NEW: European Conference on Bioassays/Potency Assays with European and US Agencies

PharmaLab
Analytics ▪ Bioanalytics ▪ Microbiology

Part of 12th PharmaLab Conference

- Bioassays/Potency Assays - Regulatory Requirements, Development and Routine Use
- Cell and Gene Therapies/ ATMP - Quality and Safety

Düsseldorf/Neuss, Germany
25 - 27 November 2024

Highlights

Bioassays/Potency Assays - Regulatory Requirements, Development and Routine Use

- Bioassay Design and Potency Testing for mRNA Therapeutics
- Automation of Assays – Enhancing Precision and Throughput
- Regulatory Developments for CGT, ATMP and Monoclonal Antibodies
- Bioassay Monitoring, Troubleshooting and OOS Investigations
- MoA Reflective In-Vitro Potency Testing for Vaccines
- Non-Similarity in Biological Assays

Cell and Gene Therapies/ ATMP - Quality and Safety

- EDQM Perspective
- dPCR & ddPCR
- CQAs of AAV based GT Products

Premium Sponsor 2024: charles river

CONCEPT HEIDELBERG
Pharmaceutical Quality Training. Conferences. Services.

Supported by ECA Academy
Your GMP/GDP Information Source

Objectives PharmaLab

2022 and 2023, the first two years after the pandemic, PharmaLab has attracted more participants to Düsseldorf/Neuss than ever before. With this success as a template, the 12th PharmaLab Congress will again be held on site in Düsseldorf/Neuss from 25-27 November 2024. The congress, which is aimed at employees and managers in all laboratory areas of the pharmaceutical industry, is composed of a pre-conference workshop, 7 international conferences from the fields of analytics, bioanalytics, microbiology and CGT/ATMP, as well as the accompanying exhibition. It will provide information on the latest developments in laboratory methods, systems, materials and the current status of regulatory requirements of pharmacopoeias and guidelines. In addition, experts from authorities, industrial quality control and contract laboratories will present their experiences with the use and qualification of analytical systems as well as with the validation of analytical methods and microbiological tests. Take advantage of this unique opportunity to learn about the state of the art in pharmaceutical laboratories and discuss current developments with speakers and colleagues.

Key Notes: 26/27 November

The Promise and Challenges of In Vitro and In Silico Models in Drug Development
Dr Julia Schüler, Charles River Laboratories
The presentation will highlight important developments in the drug development technology landscape influenced by the concept of 3R and the evolving legal landscape. General characteristics of the different applications, their translational relevance as well as adoption drivers will be discussed. Case studies from oncology drug development will help to elucidate these trends and their impact on future processes.

Trends & Challenges for the Development & Testing of Biotech Drug Products
Prof Dr Hanns-Christian Mahler, Chief Enablement Officer (CEO), ten23 health

The Organiser

CONCEPT HEIDELBERG – On behalf of the ECA Academy
P.O. Box 10 17 64, D-69007 Heidelberg
Phone +49 (0) 62 21/84 44-0 | Fax +49 (0) 62 21/84 44 34
E-mail: info@concept-heidelberg.de
www.concept-heidelberg.com

The Contacts:

For questions regarding content Bioassays/Potency Assays:
Axel H. Schroeder (Operations Director)
Phone: +49 (0) 6221/84 44 10,
E-Mail: schroeder@concept-heidelberg.de

For questions regarding content ATMP/CGT:
Clemens Mundo (Operations Director)
Phone: +49 (0) 6221/84 44 42,
E-Mail: mundo@concept-heidelberg.de

For questions regarding reservation, hotel, organisation, exhibition etc.:
Ronny Strohwald (Organisation Manager)
Phone: +49 (0) 6221/84 44-51
E-mail: strohwald@concept-heidelberg.de

PharmaLab Exhibition

Parallel to the conferences, participants will have the opportunity to visit the accompanying trade exhibition. It offers comprehensive information about available products, services and the latest developments around the laboratory.

