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# Innovative T Cell Gene Editing System for Enhanced Cancer Immunotherapy

Tech ID: 34382 / UC Case 2023-195-0

#### TECHNOLOGY DESCRIPTION

Our breakthrough epigenetic engineering platform leverages RNA-based CRISPRoff and CRISPRon tools to program durable and reversible gene silencing or activation in primary human T cells. Unlike traditional genome editing approaches that rely on DNA double-strand breaks (DSBs), our system modifies the epigenome without permanent changes to the DNA sequence, ensuring precise and stable control of gene expression with minimal cellular toxicity. CRISPRoff enables heritable silencing of target genes by depositing DNA methylation, while CRISPRon reverses methylation to activate gene expression at targeted loci, including enhancers. This optimized, clinically compatible platform supports multiplexed epigenetic programming to address critical challenges in T cell therapies, including immune evasion, tumor resistance, and T cell exhaustion.

### **Competitive Advantages**

- ▶ **Safety and Precision:** CRISPRoff avoids permanent DNA damage, eliminating risks of chromosomal abnormalities and genotoxicity associated with traditional genome editing methods.
- ▶ **Durability:** Gene silencing persists through multiple cell divisions and T cell activations, with stable effects observed in vivo.
- ▶ Multiplexing Capability: Efficient and simultaneous silencing of up to five target genes without compromising cellular viability, overcoming limitations of existing editing technologies.
- ▶ **Reversibility:** CRISPRon enables targeted activation of genes and enhancers, unlocking therapeutic possibilities for gene reprogramming.
- ▶ Clinical Translation: RNA delivery of CRISPRoff and CRISPRon is compatible with GMP manufacturing processes, ensuring scalability and regulatory alignment for therapeutic applications.

# STAGE OF DEVELOPMENT

Our platform has demonstrated robust preclinical results, showing precise, durable, and specific gene silencing in primary human T cells. CRISPRoff silencing of RASA2, combined with targeted CAR knock-in at the TRAC locus, significantly improves CAR-T cell functionality in vitro and in vivo, achieving superior tumor control and extended survival in preclinical cancer models. Additionally, CRISPRon has successfully targeted

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# OTHER INFORMATION

**KEYWORDS** 

CRISPRon, CRISPRoff,

CAR-T, Cancer

#### **CATEGORIZED AS**

- ▶ Medical
  - ▶ Disease: Cancer
  - Therapeutics

**RELATED CASES** 

2023-195-0

endogenous enhancers, inducing stable FOXP3 expression in T cells, paving the way for regulatory T cell therapies.

# **RELATED MATERIALS**

▶ Integrated epigenetic and genetic programming of primary human T cells - 10/21/2025

# PATENT STATUS

Patent Pending

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