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Advances in therapeutic CRISPR/Cas9 genome editing

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Abstract:

Targeted nucleases are widely used as tools for genome editing. Two years ago the CRISPR-associated Cas9 nuclease was employed for the first time, and since then has largely revolutionized the field. The tremendous success of the CRISPR/Cas9 genome editing tool is powered by the ease design principle of the guide RNA that targets Cas9 to the desired DNA locus, and by the high specificity and efficiency of CRISPR/Cas9-generated DNA breaks. Several studies recently used CRISPR/Cas9 to successfully modulate disease-causing alleles *in vivo* in animal models and *ex vivo* in somatic and induced pluripotent stem cells, raising hope for therapeutic genome editing in the clinics. In this review we will summarize and discuss such pre-clinical CRISPR/Cas9 gene therapy reports.

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