All details at: **www.pharmalab-congress.com/exhibitors-plan.html**

Bioassays/Potency Assays - Regulatory Requirements, Development and Routine Use | 26/27 November 2024

Objectives

The newly developed bioassay/potency assay conference will cover both existing and a number of developing regulatory guidelines and representatives from three different European and US regulatory authorities will talk about relevant guidelines, monographs and their expectations and experiences. In addition, experts from industry, contract research organisations and CDMOs as well as scientists from fields such as cell banking, mathematics/statistics and more will present their work in the development, validation and routine use of potency assays. In addition to various modalities from classical proteins to ATMPs and vaccines, experts will provide insights into the automation of procedures and the use of AI. The conference is intended to provide a platform for scientists, experts and representatives of authorities to exchange knowledge and opinions and to discuss experiences and expectations.

Background

The number of biopharmaceutical products in the clinic and on the market continues to grow. The focus is increasingly on the area of cell and gene therapeutics or ATMPs. Many of these products are characterised by a high level of complexity. As a result, their bioactivity cannot be measured using conventional analytical tests alone. In addition to the special requirements, e.g. for product and process-related factors such as difficult upscaling, handling and testing of small and very small batches, quality of the starting materials and limited shelf life, the development of suitable potency assays is often a challenge. This includes the fact that one or more assays are usually required for each product, that there are no reference standards and that the leap from development to the GMP-regulated area is not always easy. At the same time, however, bioactivity is an indispensable CQA for release analyses and must be addressed. The MoA is also not always easy to determine, especially with CGT. The FDA also says in its guide: "*However, many CGT products have complex (e.g., rely on multiple biological activities) and/or not fully characterized mechanisms of action (MOA), making it difficult to determine which product attributes are most relevant to measuring potency. A further factor is that the regulatory background for these new products is only gradually developing and the authorisation and supervisory authorities and pharmacopoeias have only recently published or still have to draft corresponding guidelines and monographs.*"

Target audience

- Representatives of the regulatory and authorisation authorities
- Specialists for biopharmaceutical manufacturing processes
- QA/QC personnel in the biopharmaceutical environment
- Laboratory staff involved in the development and routine use of bioassays/potency assays
- Project managers and outsourcing personnel
- Biologists, analytical chemists and biochemists
- Scientists from academic fields involved in the development of biopharmaceutical products

Moderator

Dr Alexander Knorre, Eurofins

Programme

Potency Testing Approaches: Challenges and Opportunities for mRNA Therapeutics
Dr Jan Michel Falcke, BioNTech

- Biological activity of mRNA products is a complex interplay between the mRNA drug substance properties and the effective delivery system to target cells
- Provide insight into the mechanism of action for mRNA products with a focus on different analytical techniques and a respective analysis of their advantages, disadvantages and key challenges
- Share industry perspective on crucial aspects regarding quality control measures and regulatory considerations on the application of potency assays as part of the release testing of commercial mRNA products
- Explore the testing of multi-construct products, adding depth to our understanding of the complex landscape surrounding mRNA product development

Challenges with Bioassay Design for mRNA Therapeutics
Thomas Ludwig, VelaLabs

- What's the principle of a relative potency assay?
- Cell-based potency assay development and its challenges
- Qualification according to ICH Q2(R2) and defining reasonable suitability criteria for routine analytic
- Observations when measuring stability study samples

A Versatile Multiplexing Technology for Complex Drugs Characterization and Potency
Dr Rosaria Esposito, bioMérieux

- Considering the challenges with the emergence of new vaccine platforms and vaccines becoming more and more multivalent, current analytics methods are no longer adapted
- The potency testing for mRNA vaccines, such as the protein expression, is complicated due to the protein's low concentrations especially in multivalent formulations
- Studying the immune response is challenging for highly multivalent vaccines, like pneumococcal. It requires complex testing plans and contributes to increase the time to market
- We will present a protocol and data for:
  - The highly sensitive detection of expressed proteins in a multiplex cell-based potency assay
  - Multiplex serological sample testing

Enhancing Bioassay Precision and Throughput with Modular Workflow Automation
Dr Sean Lin, Eurofins

- Challenges of automation within the confines of a QC release testing environment compliant with Good Manufacturing Practice (GMP) standards
- Implementation of semi-automation in various bioassays for modular workflow automation
- Improving reliability, precision and throughput of our bioassays

Harnessing the Power of Automation for Potency Assays and for Large-Scale Potency Assay Cell Bank Production
Sheri Mahan-Hunter, Pfizer

- Automation of Potency Assays
- Transfer of automated Potency Assays to GMP Laboratories
- Introduction of Frozen Ready to Use Cells (FRTU)
- Large-Scale Cell Banking Using Automation

