KSN 2017 Abstract

Translational Research on Vaso-proliferative Retinopathy using in vivo Genome Editing

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From the FDA approval of anti-VEGF aptamer to wet-type age-related macular degeneration (AMD) of choroidal neovascularization, anti-VEGF aptamer and antibody have been widely used against all kinds of vaso-proliferative retinopathy. Actually, current therapies directed at controlling vascular abnormalities in vaso-proliferative retinopathy target VEGF and can slow the progression of these diseases. While the general role of VEGF in development has been well described, the specific function of locally synthesized VEGF in the eye is incompletely understood. Recently, RNA-guided genome surgery using CRISPR-Cas9 nucleases has shown promise for the treatment of diverse genetic diseases. Yet, the potential of such nucleases for therapeutic applications in non-genetic diseases including AMD, diabetic retinopathy (DR) as well as retinopathy of prematurity (ROP) is largely unexplored. Those vision-threatening retinopathies such as AMD, DR, and ROP are leading causes of blindness in adults and children, which is associated with retinal over-expression of, rather than mutations in, the VEGFA gene.
Herein I would like to provide some my recent experimental results of therapeutic applications such as small peptide, small molecule as well as nanoparticles beyond anti-VEGF antibody. In addition, some results of in vivo genome editing with Cas9 RNPs in vision-threatening retinopathy would be provided.