UCLB Commercialising Technology

Lentivirally mediated mRNA delivery platform

Tech ID: 89-317

Available for: Licensing

Summary

This technology consists of a retroviral vector that has been engineered to enable direct expression and translation of the RNA vector genome on the target cell, bypassing the need for reverse transcription or integration into the target cell's genome. This modification leads to enhanced translation of the RNA vector genome.

Background

mRNA delivery avoids genomic integration associated with viral vectors, reducing the risk of unintended genetic alterations. This transient expression method allows precise control over gene expression levels and duration, crucial for applications requiring temporal regulation or where long-term expression could be harmful. Additionally, mRNA delivery offers versatility in targeting various cell types and tissues, potentially overcoming limitations of viral vectors such as tissue specificity and toxicity.

Technology

The academic team has created a novel HIV-1-based lentiviral (LV) vector that has been specifically engineered for the direct expression of its single-stranded RNA (ssRNA) payload upon target cell entry. The inventors redesigned and optimized the LV genome for this purpose by relocating all HIV-1 elements to the 3' untranslated region, resulting in ribosomal entry occurring at the 7-methylguanylate (m7G) 5' cap of the vector RNA.

It has been observed that this 5' cap-dependent LV (CDLV) vector significantly enhances the efficiency of LV ssRNA translation compared to previous technologies that relied on an internal ribosomal entry site (IRES) for translation.

Moreover, it was demonstrated that CDLV technology can deliver transient gene expression to mouse liver *in vivo*, matching the expression of DNA-based IDLV genomes over a 24-hour period. This introduces CDLV as a novel platform technology for potential use in transient treatment and manipulation of target cells.

Market

The market potential for mRNA delivery technology is substantial and dynamic. With the demand for safer, more efficient gene delivery solutions on the rise, the market for effective mRNA delivery systems is primed for robust growth and substantial returns on investment.

The therapeutic applications include vaccinations, protein replacement therapies, and genetic disease treatments, promising significant advancements in medical care.

Team

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IP

Patent pending in US and EU WO2021148805

Further Information

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