



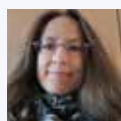
Speakers



Dr Kerstin Brack
Charles River Laboratories



Dr Sabine Hauck
Chair of ECA ATMP Interest Group



Dr Ulrike Herbrand
Charles River Laboratories



Anna Liznar
PathoQuest

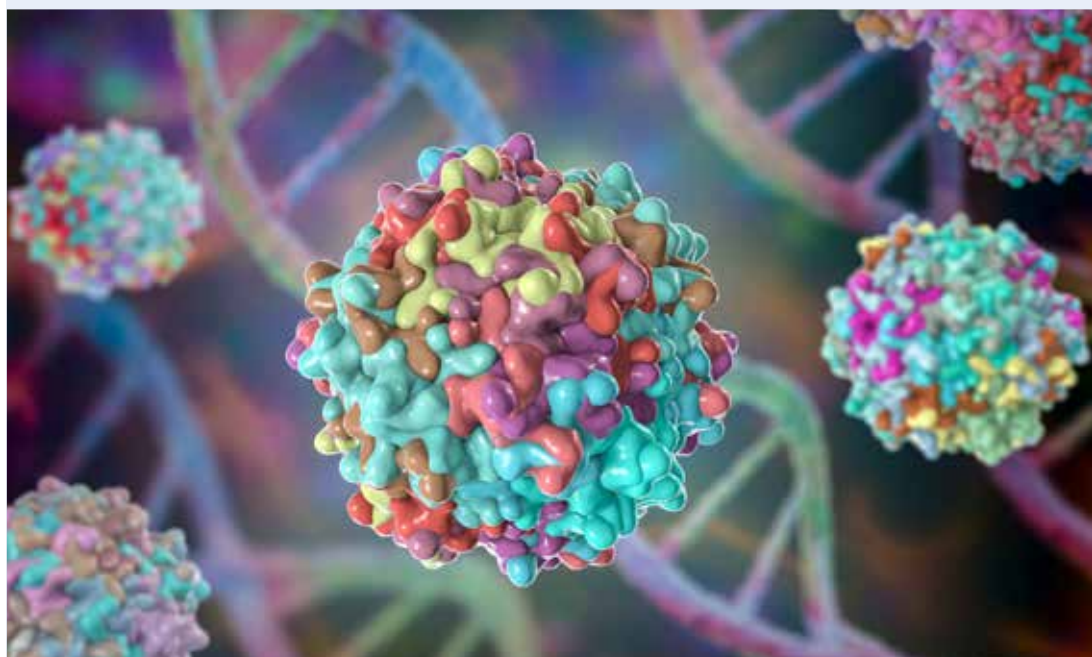


Roland Pach
Roche

SMART AAV Analytical Method Toolbox



Live Online Training on 17 June 2025



Highlights

- Case Studies
- CQA
- Method and Formulation Optimization
- Upscale/Scaling Production
- NGS

Suitable analytical methods to assess
AAV quality during development and
manufacturing

Objectives

Following a brief introduction to ATMPs and its regulations, this course will explore optimization strategies for the formulation, production, and analysis of adeno-associated viruses (AAVs). Participants will understand challenges and learn practical solutions for efficiency and compliance. The course highlights common obstacles and offers practical solutions demonstrated through case studies, providing insights into applying GMP principles in different scenarios.

In addition, expert speakers from manufacturing, research, and consultancy will share their experiences and lessons from GMP-compliant environments. Through their presentations, participants will gain a well-rounded understanding of the challenges and best practices in maintaining GMP compliance.

Background

Gene therapies are rapidly gaining popularity as a cutting-edge approach to treating a wide range of diseases, offering enormous potential to address conditions from degenerative diseases to rare congenital neurological disorders. These therapies have the potential to revolutionize medicine by targeting the underlying genetic causes of illnesses, providing long-lasting or even permanent therapeutic effects. Central to many DNA-based gene therapies is the use of viral vectors to deliver genetic material directly into the patient's cells, and among the various viral vectors available, the most commonly used and highly regarded is the adeno-associated virus (AAV).

The importance of AAV in the field of gene therapy cannot be overstated. Discovered in 1965, AAV has become the preferred vector due to its ability to efficiently transfer genetic material while exhibiting a relatively low immunogenic profile, meaning it is less likely to provoke an unwanted immune response. Additionally, AAV is known for its ability to transduce both dividing and non-dividing cells, making it a versatile tool for a variety of gene therapy applications. Its proven safety record, combined with its effectiveness in delivering therapeutic genes, has led to its widespread adoption in clinical trials and commercial gene therapies. AAV-based gene therapies are already showing promise in treating conditions such as spinal muscular atrophy, hemophilia, and certain inherited forms of blindness, highlighting their growing role in modern medicine.

However, as with any biopharmaceutical product, there are significant challenges associated with the development and production of AAV therapies, particularly in defining the appropriate Critical Quality Attributes (CQAs) that ensure the safety, efficacy, and consistency of the final product. Establishing these CQAs is a complex and highly regulated process, and failure to do so correctly can lead to inefficiencies, wasted resources, and even setbacks in the development process. Determining the best way to define and measure these CQAs—without wasting valuable time and financial resources—is a critical task for any organization working in AAV manufacturing.

This course will address these challenges head-on, providing valuable insights into how to effectively define CQAs for AAV products. It will explore strategies to optimize both the analytical methods and the manufacturing processes involved in the production of AAV-based gene therapies and their Quality Control. Participants will learn how to navigate these complexities efficiently and gain practical tools for improving the quality and scalability of AAV production, ultimately helping to bring these life-changing therapies to patients more quickly and reliably.

