ON-DEMAND, SHORT CLASS | LEVEL TWO | 50-MINUTES

SUGGESTED PREREQUISITE: THE BIOLOGY OF BIOTECH, INTRODUCTION TO GENETIC ENGINEERING, BIOMANUFACTURING

biotechPrimer
Life Science Training From Industry Experts

AAV Gene Therapy Manufacturing For Non-Scientists

OVERVIEW

AAV Gene Therapy Manufacturing For Scientists explains the features and functions of AAV viral vector platforms, focusing on Triple Transient Transfection. An explanation of the upstream bioprocessing, cell harvesting, downstream bioprocessing, fill and finish and packaging of AAV gene therapy products is given. The class ends with a review the CMC regulatory component of AAV manufacturing.

Five Takeaways:

1. Fluency in the structures and functions of AAV.
2. Knowledge of how AAV serotypes influence tissue tropism.
3. Compare and contrast the four primary AAV vector platforms.
4. Understanding the manufacturing workflow's purpose: upstream bioprocessing, cell harvesting, downstream bioprocessing, fill and finish, and packaging.
5. Survey of the key guidances for CMC testing of AAV viral vectors.

AGENDA

AAV Properties

- AAV structures and functions
- AAV serotypes
- Tissue tropism of popular AAV serotypes
- AAV characteristics

AAV Production Platforms

- 4 AAV production platform comparisons
- Key features of AAV vector DNA
- The AAV cassette
- Cell bank production
- Master cell bank and working cell bank
- AAV double-stranded and single-stranded DNA

AAV Upstream Bioprocessing

- Stages of AAV manufacturing
- Suspension and adherent cell lines
- Upstream bioprocessing steps
- Small and large batch production
- Bioreactors: hyperstacks, icellis

AAV Downstream Bioprocessing

- Cell harvesting
- Downstream bioprocessing
- Purification platforms: chromatography, filters, centrifugation
- Fill and finish
- Packaging
- AAV viral vector manufacturing workflow overview

AAV CMC

- Role of CMC
- ICH Q5A, ICH Q5B, ICH Q5D, ICH Q5E
- Identity testing
- Potency testing
- Quality testing
- Purity testing
- Sterility testing
- CMC regulatory and development considerations
- AAV manufacturing challenges