MASTER THESIS - starting from spring 2025

Optimizating a Human Cell Line for Viral Vector Manufacturing in Gene Therapy

The goal of this project is to optimize a human production cell line for the manufacturing of viral vectors used in gene therapy.

Adeno-associated viruses (AAVs) are single-stranded DNA viruses whose genome is encapsulated by a capsid composed of three different proteins. AAVs belong to the genus Dependoparvoviridae and rely on helper viruses for replication. Due to their lack of pathogenicity and their potential as transfer vectors for gene therapy—particularly for monogenic disorders—they have gained increasing significance in recent years.

Despite many advancements, challenges remain in the biopharmaceutical production of recombinant AAVs (rAAVs) for gene therapy. The dependence on helper viruses significantly influences the manufacturing process when using production cell lines. Therefore, optimizing these production cell lines is crucial to enhancing both the yield and quality of AAV particles.

As part of this master's thesis, rAAV production will be improved through the targeted modulation of helper genes. These approaches aim to contribute to increasing both the productivity and quality of rAAVs.

Methods

The project involves cell and molecular biology techniques such as human cell culture, transient transfection, flow cytometry, cloning, and quantitative PCR, as well as virology techniques including transduction and titer assays.

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