Target Audience

This course is addressed to all people involved in the day-to-day work of AAV with manufacturing, method development and optimization and analysis.

Moderator

Clemens Mundo, Concept Heidelberg

Programme

Introduction in ATMP and their Regulations (Dr Sabine Hauck)

- EMA and ICH guidelines for ATMPs, focusing on Gene Therapy products
- GMP for ATMPs
- Pharmacopoeial texts for AAV (Pharm. Eur. 3186, 5.34 and 5.2.12, USP viral vectors)

AAV Bioprocess – Fundamentals & Trends (Roland Pach)

- AAV platforms – Pro & Cons
- Manufacturing process
- CPP & cQAs
- Challenges & trends

Chemistry, Manufacturing and Control (CMC) Testing for AAV Therapeutics (Dr Rudolf Zirwes)

- Identity testing
- Purity testing
- Safety testing

From AAV Plasmids to Drug Product – Quality Testing with iDTECT NGS on GMP Grade (Anna Liznar)

- Virus detection for adventitious virus testing
- Genome Identity for sequence verification
- An outlook into genome integrity & residual DNA characterization

AAV Analytical Toolbox for Stability Assessment (Dr Sabine Hauck)

- Selection of suitable methods
- Analysis of the stability indicating power
- Learnings for formulation development

Bioactivity for AAV Therapeutics (Dr Ulrike Herbrand)

- Matrix approach
- MoA reflection
- Challenges related to references and control items

Speakers



Dr Kerstin Brack
Charles River Laboratories
Scientific Director, Global Biosafety

Dr Kerstin Brack works as a Scientific Director at Charles River Laboratories Germany GmbH since 2018 and is a subject matter expert for biosafety testing of biologicals. She holds a Diploma in Biology from the University of Bremen (Germany) where she subsequently also earned her PhD in Virology. In 2001 Kerstin joined NewLab BioQuality AG (now Charles River Laboratories Germany GmbH) as a Study Director in the Virology Department. Between 2004 and 2018 Kerstin headed the departments for biosafety testing and bioassay services at Charles River Laboratories Germany.



Dr Sabine Hauck
dequra pharma consult hauck

Sabine Hauck has 20+ years of experience in the biotech industry, in which she held various positions in pharmaceutical development, quality assurance, regulatory affairs and corporate development. Her product experience spans from small molecules to cell therapies and includes a variety of dosage forms. After gaining experience in several biotech companies she is now providing freelance consulting and trainings for biotech and biopharma companies in the field of pharmaceutical development, quality assurance, and regulatory affairs. Sabine is also active as the chair of the ECA ATMP interest group.



Dr Ulrike Herbrand
Charles River Laboratories
Scientific Director Global *in vitro* Bioassays

Ulrike Herbrand joined Charles River Laboratories in 2007. She is Scientific Director Global *in vitro* Bioassays and Head of the Bioassay Research & Development team at Charles River Laboratories' site in Erkrath, Germany. She gained a PhD in biological sciences during her time at the Max-Planck-Institute for Molecular Physiology in Dortmund (Germany) and worked five years at post-doctoral positions at medical research centers in the field of cancer research. She is an expert in mechanism of action-reflecting bioassays for protein therapeutics as well as for advanced therapy medicinal products.



Anna Liznar
PathoQuestUSP
Business Development Manager

Anna Liznar is a graduate from the University of Bayreuth in the field of RNA biochemistry. Anna is RNA and NGS enthusiast and has worked in the field of RNAi, Transcriptomics, Genomics and now Quality Testing of Biologics with NGS at PathoQuest.



Roland Pach
Roche
Global Analytical Expert CMC Cell- & Gene Therapy & alternative formats

Prior Roche, he was leading the Analytical Development department at Berna Biotech and the QC department of Bio-Process Development at Merck Serono. Roland is the global CMC Analytical Technical Lead in the cancer vaccines and cell- & gene therapy (CGT) area of Roche more than 10 years. In his assigned area, he represents Roche in external development projects, industrial consortiums like CGT BioPhorum and numerous due diligences of in-licensing candidates or companies in the CGT fields. In his second role at Roche as global technical development leader, he had led successfully new formats like immunotoxins from pre-clinics into entry to human (EiH).

Your Benefit: Internationally Acknowledged Certificate from ECA Academy



The EU GMP Guide requires: „... All personnel should be aware of the principles of Good Manufacturing Practice that affect them and receive initial and continuing training,...“. This is why you receive an acknowledged participant certificate, which lists the contents of the seminar in detail and with which you document your training.

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Live Online Training on 17 June 2025

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Date of the Live Online Training
Tuesday, 17 June 2025, 09.00 h – 15.30 h CEST

Technical Requirements

We use WebEx for our live online training courses and webinars. At <https://www.gmp-compliance.org/training/online-training-technical-information> you will find all the information you need to participate in our trainings and you can check if your system meets the necessary requirements to participate. If the installation of browser extensions is not possible due to your rights in the IT system, please contact your IT department. WebEx is a standard nowadays and the necessary installation is fast and easy.

Fees (per delegate, plus VAT)

ECA Members € 790
APIC Members € 840
Non-ECA Members € 890
EU GMP Inspectorates € 445
The fee is payable in advance after receipt of invoice.

Registration

Via the attached reservation form, by e-mail or by fax – **or search and register directly at www.gmp-compliance.org under the number 21882.**

Presentations/Certificate

The presentations will be made available to you prior to the Live Online Training as PDF files. After the event, you will automatically receive your certificate of participation.

You cannot attend the live online event?

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Conference Language

The official conference language will be English.

Organisation and Contact

ECA has entrusted Concept Heidelberg with the organisation of this event.

CONCEPT HEIDELBERG

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