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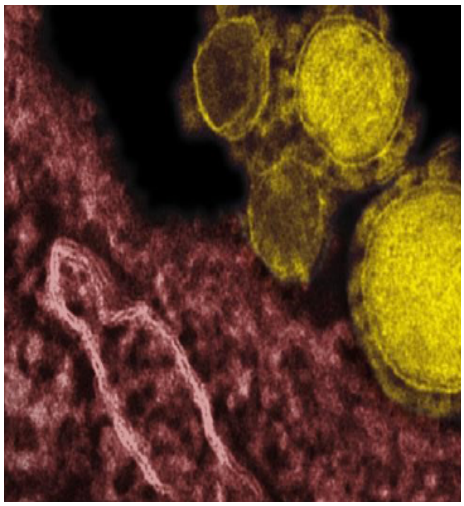

***IN VIVO* AND *EX VIVO* DELIVERY**

Efficient Delivery of Target Genes for HSC and T-Cell Based Therapies

Brief Description of Technology

Optimized Cocal vector and producer cell line for pseudotyping of therapeutic vectors.

BUSINESS OPPORTUNITY
Exclusive license
Sponsored research

TECHNOLOGY TYPE
Platform technology
Gene therapy
Cell therapy

STAGE OF DEVELOPMENT
Clinical

PATENT INFORMATION
EP 2427577
WO 2016118775

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Technology Overview

Gene and cell therapy-based therapeutics to address orphan monogenic diseases, viral diseases, and CAR-T and engineered TCR immunotherapy are dependent on efficient engineering of the target cell population. Lentiviral vectors (LVs) are the current standard for manipulation of hematopoietic stem cells (HSCs) and T cells. Dr. Kiem's research group has demonstrated that Cocal pseudotyped lentiviral vectors exhibit higher titers, broader species and cell-type tropism, and improved serum stability and decreased serum neutralization in patient samples, compared to the current gold standard, VSV-G. These advantages allow for efficient manipulation *in vivo* or *ex vivo* of the target cell population resulting in higher engraftment *in vivo*. In recent studies, Dr. Kiem has demonstrated that cocal LVs transduce CD34+ and CD4+ T cells with greater efficiency than the commonly used pseudotype VSV-G lentivirus.

Applications

- Efficient *in vivo* gene therapy
- Engineering HSCs and T cells *ex vivo* for autologous cell therapy

Advantages

- Cocal LVs transduce human, nonhuman primate, and canine HSCs allow for streamlined preclinical to clinical translation
- Validated producer cell line that stably expresses Cocal envelope for scalable manufacturing
- Optimized Cocal envelope outcompetes VSV-G in head to head comparisons

Market Overview

Autologous stem cell and non-stem cell based therapies utilize patient cells which are cultured, modified, and then reintroduced into the donor patient's body. The autologous cell therapy is expected to represent a $2.2 billion industry by 2017.

Investigator Overview

Hans-Peter Kiem, MD, PhD, Clinical Research Division