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HIGH-LEVEL RECOMBINANT ADENO-ASSOCIATED VIRUS PRODUCTION

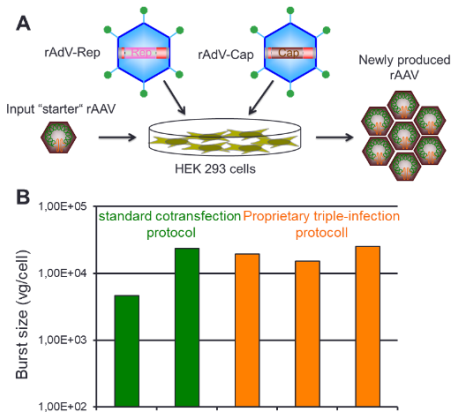
Keywords: viral vector, gene therapy, adeno-associated virus, manufacturing, GMP-level

INVENTION NOVELTY

The invention comprises a highly efficient methodology for high-level production of adeno-associated virus (AAV) vectors for gene therapy. This overcomes some of the major issues of transient transfection platforms, which are currently the mainstay for AAV vector manufacturing.

VALUE PROPOSITION

Standard cotransfection protocols to produce rAAV vectors are limited by efficiency and reproducibility of the transfection step as well as costs for the required highly purified plasmid DNA. These limitations greatly delay and hinder the seminal clinical use and translation of AAV technologies. The proposed platform technology circumvents these limitations by employing a novel triple infection protocol, providing the basis for a scalable, cost-effective (up to 50% reduction of costs), flexible and robust AAV vector manufacturing platform – a greatly desired solution for high-efficient gene therapy vector production at GMP-level.



A: Schematic representation of proprietary triple-infection protocol; B: Yields (burst sizes) of affinity purified rAAV-2 preperations.

TECHNOLOGY DESCRIPTION

By employing a novel triple viral vector infection protocol in mammalian cells, the technology enables the direct amplification of the desired AAV vector product. This new platform is based on first in class proprietary recombinant adenoviruses (rAdV) for AAV Rep and Cap expression, which can be easily propagated and exhibit high genomic stability. The methodology facilitates an efficient infection-based rAAV-packaging in adherent and suspension HEK293 cells, generating high titers of recombinant AAV with adjustable proportions of full and empty capsids. Notably, the proposed technology offers the following advantages compared to state of the art technologies:

+ Simple upscaling through adaption to HEK293 suspension cells

+ Highly reproducible production yields: High stability, high titers

+ Easy adaption to different AAV serotypes and novel capsid variants

+ High flexibility regarding new transgene variants (no requirement for stable cell lines)

COMMERCIAL OPPORTUNITY

This opportunity is available for in-licensing; strategic partnership for further development is highly welcomed.

DEVELOPMENT STATUS

The method was initially developed for AAV2 production in adherent cell culture. It has now been adapted for the use in suspension HEK293 cells and for various AAV serotypes. Upscaling to large production volumes and extensive down-stream processing for efficient purification of the rAAV vector product are currently established.

PATENT SITUATION

Priority European patent application and PCT application have been filed with priority of 2021.

FURTHER READING

Weger, S. et al. High-level rAAV vector production by rAdV-mediated amplification of small amounts of input vector (Submitted).

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