Advancing Potency Assay Automation
Dr Katharina Künzel, Boehringer Ingelheim

- Challenges of potency assay automation
- Automation in development and routine

Potency Assurance for Cellular and Gene Therapy Products
Dr Andrew Byrnes, FDA/CBER

- An overview of FDA's draft guidance on potency assurance for cellular and gene therapy products
- Advice on potency assays
- Other aspects of potency

Potency Assays as Part of Release Testing for ATMPs – Focus on AAV
Dr Christoph Mück, AGES

- How is potency defined?
- Requirements for potency assays
- Potency assays for adeno-associated virus ATMPs
- Challenges in assay development

Development of Methods for Comparative Analysis of the Potency of Monoclonal Antibodies
Dr Lilija Miller, Paul-Ehrlich Institut

Growing number of monoclonal antibodies leads to a large number of potency assays

- In case of e.g. counterfeiting or theft of monoclonal antibodies, the Paul-Ehrlich-Institut (and also other authorities) cannot perform ad hoc potency testing due to lack of expertise/experience and the large number of possible potency assays
- In a BMG-funded project, comparative analyses of the potency of monoclonal antibodies are to be established and thus ideally reduce the number of potency assays to be kept in stock

Qualification of Analytical Cells for GMP Potency Assays – Guidance from a Different World
Dr Oliver Wehmeier, acCELLerate

Partial Dose-Response Curves - Contributions to the Discussion on "Allowed" Non-Similarity in Biological Assays –
Dr Ralf Stegmann, Stegmann Systems

- Impact of accepted non-similarity on relative potency estimation
- Revising margins and criteria for acceptable non-similarity
- Simulation Studies: Illustrating impact and risks through simulations
- Practical implications and risks of non-similarity acceptance

Trending and AI Prediction for Improving of Assay Performance
Dr Jan Amstrup, Novo Nordisk

- Each bioassay is a detective story, generating data en masse
- Showing how these data can be used constructively
- Using Random Forest algorithm to create a tool that can predict the likelihood of a given sample to pass the assay system suitability tests
- Establishment of an assay trending dashboard

Continuous Bioassay Monitoring and Troubleshooting in QC, 3 Case Studies
Dr Steffen Pahlich, Novartis

- Cell-based potency assay are complex methods for the determination of the biological activity of Biologics
- In a commercial QC environment, continuous assay performance monitoring and trending of relevant assay parameter is key to ensure the methods remains in a state of control
- In this talk, case studies about bioassay performance and related troubleshooting based on trending data will be presented

Unique Aspects of Bioassay OOS Investigations
Dr Robert de Lange, Roche

- Guidances
- Retesting within a full-scale Bioassay
- OOS investigation
- Outliers
- Examples

MoA Reflective In-Vitro Potency Testing for Vaccines
Dr Sascha Karassek, Charles River Laboratories

- In vitro vaccine potency testing aligned with 3R
- Case study
- Validation considerations
- Stability indicating properties

Implementation of Concepts from ICH Q14 into Practice - Case Study for a Cell-Based Assay
Dr Simon Anderhub, Novartis

- ICH Q14 provides opportunities for enhanced flexibility during the commercial lifecycle
- As a trial, elements of ICH Q14 were implemented for a bioassay. This included establishing an Analytical Target Profile, conducting parameter risk assessments and proposing established conditions
- This exercise enhanced our understanding of ICH Q14's practical implications and helped to identify areas of improvement in our development processes

The Social Event

On the evening of the first congress day, on 26 November 2024, all congress delegates and speakers are invited to a „Get together" in the Congress Center. Take advantage of this opportunity for an information exchange and enjoy the laid-back atmosphere and the entertainment programme.

