AAV Gene Therapy Manufacturing

45-MINUTE ONLINE COURSE | LEVEL 2

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

OVERVIEW

AAV Gene Therapy Manufacturing explains the design, function, and features of adeno-associated virus (AAV) systems, details on specific platforms used for transfection, and methods of validation and purification after the gene therapy product is created.

Five Takeaways:

1. Describe the characteristics of the naturally occurring Adeno-associated virus
2. Discuss the features of a mature AAV vectors
3. Describe the four primary platforms used to create AAV vectors
4. Explain how each of the following are characterized with respect to viral vectors: Safety, Identity, Potency, Quality, and Purity
5. Explain key regulatory considerations for AAV manufacturing

AGENDA

- **Adeno-Associated Virus Overview** provides an overview of the properties of the naturally occurring Adeno-associated virus including its origin and characteristics
- **AAV Vectors** describes how the adeno-associated virus is utilized in gene therapy using AAV vectors, including the composition of AAV viral vectors as well as the general characteristics of the types of genes that can be transfected using these vectors.
- **AAV Manufacturing and Control** discusses AAV Manufacturing and control including an introduction to manufacturing, the four primary platforms for AAV production, the characterization of AAV manufacturing products, and the regulation of AAV production.