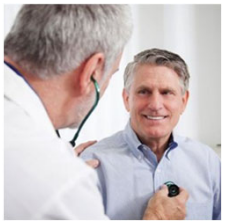
European Federation of Pharmaceutical Industries and Associations

Welcome & Introduction to the EFPIA Manufacturing and Quality Expert Group (MQEG) - Biomanufacturing Satellite Session

Markus Goese, F. Hoffmann-La Roche Ltd, on behalf of EFPIA MQEG Biomanufacturing subteam






CASSS CMC Strategy Forum EU

Stockholm – Oct. 16, 2023

Welcome to Stockholm!

Source: https://www.visitstockholm.com/

Presentation Outline

1. Welcome & a few facts about EFPIA

2. Highlights of EFPIA MQEG Biomanufacturing team's achievements & Outlook 2023-2024

3. Agenda of this year's MQEG Biomanufacturing Satellite Session at CASSS

REPOSITIONING INDUSTRY AS A PARTNER IN HEALTHCARE

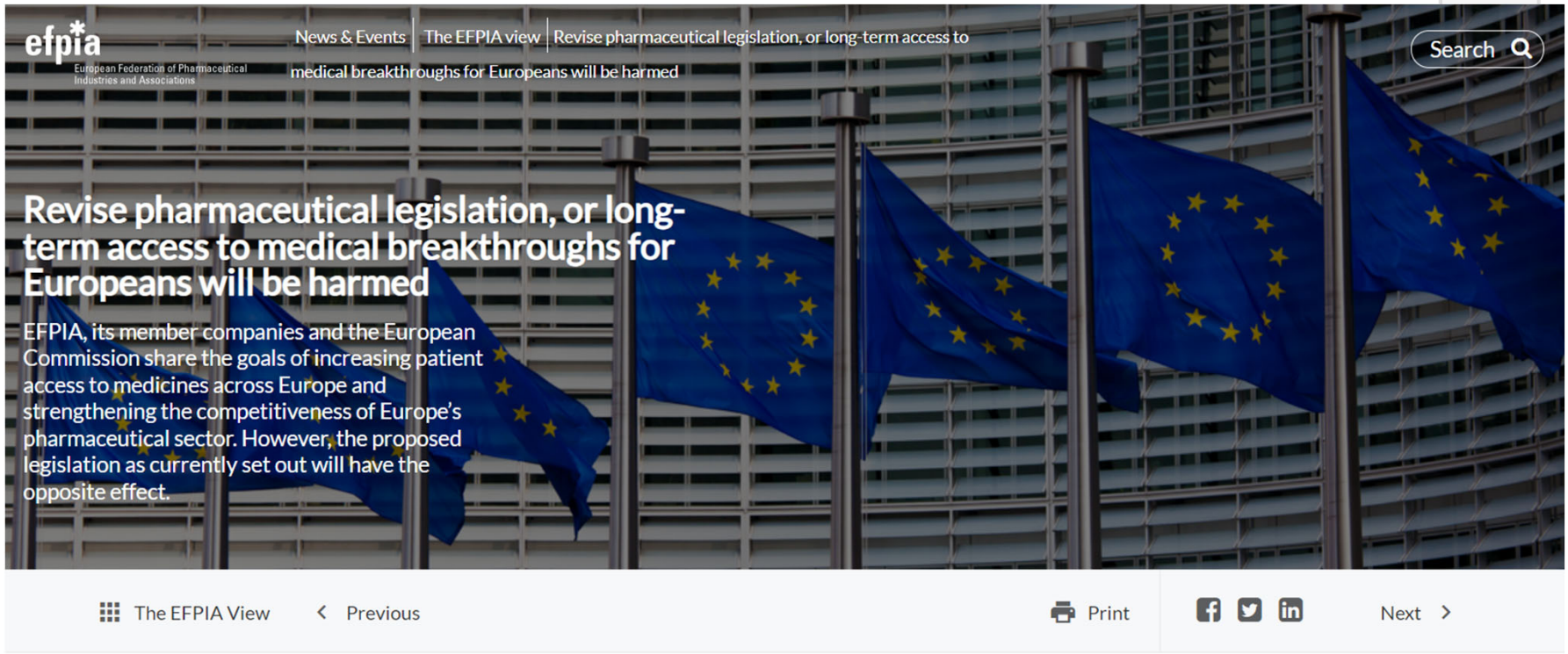
#WeWontRest

About EFPIA


56
staff members
38
member companies
37
member associations
17
SMEs
36
partners in research
15
Vaccines Europe
An industry for healthy lives
Members

