Precise Genome Engineering: from Nucleus to Mitochondria

Kayeong Lim, Ph.D.
Principle Investigator, Senior Researcher
Brain Science Institute, KIST

Advances in genome engineering tools have opened up the possibility of directly targeting and modifying genomic sequences in almost all organisms. Base editing, a breakthrough genome engineering technology, can efficiently induce point mutations at target sites without generating double-strand DNA breaks (DSBs). Base editing enables C-to-T or A-to-G conversions in cell lines, animals, and plants, allowing researchers to study the functional effects of single-nucleotide polymorphisms (SNPs) and offering great hope for correcting the pathogenic mutation in gene therapy. In particular, the recently developed mitochondrial base editing strategy has broad implications for studying and treating mitochondrial diseases. Here, I will present novel methods for precise base editing in nuclear and mitochondrial DNA, using recombinant fusion proteins of programmable DNA-binding proteins and deaminases. In order to address the critical issues for therapeutic use, base editing has the potential to advance its specificity and versatility.

Keywords: Genome engineering, Base editing, Mitochondrial DNA editing, Programmable deaminases