

Project Summary

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New cellular therapeutic approaches for curative treatment of acute myeloid leukemia

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Despite recent advances, patients with relapsed or refractory acute myeloid leukemia (AML) still have limited durable treatment options. CD371-targeted CAR T-cell therapy has shown promising anti-leukemia activity in several clinical trials, but severe myelosuppression can occur because CD371 is also expressed on normal hematopoietic cells, highlighting a central challenge for cellular immunotherapy in myeloid malignancies. To overcome this limitation, we are investigating CRISPR-based genome editing strategies to modify normal hematopoietic stem and progenitor cells so they are less susceptible to CAR-mediated injury while preserving normal hematopoiesis. We will test the gene-edited hematopoietic stem cells in laboratory and preclinical models. Our goal is to preserve normal hematopoiesis while keeping the therapy effective against leukemia and minimizing side effects. By integrating stem cell biology, genome engineering, and translational modeling, this work aims to reduce on-target, off-tumor toxicity and enable safer and more durable next-generation cellular therapies for AML and other myeloid malignancies. (150 words)