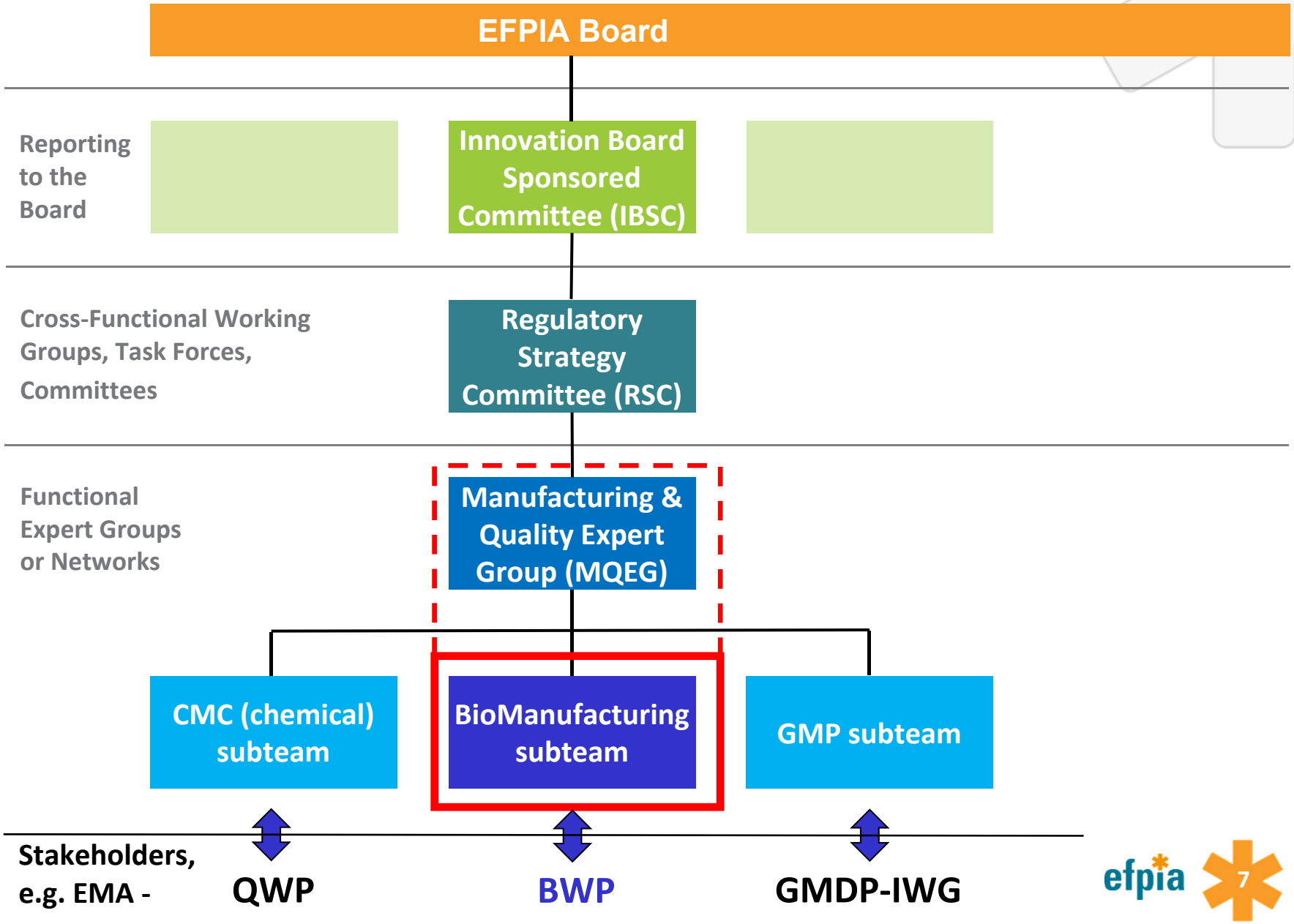
New “EU Pharma package” published in April 2023

