Breakfast

06:30 - 08:30 Monday, 16th October, 2023
Panorama Restaurant

Breakfast included for hotel guests that booked in the CASSS block.

Registration

07:30 - 08:30 Monday, 16th October, 2023
Galleria Hall

Registration will be open 07:30 - 12:00 and 13:15 - 17:00.

EFPIA Biomanufacturing Group Satellite Session Part I

08:30 - 09:30 Monday, 16th October, 2023
Auditorium
Fionnuala O'Driscoll, Helen Newton, Karoline Bechtold-Peters

The EFPIA Biomanufacturing Working Group is a cross-company industry team working to aid the development of biological products for patients. Through areas of special interests, the group supports and develops cutting edge science and technology strategies. In the first half the session the working group will showcase some of the current concept papers under development.

The second session will cover the topic of “Immunogenicity”. Immunogenicity of biologic agents is a topic we have been dealing with for a long time. The mechanisms of initiation are complex and the consequences clinically relevant. With novel modalities we are approaching new fields beyond the classical antibodies. Several years of studies in the ABIRISK IMI Consortium have yielded important insights that shed new light on the determinants of immunogenicity, better understand its molecular origin, and improve tests to predict the likelihood that a molecule will trigger an immune response.

The Satellite Symposium will present modern in silico and in vitro assays for characterizing immunogenicity and the limitations of these methods. These can be better standardized than immunogenicity studies in humans with sometimes very broad ranges for the expression of anti-drug antibodies. Furthermore, humanized animal models are now available, which also allow repeated administration of a therapeutic antibody.

Besides the structure and specific epitope regions of the antibody, post-translational changes such as oxidation but especially the formation of higher molecular species have been described as triggering factors for immunogenicity. A presentation will highlight the mechanism of aggregate formation and recent prediction possibilities. In contrast, in the case of vaccines, immunogenicity is desired and must be considered a CQA in terms of potency. Synergistic considerations of the biologics world and the vaccine world are encouraged here.

Overall, this session will also discuss how a Patient Centric Specification for higher molecular weight species and aggregates can be defined by in vitro and in vivo models as well as scientific understanding and simulations, without a mandatory and sole clinical qualification in human studies.

Session Speakers:

Welcome & Introduction to the EFPIA MQEG Biomanufacturing Satellite Session

Markus Goese, F. Hoffmann-La Roche Ltd.

Clonality, Characterisation and Viral Safety of Cell Lines

Elodie Charbaut Taland, *Merck KGaA*

Antibody Drug Conjugates

Nienke Vriezen, *Byondis BV*

Agile Aseptic Manufacturing – Advancements Towards Flexible Decentralized Manufacturing of Biologics Applying a Control Site Concept and Rapid Environmental Monitoring

Karoline Bechtold-Peters, *Novartis Pharma AG*

EFPIA Survey Output – Quality Strategies for Expedited Access

Diane Wilkinson, *AstraZeneca*

Satellite Session Part I: Panel Discussion - Q&A

09:30 - 10:00 Monday, 16th October, 2023
Auditorium
Karoline Bechtold-Peters

Additional Panelists:

Seán Barry, *HPRA-Health Products Regulatory Authority*

Klara Tiitso, *European Medicines Agency*

Networking Break

10:00 - 10:30 Monday, 16th October, 2023
Galleria Hall

Satellite Session Part II

10:30 - 11:45 Monday, 16th October, 2023
Auditorium

Session Speakers:

The Molecular Landscape of Anti-Drug Antibodies Following Treatment with Biologics

Yariv Wine, *Tel Aviv University*

In Vitro Approaches to Assess the Immunogenicity Potential of Biotherapeutic Drug Candidates

Hannah Morgan, *Novartis*

Outcomes and Take Homes of the ABIRISK Consortium

Sebastian Spindeldreher, *Integrated Biologix*

Clinical Perspectives on Immunogenicity of Biotherapeutics

Florian Deisenhammer, *Innsbruck Medical University*

How to Connect Patient Centric Specifications with Immunogenicity of Biotherapeutics - A Regulator's Perspective

