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Genome editing *in vivo* with the delivery of Cas9 ribonucleoprotein and donor DNA complexed to gold nanoparticles

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The CRISPR/Cas9 technology has the potential to achieve therapeutic genome editing with high specificity and efficiency. However, the applications of Cas9 are limited by the lack of delivery vehicles that can simultaneously transduce proteins, guide RNA, and donor oligonucleotides into cells for genome editing. In this report we demonstrate that gold nanoparticles conjugated with DNA and assembled with endosomal disruptive polymers, can deliver Cas9 protein, guide RNA (gRNA) and donor DNA into various cells, including stem cells, and induce homology directed repair (HDR) with exceptional efficiency. Cellular treatment of the vehicle resulted in efficient cellular uptake. HDR experiments with donor ssDNA with restriction enzyme site showed that 15 nm gold nanoparticles complexed with Cas9 RNP can induce HDR in human embryonic stem (hES) and human induced pluripotent stem (hiPS) cells, with a significantly high efficiency of 5-6%. Compared to the conventional nucleofection method that resulted in low HDR efficiency in stem cells with high toxicity, GNP platform can efficiently deliver Cas9 RNP without cell detachment from culture plate and with significantly low toxicity. In addition, we demonstrate that gold nanoparticles can deliver Cas9 RNP *in vivo* to muscle tissue in mice. Intramuscular injection in mice resulted in significant uptake of fluorescently labeled Cas9 RNP in muscle tissue. We have developed a new delivery vehicle that can deliver both Cas9 RNP and donor DNA together for efficient HDR. Efficient editing of endogenous genes was achieved in many mouse and human cells.

Biography

Kunwoo Lee is getting his PhD in May 2016. He has published more than 8 papers in reputed journals.

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