How to bridge the production gap in gene therapy

Significant scientific breakthroughs in molecular biology over the last decade also fueled gene therapy development. As the number of clinical trials in gene therapy applications increases, the need for industrial scale viral vector production follows. Current production systems cannot keep up with the growing demand – the market is urgently looking for novel solutions.

CEVEC Pharmaceuticals GmbH, a German-based expert in the production of tailor-made recombinant glycoproteins and gene therapy vectors, has developed what scientists are looking for – fully scalable, clinical-grade good manufacturing practice (GMP) compatible solutions for gene therapies.

Existing manufacturing methods for gene therapy vectors are sufficient for volumes demanded in rare diseases with small numbers of patients. However, when addressing more common diseases with gene therapy approaches, such as Alzheimer's, Parkinson's, or Rheumatoid Arthritis, manufacturing issues arise in late-stage clinical development and commercial production. The main issues are limited scalability, time-consuming and cost-intensive processes and, in some cases, the lack of sufficient reproducibility.

Vectors at industrial scale

The growth and the productivity of commonly used adherently growing cells, including HEK293 cells, is strictly limited by the available surface. As a result, production volumes can only be increased together with the number of cell factories and personnel. CEVEC has developed a suspension cell system that masters the challenges of scale-up.

CAP-GT suspension cells grow to high densities, are easy to handle, and adaptable to all current bioreactor formats, enabling industrial-scale production of viral vectors, including adeno-associated virus (AAV), lentivirus (LV), and adenovirus (AV).

Continuous production

Another, even bigger hurdle in industrial-scale production of viral vectors is the need for transient transfection, introducing the gene of interest as well as the genes needed to produce the virus in the corresponding cells. This manual step requires high amounts of (GMP-grade) DNA and adds costs and variability to the production process and limits the scale of a production run to a couple of hundred liters.

Helper-free packaging cell lines

To overcome this limitation, CEVEC has developed a stable production process that allows production of AAV vectors without the need for transient transfection or helper viruses.

Based on its proprietary CAP-GT platform, CEVEC has successfully introduced a novel stable AAV system that allows for large-scale production in all existing bioreactor formats. CEVEC's stable viral packaging cell lines neither require a laborious transient transfection step nor the infection with a helper virus, which is expensive to produce and must be removed during downstream processing. After stable integration of the gene of interest, CEVEC's AAV packaging cells allow for industrial-scale production comparable to current antibody manufacturing processes, paving the way for common gene therapy applications.

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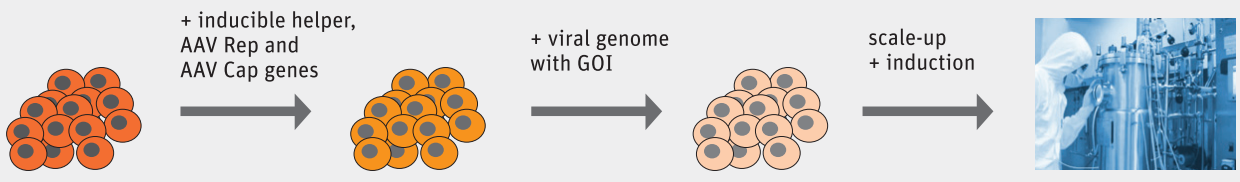
Stable production of AAV vectors in CAP-GT packaging and producer cells



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