EFPIA strongly involved in calling for revisions



> Revise pharmaceutical legislation, or long-term access to medical breakthroughs for Europeans will be harmed

Simplified EFPIA Governance & MQEG group



EFPIA MQEG BIOMANUFACTURING subteam

Some key activities 2023-2024

ICH support:

- ICH Q1/Q5C Stability revision
- ICH Q3E Extractables & Leachables
- ICH Q5A(R2) Viral safety
- ATMPs: CMC and GMP aspects (in new ICH CGTDG)

Interactions with EU regulators:

- BWP IP meeting, QIG Lessons Learned Focus Group (LLFG)

Work on industry position papers:

- Expansion of EU Masterfile Concept
- Antibody-drug conjugates (new team)
- Clonality, characterization & viral safety of cell lines (NGS & others)
- Multi-Attribute Method (MAM) by MS in QC
- Risk-based setting of sterile filtration bioburden limits
- Polysorbate

EFPIA integral to ICH successes

efpia leadership

- 60 experts on ICH working groups
- Hosted 1st meeting in Brussels
- 1 of 6 founding members
- 2 Management Committee seats

EFPIA MQEG and its subteams support all nominated EFPIA experts in all active ICH Q & M (where relevant) working & discussion groups

Example for EFPIA Input into EU Pharma Leg. revision

Further expand the Masterfile concept to fully enable manufacturing innovation

- EFPIA has published together with Vaccines Europe and CEPI a position paper entitled "**Expanding Master Files for human medicinal products in the EU/EEA**" which describes in detail the status quo and much needed enhancements to the Masterfile system in the EU.

efpia
European Federation of Pharmaceutical Industries and Associations

CEPI

Vaccines Europe
An industry for healthy lives

Expanding Master Files for human medicinal products in the EU/EEA

Executive Summary

Sponsors for Marketing Authorisation Applications of biological medicinal products (e.g. recombinant proteins, advanced therapy medicinal products, vaccines) frequently rely on collaboration with third party manufacturers to source components required to produce new, innovative medicines. These materials often have intellectual property held by the third-party suppliers, however, the current European regulatory framework has little capacity to protect proprietary confidential information between collaborating parties for biologicals, whereas small, synthetic molecule products have tools such as Active Substance Master Files with 'open' and 'closed' parts to protect IP. Other Master File tools currently exist in the EU, for vaccines with the Vaccine Antigen Master File (VAMF), the recent veterinary vaccine Platform Technology Master File (vPTMF), and for plasma-derived products with the Plasma Master File (PMF).

EFPIA Position: A further expansion of the master file concept to include platform technology master files would enable a world-leading regulatory framework for new pharmaceutical manufacturing technologies in Europe.

EMA-BWP IP Meeting Sept 6, 2023 - Final agenda extract

EFPIA MQEG Biomanufacturing subteam once more active with significant contributions

Agenda – BWP Interested Parties Meeting

6 September 2023 (13:30 – 17:00) – WebEx (virtual)

Time	Description
13:40-13:50	BWP Priorities and update on working parties reorganisation
13:50-14:15	**Industry experience with PRIME Toolbox** • Presentation from EFPIA • Discussion
14:15-14:40	**Towards a Risk-based Approach to Process Validation for Biotechnology Products, including ATMPs** • Presentation from EFPIA • Discussion
15:20-15:45	**Principles and approaches for clinically relevant/"patient-centric" specifications** • Presentation from EFPIA/Vaccines Europe • Discussion
16:10-16:35	**Innovation and Regulatory Flexibility to Achieve Climate, Environment and Sustainability Goals for Biological Products** • Presentation from EFPIA • Discussion

Biomanufacturing/ QC by MAM team published two important papers in peer-reviewed journal