Speakers

Dr Jan Amstrup
Novo Nordisk, Principal Scientist
CMC Bioanalysis, DP & Analytical Development

Dr Lilija Miller
Paul-Ehrlich Institut – German Federal Agency for Vaccines and Biomedicines
Deputy head of department and laboratory head (bioassay laboratory)

Dr Sean Lin
Eurofins BPT Munich
Scientist / QC manager for the Biologics and Bioassay Testing – Development & Validation department

Dr Sascha Karassek
Charles River Laboratories
SME Bioassay

Dr Robert de Lange
Roche
Senior QC Expert (Bioassays)
Analytical Science, End to End Product Quality

Dr Oliver Wehmeier
acCELLerate
Managing Director

Dr Steffen Pahlich
Novartis
QC Head BDSS Basel

Dr Simon Anderhub
Novartis
Senior Expert Science & Technology

Dr Jan Michel Falcke
BioNTech
Director global AS&T

Dr Andrew Byrnes
FDA/CBER
Director of the Division of Gene Therapy 1 at FDA's Center for Biologics Evaluation and Research.

Thomas Ludwig
VelaLabs
Head Cellbased Assay Group

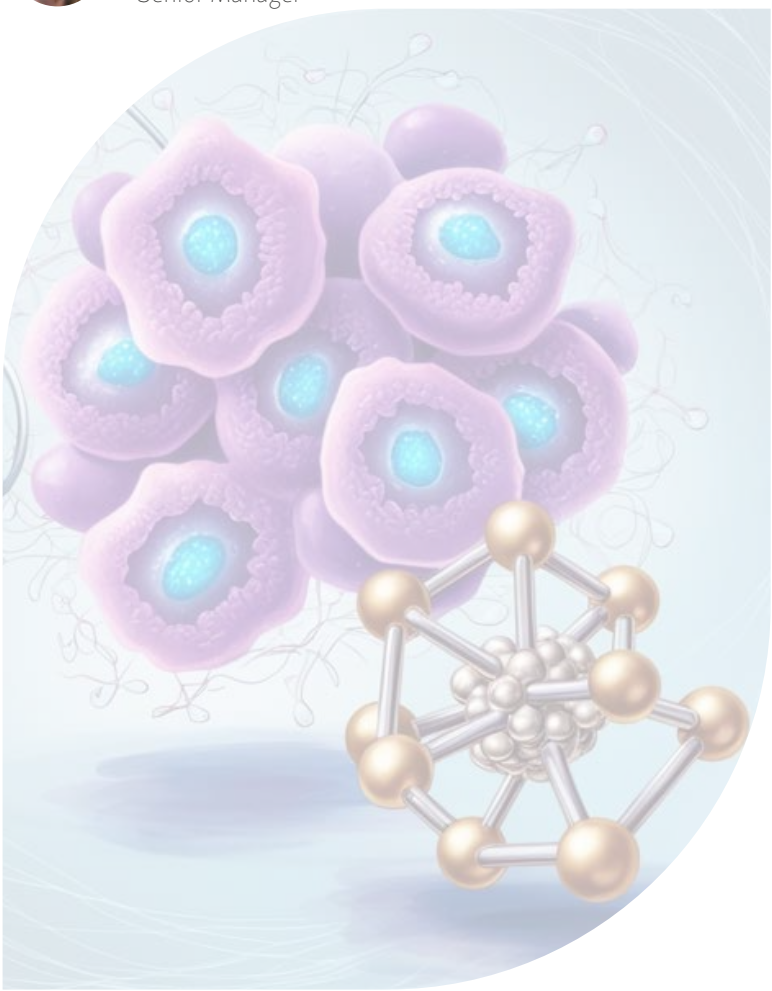
Dr Rosaria Esposito
bioMérieux
Global Field Scientist

Dr Ralf Stegmann
Stegmann Systems
CEO

Dr Christoph Mück
AGES
Quality assessor for biologics

Dr Katharina Künzel
Boehringer Ingelheim
Scientist in a potency assay laboratory of the department Analytical Development Biologicals

Sheri Mahan-Hunter
Pfizer
Senior Manager



Media Partners 2024:

European Biotechnology
LABORWELT
transkript
BioPharma Asia
rapid microbiology
GIT LABOR-FACHZEITSCHRIFT

Cell and Gene Therapies/ATMP Conference
26/27 November 2024

Objectives

This meeting is aimed at manufacturers and developers of cells, tissues, cell- and tissue-based products or ATMPs and deals with microbiological and analytical quality requirements, suitable methods and test systems and their implementation and validation. Representatives from authorities and colleagues from small-scale and industrial manufacturing and consulting will explain the current regulatory requirements and share their knowledge.