Mats Welin, *Swedish Medical Products Agency*

Satellite Session Part II: Panel Discussion - Q&A

11:45 - 12:25 Monday, 16th October, 2023
Auditorium

Additional Panelist:

Steffen Gross, *Paul-Ehrlich-Institut*

Concluding Remarks

12:25 - 12:30 Monday, 16th October, 2023
Auditorium

Networking Lunch

12:30 - 13:45 Monday, 16th October, 2023
Panorama Restaurant

CASSS Welcome and Introduction

13:45 - 14:00 Monday, 16th October, 2023
Auditorium

Welcome to the 17th European CMC Strategy Forum

Session I: The Art of Specification Setting

14:00 - 15:20 Monday, 16th October, 2023
Auditorium
Sandra Auguste-Bowler, Teresa Pepper, Mats Welin

The fundamental principles for setting specifications as described in ICH Q6A/6B from 1999 are still relevant, but now need updating to also accommodate the new and evolving technologies such as continuous manufacturing, new analytical technologies and Real Time Release Testing (RTRT) in lieu of drug substance, drug product and stability testing parameters. Apart from the traditional approaches to setting specifications based on batch data, there is also a need to include alternative approaches to setting specification, especially in light of the accelerated development and regulatory pathways where too few batches are available for setting meaningful specifications. The following topics will be discussed with examples in this session to explore the issues that could be addressed in the updates to the ICH specification guidelines:

1. Setting specifications based on batch data versus specifications based on clinically meaningful outcomes.

2. In absences of product specific clinical data supporting acceptance criteria that are sufficiently wide to cover product variability, what tools are there to go beyond what has been used in clinical trials and still be able to verify that the product is safe and efficacious?

3. How to set acceptance criteria for products where there is an unmet clinical need (e.g. PRIME products) where only limited clinical data are available and very few batches have been used in clinical trials.

4. Is there a role of statistical and modelling approaches in the setting of acceptance criteria?

5. Use of new analytical technologies (PAT), NIR and MS techniques in real-time release instead of end-product specifications?

6. Setting of specifications for biological-chemical products, e.g. ADCs, oral formulations of peptides/biologicals.

7. How to link the setting of specifications with other relevant ICH guidance.

Session Speakers:

Specifications and Lifecycle Management

Theresa Ahern, *Eli Lilly and Company*

Using Platform Approaches with Vaccines to Support Rapid Development and Launch

Cristiana Campa, *GlaxoSmithKline*

Extending a Commercial Specification Beyond the Clinically Qualified Limits, How to Overcome the Challenges

Vanessa Auquier, *UCB Pharma S.A.*

Networking Break

15:20 - 15:45 Monday, 16th October, 2023
Galleria Hall

Session I: Panel Discussion - Q&A

15:45 - 17:00 Monday, 16th October, 2023
Auditorium

Additional Panelists:

Seán Barry, *HPRA*

Willie Wilson*, CDER, FDA*

Andrew Chang, *Novo Nordisk*

Welcome Reception and Networking Dinner

18:00 - 22:00 Monday, 16th October, 2023
Vasa Museum

Join us for a memorable evening at the Vasa Museum including dinner and a private tour. Meet in the lobby by 18:00 to take the provided buses.

Breakfast

06:30 - 09:00 Tuesday, 17th October, 2023
Panorama Restaurant

Breakfast included for hotel guests that booked in the CASSS block.

