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CELL AND GENE THERAPY

# Effective Nucleic Acid Delivery For T-Cell Engineering

## Brief Description of Technology

Methods and tools to significantly increase tandem delivery of transgenes and guide RNAs to primary T cells for use in immunotherapy applications and functional screens.

**BUSINESS OPPORTUNITY**
Exclusive license
Non-exclusive license
Sponsored research

**TECHNOLOGY TYPE**
Therapeutic
Manufacturing
Tool

**STAGE OF DEVELOPMENT**
Preclinical *in vitro*

**PATENT INFORMATION**
Patent pending

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## Technology Overview

Genetic engineering involves both the transfer of genetic components as well as the directed editing of specific targets. Primary T cells represent an important target for gene editing approaches due to their use in immunotherapies, where ex-vivo modifications to introduce antigen recognition and signaling molecules are routinely performed. CRISPR-Cas9 screens for gene knockdown, which rely on guide RNAs designed to bind specific loci, have been underutilized in primary T cells due to inefficient delivery of guide RNAs to the cells using conventionally available lentiviral vectors. Hutch researchers have created a new T-cell Optimized for Packaging (TOP) lentiviral vector which drastically increases the efficacy of simultaneous sequence delivery of both guide RNAs and transgenes into primary T-cells. This could facilitate high-throughput screens in primary cells with or without reporter transgenes and could allow genetic therapy applications using paired transgenes and guide RNAs against targets of interest. Therefore, the TOP vector represents an optimal tool for tandem delivery of guide RNAs and transgenes to primary T cells for applications to ranging from immunotherapy treatments to functional screens.

## Applications

- Immunotherapies
- HIV Research and therapeutics
- Other processes which would require genetic engineering of T-cells

## Advantages

- Increasing transduction efficiency could reduce manufacturing costs as well as decrease the number of cells needed.
- Efficient tandem delivery of guide RNAs and transgenes allows for precise manipulations of T-Cells

**Market Overview:** The global genome editing market is estimated to grow from USD 1.4 billion in 2018 to USD 4.4 billion by 2023, at a CAGR of 25.7% during the forecast period. The global immunotherapy market is forecast to reach USD 126.9 billion by 2026, which represents a CAGR of 9.6% between 2019 and 2026.