Background

Modern regenerative medicine systems such as cells and tissues or ATMPs (gene therapeutics, somatic cell-based products and tissue-based products) represent an innovative group of medicinal products that is becoming increasingly important. With the entry into force of several regulatory directives, e.g. the European Directive EC 1394/2007 for ATMPs, such products have been classified as medicinal products and as such must comply with EU requirements for medicinal products. Although the biopharmaceutical industry has significantly intensified its activities in this area, many of these products are developed and manufactured at universities, hospitals and in small and medium-sized enterprises. These university or medical roots lead to special challenges for the respective institutions as well as for the regulatory authorities in meeting compliance requirements for quality, safety and GMP aspects and approval. The frequently given manufacturing conditions also contribute to this, e.g. the open manipulation of cells and tissues necessary for obtaining such products on a medical-surgical level, or the short shelf life of the obtained end product. And potentially there are always conflicts when it comes to the relevance of different guidelines, e.g. when an Annex 1, or an Annex 2 or a WHO Guideline does not harmonize with the ATMP Guideline.

Target audience

- Representatives of the regulatory and authorisation authorities
- Specialists for biopharmaceutical manufacturing processes
- QA/QC personnel in the biopharmaceutical environment
- Project managers and outsourcing personnel
- Analytical Experts

Moderator

Dr Sabine Hauck, Chair of the ECA ATMP Board
Dr Ulrike Herbrand, Charles River Laboratories

Programme

Efficient Microbial Control Concepts for ATMPs
Dr Holger Kavermann, Roche

- Differences in microbial control strategies between traditional biotech products and ATMPs
- Efficient control for low batch yield ATMP production processes
- Bioburden and Sterility Testing Requirements for IPC, Drug Substance and Drug Product

Critical Quality Attributes of AAV based GT Products
Dr Roland Pach, Roche

- Industrial perspective on the determination of CQAs
- Impact on techniques and strategies
- Product related impurities and potency

A ddPCR Method for Multiplex Determination of AAV Genome and Vector Titer
Dr Christian Schiller, Eurofins

- Parameters for quality control of AAV vectors
- Basic principles of ddPCR technology
- Qualification results of the multiplex assay for AAV genome and vector titer determination

Microbial Control for ATMP Facilities
Cecilia Pierobon, Steris

- Regulatory requirements for ATMPs
- Disinfection Technologies
- Disinfectant Selection and Efficacy
- Case study

Key Insights about CAR-T Therapy from Concept to Clinic
Dr Daniela Rozkova, SCTbio

- From tech transfer to clinical production
- Navigating challenges & innovations in Autologous CAR-T Cell Therapy
- Lessons learned

Digital PCR Applications for Cell and Gene Therapy – Standardization for a High-Quality Process development
Dr Andreas Hecker, QIAGEN

- QIAcuity in Biopharma: workflow benefits and CGT applications that can be addressed with dPCR
- Standardized and reproducible AAV sample processing and vector titer determination
- Analysis of genome integrity and stability of AAV vectors using digital PCR
- High sensitivity residual host cell DNA quantification
- Fast, sensitive, and compliant Mycoplasma dPCR workflow

In-Process and Release Testing of Cell Therapy Applications
Caroline Paeschke, Minerva

- Short introduction into digital PCR
- Overview of required QC tests
- Determination of vector copy number in CAR-T cell therapeutics
- Assay design and development

European Pharmacopeia Perspective
Dr Solène Le Maux, EDQM

- Activities of the Ph. Eur. in the ATMP field
- Focus on the new approach to gene therapy texts
- Review of the cell-based preparations chapter under elaboration
- Updates on alternative rapid microbiological methods

Test for Microbial Purity on MCBs
Christine Weiß, Labor LS

- What are the requirements for the test?
- Challenges and stumbling blocks during test performance

What is the Value of Design-of-Experiment Approaches in the Development of Cell-based Potency Assays?
Dr Johannes Solzin, Boehringer Ingelheim

- Comparison of different optimization strategies and different DoE-approaches for bioassays within development of biopharmaceuticals
- How to minimize number of experiments and maximize statistical power of a DoE
- Choosing the right response for DoE: Which parameter(s) describe the performance of a bioassay?
- Case studies of assay development: from potency assays for antibodies to new infectious titer assays for viruses-based ATMPs