ELSEVIER

European Journal of Pharmaceutics and Biopharmaceutics

Volume 191, October 2023, Pages 57-67

Research paper

Compliance and regulatory considerations for the implementation of the multi-attribute-method by mass spectrometry in a quality control laboratory

Annick Gervais [a], Eef H.C. Dirksen [b], Thomas Pohl [c], Karoline Bechtold-Peters [d], Will Burkitt [e], Valerio D'Alessio [f], Simone Greven [g], Andrew Lennard [h], Xue Li [i], Christopher Lössner [j], Ben Niu [k], Dietmar Reusch [l], Tomás O'Riordan [m], Justin W. Shearer [n], David Spencer [o], Wei Xu [p], Linda Yi [q]

Abstract

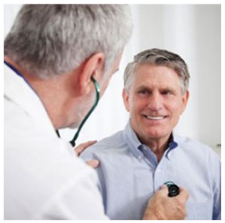
Multi-attribute methods employing mass spectrometry are applied throughout the biopharmaceutical industry for product and process characterization purposes but are not yet widely accepted as a method for batch release and stability testing under the good manufacturing practice (GMP) regime, due to limited experience and level of comfort with the technical, compliance and regulatory aspects of its implementation at quality control (QC) laboratories. This article is the second part of a two-tiered publication aiming at providing guidance for implementation of the multi-attribute method by peptide mapping liquid chromatography mass spectrometry (MAM) in a QC laboratory. The first part [1] focuses on technical considerations, while this second part provides considerations related to GMP compliance and regulatory aspects. This publication has been prepared by a group of industry experts representing 14 globally acting major biotechnology companies under the umbrella of the European Federation of Pharmaceutical Industries and Associations (EFPIA) Manufacturing & Quality Expert Group (MQEG).

European Federation of Pharmaceutical Industries and Associations

EFPIA Biomanufacturing Satellite Session at CASSS European Strategy Forum 2023

Fionnuala O'Driscoll (Eli Lilly), Karoline Bechtold-Peters (Novartis) and Helen Newton (MSD)






EFPIA Satellite Session - Scope

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. [...]

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.

A Q&A and lively panel discussion is planned.

High Level Agenda – EFPIA Satellite Session

Introduction and Concept Paper Updates

- **8.30-8.40:** Welcome & Introduction to the EFPIA MQEG Biomanufacturing Satellite Session - Markus Goese, Roche
- 8.40-8.50: Clonality, Characterisation and Viral safety of cell lines - Elodie Charbaut Taland, Merck KGaA
- 8.50-9.00: Antibody Drug Conjugates - Nienke Vriezen, Byondis
- 9.10-9.20: Control site concept for agile & mobile manufacturing - Karoline Bechtold-Peters, Novartis
- 9.20-9.30: EFPIA survey output – Quality Strategies for Expedited Access – Markus Goese, Roche
- **9.30-10.00: Q&A Panel Discussion** – with Klara Tiitso (EMA) and Seán Barry (HPRA) as panelists
- **10.00-10.30: Networking Break**

Scientific Session - Immunogenicity

- **10:30-10:45:** Immunogenicity against biotherapeutics - overview of mechanisms - Yariv Wine, Tel Aviv University
- 10:45-11.00: Clinical perspectives on immunogenicity of biotherapeutics - Florian Deisenhammer, University Hospital of Innsbruck (Tirol Kliniken), Austria
- 11:00-11:15: Non-clinical immunogenicity risk assessments including in-vitro assays - Hannah Morgan, Novartis
- 11:15-11.30: Outcomes and take homes of the ABIRISK Consortium - Sebastian Spindeldreher, Integrated Biologix
- 11:30-11.45: How to connect patient centric specifications with immunogenicity of biotherapeutics - A Regulator's perspective - Mats Welin, MPA
- **11:45-12:25: Panel Discussion** – with Steffen Gross (PEI) as panelist
- **12:25-12:30: Concluding remarks**

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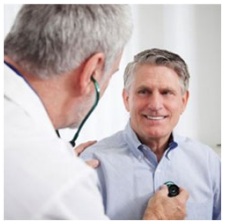
Thank you!



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