingam

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Abstract

Generation and precise genetic correction of patient-derived hematopoietic stem progenitor cells (HSPCs) and human induced pluripotent stem cells (hiPSCs) has great potential in regenerative medicine. Gene targeting is not a very efficient process in mammalian cells; only ~1 in 10^6 cells provided with excess template sequence undergo the desired gene modification. However, when a defined genomic double-stand break (DSB) is introduced, homologous recombination (HR) is induced efficiently at that site, in a large fraction of cells in a population. Thus, generation of specific desired genomic DSB has been the limiting step in HR technology for genetic-modification Such targeted genetic manipulations can now be achieved using programmable such as zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs) and CRISPR-Cas9 system. They have become powerful tools for targeted insertion of an exogenous transgene by homology-directed repair. In this presentation, I will discuss on genome engineering of patient-derived disease-specific hiPSCs and HSPCs using gene-editing nucleases for sickle cell anemia and beta-thalassemia.

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