Gene editing toward advanced cell therapy for diabetes

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Cell-based therapy for diabetes using stem cell-derived pancreatic β cells is still in its early stages, largely due to problems such insufficient insulin secretion and premature cell death. To address these critical barriers, we aim to engineer β-cell therapeutics using CRISPR-Cas9-mediated gene editing. Toward this goal, we first develop new gene-editing methods to overcome hurdles such as uncontrolled DNA repair outcomes and off-target DNA cleavages. Specifically, we employ Cas9–DNA conjugates and small-molecule Cas9 inhibitors to ensure precise and safe gene editing. We then apply these new gene-editing tools to creating designer β-cell therapeutics. Overall, our group develops and employs new gene-editing technologies to advance cell-based therapeutics.