Registration

08:00 - 09:00 Tuesday, 17th October, 2023

Galleria Hall

Registration will be open until 17:00

Session II: Characterizing and Controlling Modes of Action

09:00 - 10:30 Tuesday, 17th October, 2023
Auditorium
Tara Sanderson, Ilona Reischl, Mihaela Buda

Establishing methods that demonstrate control of a drug's mode of action and providing sufficient characterization are amongst the most critical assays required on the specification. They also represent some of the most complex assays to develop and validate, especially for complex biologics such as ATMPs, ADCs and viruses. This session will discuss approaches and challenges to establishing effective methods used for the characterisation and control of a drug's mode of action. With presentations and panellists from EU health authorities and the FDA, we will examine the regulatory expectations for establishing functional assays from early development of the product through to marketing authorisation (including the use of surrogate methods) for different types of biological molecules. We will also present current ways of working and case studies from industry focusing on the challenges from moving from early development assays to late phase and commercial cell-based assays and the complexities of establishing potency methods for complex molecules such as ATMPs.

Session Speakers:

Critical Requirements for Potency Assays

Markus Tomek, *AGES*

Moving From mAbs to rAAV: How Long Is the Journey to Assess Product Potency? Focus on Differences/challenges That Spice Up the Potency Road to Gene Therapy.

Gaël Debauve, *UCB Pharma S.A.*

In Vitro Potency Assay for Follitropin Alfa: a Case Study of Worldwide (Ongoing) Registration

Morgane Rochemont, *Merck & Co., Inc.*

Considerations on mRNA Products Potency Testing

Pawel Widomski, *BioNTech*

New Member Networking Break

10:30 - 11:00 Tuesday, 17th October, 2023
Galleria Hall

Networking Break

10:30 - 11:00 Tuesday, 17th October, 2023
Galleria Hall

Session II: Panel Discussion - Q&A

11:00 - 12:15 Tuesday, 17th October, 2023
Auditorium

Additional Panelists:

Andreea Barbu, *MPA, Sweden*

Katrin Buss, *BfArM Germany*

Networking Lunch

12:15 - 13:45 Tuesday, 17th October, 2023
Panorama Restaurant

Session III: Antibody Drug Conjugates (ADCs)

13:45 - 15:05 Tuesday, 17th October, 2023
Auditorium
Helen Newton, Vanja Cankovic, Kristina Martinell

Antibody drug conjugates (ADCs) are by nature complex molecules with inherent structural heterogeneity. ADCs draw the small molecule and biologics worlds together, creating a unique, qualitatively different modality. One of the particular challenges this modality brings about is the successful resolution of product characteristics, especially the ones relevant to the mechanism of action MOA) and consequently to clinical performance. ADCs are designed to work through cytotoxicity elicited by the toxic payload linked to the antibody, but other MOAs can, and do contribute to the clinical efficacy. Qualification and quantification of MOAs are challenging and are linked to setting the proper control strategy that can be placed at several levels.

To further add to the complexity, there are different drug linker chemistries and conjugations methods that can be tailored to specifically address a therapeutic need, but the complexity of combinations might also preclude a unique view on all ADCs.

This session will, on one hand focus on specific challenges that the industry is facing during development – and on the other hand on the challenges regulators face when reviewing the dossiers. By approaching the topic from both sides, it will give the audience a comprehensive picture on ADCs, that are more and more, taking the central stage across many therapeutic indications.

Session Speakers:

Development of Antibody Drug Conjugates as Specific and Potent Medicinal Products - A Regulatory Perspective

Steffen Gross, *Paul-Ehrlich-Institut*

Could One Size Fit All - Why Not?

Kavita Aiyer, *Seagen Inc.*

Overarching Platform Control Strategies and Reducing Repeated Testing

Colm Reddington, *AstraZeneca*

Networking Break

15:05 - 15:30 Tuesday, 17th October, 2023
Galleria Hall

Session III: Panel Discussion - Q&A

15:30 - 16:45 Tuesday, 17th October, 2023
Auditorium

Additional Panelists:

Martijn van der Plas, *MEB-Medicines Evaluation Board*

Damian Ittig, *Swissmedic*

Breakfast

06:30 - 09:00 Wednesday, 18th October, 2023
Panorama Restaurant

Registration

08:00 - 09:00 Wednesday, 18th October, 2023
Galleria Hall

Registration will be open until 17:00 pm

Session IV: Stability

09:00 - 10:25 Wednesday, 18th October, 2023
Auditorium
Heli Suila, Joan Malmstrøm, Ronald Imhoff

Stability testing is one of the major parameters needed, to provide evidence for the quality of a drug substance or drug product. Real time real condition stability data fully covering the period of the claimed shelf life is currently requested for biological products. To accelerate development there is a need to develop new approaches for stability strategies using modern analytical technologies and tools. Increasing attention is focusing on the use of platform data/prior knowledge which reduces the requirement for product specific data.