Implementation of ICH Q14 and USP <1220>: A challenge in the Highly Competitive mRNA Vaccine Field
Dr Isabelle Moineau, AKTEHOM & Dr Marc Francois-Heude, Sanofi

- Implementation of an optimized Life Cycle Management process for analytical methods
- Case study: design of analytical methods to Ongoing Procedure Performance Verification

Continuous Microbial Monitoring in ATMP Facilities in Compliance with the New EU GMP Annex1
Dr Emad Albarouki, PMS

- Microbial monitoring in line with the new GMP Annex1
- Continuous microbial monitoring in ATMP aseptic processing is a key element.
- Standard microbial sampling (active vs. passive)
- Rabid Microbial Monitoring (RMM) as a potential alternative microbial Sampling method
- Continuous active air monitoring is a solution in grade A aseptic filling

Spilling the Tea on a Robust CCS for ATMPs
Marsha Steed, Steed MicroBio

- Establishing a CCS for ATMPs
- Are there regulations for CCS for ATMPs?
- Material transfer contamination controls for ATMPs
- Use of isolators for ATMP manufacturing
- Particulates in ATMP products and impact of single use systems

Speakers

Dr Holger Kavermann
Roche, Head of QC Department for Microbiology, EM and Cleaning Validation

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

Dr Christian Schiller
Eurofins, Group Leader at Eurofins BioPharma Product Testing

Cecilia Pierobon
Steris, Technical Services Manager

Dr Daniela Rozkova
SCTbio, Chief Technology Officer

Dr Andreas Hecker
QIAGEN, Associate Director Global Product Management

Caroline Paeschke
Minerva Biolabs
Product Management

Dr Solène Le Maux
EDQM, Scientific Programme Manager

Christine Weiß
Labor LS, Head of department Special microbiological studies / KBT

Dr Johannes Solzin
Boehringer Ingelheim, Laboratory Head, Senior Principal Scientist

Dr Isabelle Moineau
AKTEHOM, Analytical Expert Consultant

Dr Marc Francois-Heude
Sanofi, Head of biochemistry and biophysics mRNA unit

Dr Emad Albarouki
PMS, Field Application Specialist for Micro & Sterility

Marsha Steed
Steed MicroBio, Sterility Assurance Expert Sr. Consultant/Founder & President

BOOK ONLINE NOW !

At www.pharmalab-congress.com/registration-congress.html or use the QR code on the right.

To avoid incorrect information, please give us the exact address and full name of the participant.

- **25 November 2024: Pre-Conference - 590 € plus VAT**
- **26+27 November 2024: PharmaLab Congress & Exhibition (day 1 + 2) - ~~1.380 €~~ 1.180 €* plus VAT**
- **26 November 2024: PharmaLab Congress & Exhibition (day 1 only) - ~~690 €~~ 590 €* plus VAT**
- **27 November 2024: PharmaLab Congress & Exhibition (day 2 only) - ~~690 €~~ 590 €* plus VAT**

***Early Bird Discount until 16 September 2024!**

Conference Language: The official conference language will be English.

Particularities of the PharmaLab Congress:

With a one-day ticket/two-day ticket for the PharmaLab Conferences (26/27 November 2024) you can attend any conference offered that day/both days. It includes participation in any conference on that day/on both days and the visit of the exhibition. In addition, it comprises lunch and beverages during the conferences and in breaks (on one or both days) as well as the social event on the evening of the first congress day.

Please mark if you would like to attend the Social Event.

To be able to prepare the conference rooms, please choose the conference you are most interested in during the online registration process.

Content last updated:
The status of the content is as of **02.09.2024**.
The latest content can be found on the PharmaLab website at https://www.pharmalab-congress.com.

Please note

There will be no hotel/ room reservations via Concept Heidelberg. Please book your hotel room directly with the reservation form which you will receive together with your confirmation/invoice! Charges are payable after receipt of the invoice. There will not be any print-outs at the Congress. Instead you will receive all presentations prior to the Congress as downloads. presentations of this Course will be available for download and your print-out one week before the conference.

Please note that no printed materials will be handed out on site and that there will not be any opportunity to print the presentations on site. After the event, you will automatically receive your certificate of participation.

Stay informed with the GMP Newsletters from ECA

The ECA offers various free of charge GMP newsletters for which you can subscribe to according to your needs.
Go to www.gmp-compliance.org/gmp-newsletter