



Speakers



Petra Falb
AGES



Pierre A. Morgon
PharmD, LL.M, MRGN Advisors



Dr Bernd Renger
Bernd Renger Consulting



Dr Frank Sielaff
GMP Inspector



Dr Lyudmil Tserovski
BioNTech



Peter Walters
CRB

Genetically Modified Vaccines – Additional Guidance needed?



Live Online Training on 08 December 2021



Use of regulations and interaction with authorities

Highlights

- Regulatory Frame and Authority Expectations
- Case Study – mRNA Product – Regulatory Requirements and Hurdles
- Business and Healthcare Policy Perspectives - from Acceptability across the Entire Landscape of Stakeholders to the Contribution to Availability and Accessibility
- Quality and Facility - Requirements and Stumbling Blocks on the Way to Market Authorisation

Objectives

Genetic modification technologies were widely used for vaccine development during the COVID-19 pandemic, for other vaccines and for veterinary vaccines. Several products have been approved around the world. In the EU, products that protect against infection with an infectious organism are by definition vaccines. And as such, vaccines are excluded from the ATMP guidelines - even if technologically they use techniques known from gene therapy. Particularly in the case of approaches that were not previously approved as medicines, such as mRNA lipid nanoparticles, some companies face a regulatory environment that does not seem fully transparent to them and in which there appears to be a lack of appropriate guidance. This Live Online Training discusses the regulatory landscape for such genetically modified vaccines, especially when these gene therapy technologies overlap with traditional vaccine areas and vaccine regulations. This workshop will highlight the current regulatory situation and use case studies from manufacturers to show the challenges and hurdles in development, manufacturing and approval. It also seeks to identify the extent to which further guidance is needed beyond the existing guideline documents. One question could also be how this is viewed internationally when the requirements or classifications of the products differ, e.g. in Europe and in the USA.

Background

The quality of a medicinal product, and thus also that of a vaccine, is of crucial importance for its safety and efficacy and thus for its suitability for the patient. Given the growing recognition of the increasingly important role of biological medicines in healthcare worldwide, competent authorities have already published a whole range of guidelines and helpful documents. Products such as ATMPs, but also genetically modified vaccines, have the potential to transform patient and healthcare delivery worldwide. However, the development, characterisation and manufacture of these innovative medicines is particularly challenging due to their high complexity and emerging technologies. In the course of development, transfer and manufacture, new questions often arise that also need to be clarified from the regulatory side. Are further specifications from the authorities necessary for this? Where are there still gaps? What experience have manufacturers gained so far in the approval process? Especially with a view to worldwide use.

Target Audience

This Live Online Training is aimed at all staff and regulatory representatives involved in the development, manufacture, quality assurance and authorisation of vaccines and/or ATMP in general and gene therapy medicines in particular.

Programme

Welcome and Introduction

Dr Sabine Hauck and Axel H. Schroeder

Current Regulatory Framework for Genetically Modified Vaccines

Petra Falb, AGES

- Relevant guidelines
- Licensing regulatory background

Case Study – mRNA Product – Regulatory Requirements and Hurdles

Lyudmil Tserovski, BioNTech

- Basic mechanism of action of mRNA based vaccines and anti-cancer ATMPs
- GMP aspects of mRNA-based medicinal products (manufacturing and control)
- Regulatory aspects depending on the type of medicinal product (ATMP or Vaccine)
- Challenges concerning the supply chain and the QP certification

Regulatory GMP Landscape

Frank Sielaff, c/o Local Council Darmstadt

- Relevant GMP guidelines
- What is in focus of a GMP inspection and what is not?
- GMP challenges

Possible (Quality) Pitfalls on the Way to Marketing Authorisation

Bernd Renger, former Chair of the European QP Association

- What quality systems and GMP compliance level to expect
- API and formulated API (LNP)
- Novel excipients
 - Functional and structural lipids
 - Polymerosomes
- Oligodeoxynucleotide adjuvants

Vaccines and Gene Therapy - Same but Different

Peter Walters, CRB

- Way of manufacturing vs. route of administration vs. legally classification
- The complication that vaccine technologies have evolved, but are still regulated under different conditions
- How the future of the industry will possibly need to be updated in the wake of gene therapy innovation

Business and Healthcare Policy Perspectives

Pierre A. Morgon, PharmD, MBA, LL.M

- From acceptability across the entire landscape of stakeholders to the contribution to availability and accessibility

Speakers

**Petra Falb, AGES**

Vaccines expert (AGES-BASG) - Austrian CVMP Member (EMA) - CVMP Immunologicals Working Party (EMA)

Petra Falb studied at Veterinary University Vienna, Austria. From 1998 – 2001 she worked as scientist at the Institute for Virology and later at the Institute for pathology. 2001-2003 she was self employed as veterinary surgeon. In 2003 she joined the AGES with responsibilities in quality assessment of human and veterinary vaccines (national, decentralised and centralized procedures). Until 2016, her focus was on viral vaccines. In 2017 she took over new responsibilities for veterinary vaccines.