This session will focus on:

1. The use of prior knowledge to support clinical trial application.

2. In-use stability requirements (across regions)

Session Speakers:

Best Practices for Design and Performance of In-Use Stability and Compatibility Studies

Jonas Fast, *F. Hoffmann-La Roche Ltd.*

and

Jing Liu*, Seagen Inc.*

Advanced Kinetic Modelling: A Universal Tool for Stability Predictions of Biotherapeutics and Vaccines

Sreejith Ramesh*, Sanofi*

Stability Predictions for mAbs Using Arrhenius-Based Kinetics

Mitja Zidar, *Novartis*

Leveraging Prior Knowledge to Support Early Phase Clinical Trial Application: Regulatory CMC Considerations and Case Studies

Scott Roberts, *Novo Nordisk*

Networking Break

10:25 - 10:45 Wednesday, 18th October, 2023
Galleria Hall

Session IV: Panel Discussion - Q&A

10:45 - 12:00 Wednesday, 18th October, 2023
Auditorium

Additional Panelists:

Niklas Ekman, *Finnish Medicines Agency (FIMEA)*

Andrew Lennard, *Amgen Limited U.K*

Networking Lunch

12:00 - 13:15 Wednesday, 18th October, 2023
Panorama Restaurant

Session V: Collaborative Approaches and Reliance Procedures

13:15 - 14:35 Wednesday, 18th October, 2023
Auditorium
Seán Barry, Diane Wilkinson, Kowid Ho

Global regulatory filings are becoming increasingly challenging, at both initial submission, as well as in the post approval lifecycle. The regulatory submission for a given medicinal product must be approved independently by numerous national agencies, which can lead to delays in timely access for patients. Therefore, there is a pressing need to increase the efficiency in the global regulatory approval process. One way this can be achieved is through closer collaboration and reliance between international regulatory agencies. Several international collaborative approaches have been used to great effect in recent years, for example Project OPEN, Project ORBIS, ACCESS, the WHO Collaborative Registration Procedure and others.

In this session we will explore the current landscape of collaborative and reliance approaches in the context of CMC submissions. We will get the perspective from both industry and regulators on the benefits and challenges of recent pilot programs such as the ICMRA collaborative assessment pilot, and their thoughts on the future landscape for these innovative approaches.

Session Speakers:

EMA Perspectives on International Convergence and Collaboration for CMC Submission

Klara Tiitso, *European Medicines Agency*

The Future of Collaborative Regulatory Approaches, An Industry Perspective for CMC Data

Frank Montgomery*, AstraZeneca*

ICMRA Pilot - Regulatory View

Willie Wilson, *CDER, FDA*

Industry Experience With the ICMRA Pilot on Collaborative Assessment of PACs: Paving the Way Towards Reliance

Sylvie Meillerais, MSD and Christine Wu, *Genentech, a Member of the Roche Group*

Networking Break

14:35 - 15:00 Wednesday, 18th October, 2023
Galleria Hall

Session V: Panel Discussion - Q&A

15:00 - 16:00 Wednesday, 18th October, 2023
Auditorium

Additional Panelists:

Samvel Azatyan, *World Health Organization*

Janis Bernat, *International Federation of Pharmaceutical Manufacturers and Associations (IFPMA)*

Closing Remarks & Invitation to the CMC Strategy Forum 2024

16:00 - 16:15 Wednesday, 18th October, 2023
Auditorium