ECA Academy
Your GMP/GDP Information Source

Live Online Training

Speakers

Dr Sabine Hauck
Chair of ECA ATMP Interest Group

Dr Ulrike Herbrand
Charles River Laboratories

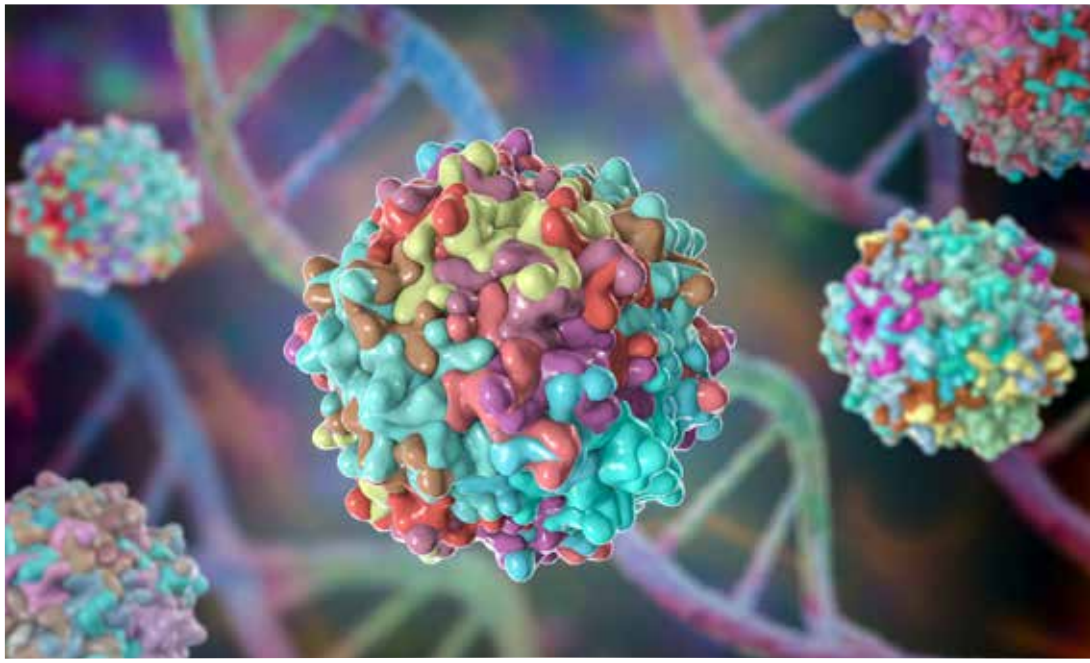
Anna Liznar
PathoQuest

Dr Amrita Pai
Charles River Laboratories

Dr Rudolf Zirwes
Charles River Laboratories

SMART AAV Analytical Method Toolbox

Live Online Training on 06 February 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 monographs for AAV (Pharm. Eur. 3186, 5.34 and 5.12, USP viral vectors)

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

Building and Breaking AAV Processes
(Dr Amrita Pai)

- How is development representative of future manufacturing scale? What data can we collect/analyze? How do we scale up?
- What tools do we have for making quick decisions? Are they worth the potential tradeoffs?
- What happens when something goes wrong? How can we be good stewards of time/resources?

Speakers

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.

Dr Amrita Pai
Charles River Laboratories
Associate Director

Amrita Pai currently works as the Associate Director of Process Development team for the Viral Vector Manufacturing group under CRL -Rockville, USA site. Amrita finished her Bachelor of Engineering in Biotechnology from Bangalore, India. She completed her MS in Biochemistry and Molecular Biology as well as her PhD in cell Biology from Georgetown University. Amrita has over 5 years of academic research experience and over 8 years of industry experience. Her previous place of employments has been at CROs like IQVIA, federal institutions such as NIH-NIDDK, and at pharmaceutical industry like MedImmune, now Astrazeneca. In her current role at CRL Amrita leads a team of scientists who primarily focus on to developing novel processes for viral vector production and purification, such as for AAV (adeno-associated virus), Ad (Adenovirus) and such, that are used in gene therapy phase-I clinical trials.

Dr Rudolf Zirwes
Charles River Laboratories
Global Coordinator Molecular Biology

Rudolf Zirwes joined Charles River Laboratories in 2007. He is Global Coordinator of Molecular Biology and Senior Scientist in the Research & Development team at Charles River Laboratories´ Erkrath site, Germany. He gained a PhD in cellular and molecular biology at the German Cancer Research Center (DKFZ Heidelberg). Rudolf has 20+ years of experience in the biotech industry, in which he held various positions in pharmaceutical drug discovery, development and quality control. His experience spans from small molecules to cell and gene therapeutics. In his current role, Rudolf is responsible for both local and global coordination and harmonization of assay development & validation activities for QC of advanced therapy medicinal products.

Your Benefit: Internationally Acknowledged Certificate from ECA Academy


CERTIFICATE

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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Reservation Form (Please complete in full)

SMART AAV Analytical Method Toolbox
Live Online Training on 06 February 2025

Title, first name, surname

Department | Company

Important: Please indicate your company's VAT ID Number | Purchase Order Number, if applicable

City | ZIP Code | Country

Phone / Fax

E-Mail (Please fill in)

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If you cannot attend the conference you have two options:
1. We are happy to welcome a substitute colleague at any time.
2. If you have to cancel entirely we must charge the following processing fees:
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In case you do not appear at the event without having informed us, you will have to pay the full registration fee, even if you have not made the payment yet. Only after we have received your payment, you are entitled to participate in the conference (receipt of payment will not be confirmed)! (As of January 2012).
German law shall apply. Court of jurisdiction is Heidelberg.

Privacy Policy: By registering for this event, I accept the processing of my Personal Data. Concept Heidelberg will use my data for the processing of this order, for which I hereby declare to agree that my personal data is stored and processed. Concept Heidelberg will only send me information in relation with this order or similar ones. My personal data will not be disclosed to third parties (see also the privacy policy at https://www.gmp-compliance.org/privacy-policy). I note that I can ask for the modification, correction or deletion of my data at any time via the contact form on this website.

Date of the Live Online Training

Thursday, 06 February 2025, 09.00 h – 15.30 h
All times mentioned are CET

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 € 690
APIC Members € 740
Non-ECA Members € 790
EU GMP Inspectorates € 395
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?

We also offer many of the training courses and conferences as recordings. This means that you can watch the videos of the event „on demand" – whenever it suits you – on our web server. It is quite uncomplicated and doesn't require any software – you simply watch the video on your browser. You can find all recorded events at www.gmp-compliance.org/recordings.

Conference Language

The official conference language will be English.

Organisation and Contact

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

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