**Pierre A. Morgon, PharmD, MBA, LL.M, MRGN Advisors**

CEO & Founder

Pierre studied at the university Claude Bernard and Jean Moulin Lyon. Additionally he is trained in Marketing, Marketing Management and General Management. Today he is CEO of MRGN Advisors in Geneva, Switzerland. Additionally, he is director at the board of 8 companies, and a partner in private equity funds investing in life sciences.

**Dr Bernd Renger, Bernd Renger Consulting**

CEO and former Chair EQPA

Dr Renger started at Hoechst AG. Since then, he has held several quality management positions at Mundipharma, Byk Gulden (now Takeda) and Baxter BioScience in Vienna and Vetter Pharma-Fertigung. He was a member of the European Compliance Academy (ECA) Advisory Board and is Immediate Past Chair of the European QP Association.

**Dr Frank Sielaff, c/o Local Council Darmstadt**

GMP Inspector

GMP Inspector at the Regierungspräsidium Darmstadt with focus on Inspection of drug manufacturers and laboratories in Germany and countries outside of the EU. Before joining the GMP-inspectorate Frank was several years employed in the pharmaceutical industry as Head of Quality Control and as Qualified Person.

**Dr Lyudmil Tserovski, BioNTech**

Qualified Person

Lyudmil studied mathematics with computer science as well as pharmacy at the Universities Darmstadt and Mainz. After 4 years of research and acquiring a PhD at the University Mainz he joined BioNTech IMFS in 2017 as scientist in the QC RNA Manufacturing. Today, he is a QP for ATMPs at BioNTech SE.

**Peter Walters, CRB**

Director, Advanced Therapies

Peter studied Chemical Engineering at UC Davies. From 2004 to 2012 he worked at Pacira Pharmaceuticals. He joined CRB in 2012 as Process Engineer. Today, he is Director Advanced Therapy Medicinal Products (ATMPs).

Moderators

**Dr Sabine Hauck,**
Leukocare, Chair of the ECA ATMP Interest Group**Axel H. Schroeder,**
Concept Heidelberg, Administration Manager of the ECA ATMP Interest Group**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.



If the bill-to-address deviates from the specifications on the right, please fill out here:

Reservation Form (Please complete in full)



Genetically Modified Vaccines – Additional Guidance needed?
Live Online Training on 08 December 2021, 12.00 - 17.00 h CET

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)

CONCEPT HEIDELBERG
P.O. Box 101764
Fax +49 (0) 62 21/84 44 34

D-69007 Heidelberg
GERMANY

General terms and conditions

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:

- Cancellation until 2 weeks prior to the conference 10 %
- Cancellation until 1 week prior to the conference 50 %
- Cancellation within 1 week prior to the conference 100 %

CONCEPT HEIDELBERG reserves the right to change the materials, instructors,

or speakers without notice or to cancel an event.

If the event must be cancelled, registrants will be notified as soon as possible and will receive a full refund of fees paid. CONCEPT HEIDELBERG will not be responsible for discount airfare penalties or other costs incurred due to a cancellation.

Terms of payment: Payable without deductions within 10 days after receipt of invoice.

Important: This is a binding registration and above fees are due in case of can-

cellation or non-appearance. If you cannot take part, you have to inform us in writing. The cancellation fee will then be calculated according to the point of time at which we receive your message.

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

Wednesday, 08 December 2021,

12.00 - 17.00 h CET

Technical Requirements

For our Live Online Training Courses and Webinars, we use Cisco WebEx, one of the leading suppliers of online meetings. At <http://www.webex.com/test-meeting.html> you can check if your system meets the necessary requirements for the participation at a WebEx meeting and at the same time install the necessary plug-in. Please just enter your name and email address for the test. If the installation is not possible because of your rights for the computer 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 message.

Or you register online at www.gmp-compliance.org.

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.

Conference language

The official conference language will be English.

Ordering Recordings

Independent from the Live Online Training Courses, you can also order recordings of selected Live Online Training Courses at the same conditions at www.gmp-compliance.org/recordings. These recordings will be provided on our media server. All you need to watch it is an Internet browser – no additional software.

Organisation and Contact

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

CONCEPT HEIDELBERG

P.O. Box 10 17 64

D-69007 Heidelberg

Telefon +49(0) 62 21/84 44-0

Telefax 49(0) 62 21/84 44 34

E-Mail: info@concept-heidelberg.de

www.concept-heidelberg.com

For questions regarding content please contact:

Mr Axel H. Schroeder (Operations Director) at
+49(0) 62 21/84 44 10, or at
schroeder@concept-heidelberg.de

For questions regarding organisation please contact:

Ms Sarah Schmidt (Organisation Manager) at
+49(0) 62 21/84 44 16 or at
s.schmidt@concept-heidelberg.de.