

Technology Offer

New viral packaging cell line

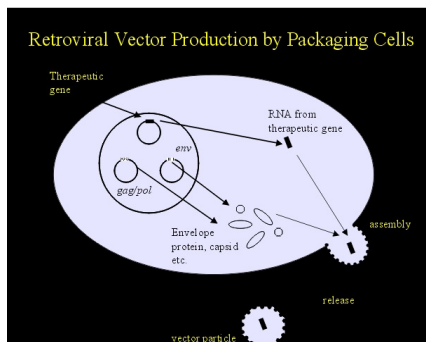
Reference Number
TO 02-00116

The Challenge

The high potential of the use of gene therapy to cure and slow down a wide spread scope of diseases has been shown in recent clinical trials. But the efficient introduction of therapeutic genes into human cells still remains the main problem. One established technique for gene transfer uses viral vectors, frequently retroviral vectors as they are robust, can be easily produced in high titers and efficiently infect a broad spectrum of cells. However, the production technology for retroviral vectors is still in early state. Inherent instability of infectious viral particles, the difficulty to produce large amounts of viruses as well as the broad host range of currently available viral envelopes and the sensitivity to human complement attack impede successful gene therapy. Presently recombinant retroviruses transducing a retroviral vector encoding for the therapeutic gene are produced after infection of the vector into packaging cell lines stably expressing necessary viral proteins (gag/pol and env). The construction of packaging cells is a tedious procedure including very time-consuming screenings. To facilitate this screening mostly therapeutic viruses carry selection or marker genes, which can cause severe problems in therapeutical settings like immune response or transduction of marker genes that turn out to be not biologically inert as they were expected to be. Therefore new processes allowing the production of large numbers of stable viruses containing no additional coding sequence besides the therapeutic gene are needed.

The Technology

The invention provides new methods for the production of virus producing cell lines. With the first step a *master producer cell line* is generated once providing a tagged cell line selected for optimized expression of various expression cassettes. With the second step the *master producer cell line* is targeted with the nucleotide acid molecule of interest. Due to the predicted integration site, the titer, the cultivation conditions and the safety record are not affected. This allows standardised easy production of virus which may be used in gene therapy.



Department of Chemical Engineering,
University of Cambridge, UK

Commercial Opportunity

The market for gene based therapeutics is predicted to increase heavily in the next years. Efficient innovative processes for virus production therefore result in important advantages:

- Time saving due to an only once generated *master producer cell line*
- Standardised production due to optimisation of cultivation conditions, titer and safety records for the *master producer cell line*
- No additional coding sequences besides the therapeutic gene

Patent situation

European patent was filed in November 2004 (EP04025919.4). An international application will follow.

Contact:

Sabina Heim, Ph.D.
Technology Manager
Ascenion GmbH

T: +49 (0)531 6181-961
F: +49 (0)531 6181-963
E: heim@ascenion.de



Berlin
Braunschweig
Munich

Ascenion GmbH
Herzogstrasse 64
80803 Munich
Germany

Intellectual Property
Asset Management

An Enterprise of the
Foundation for the
Promotion of Life Sciences