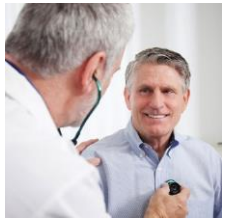
European Federation of Pharmaceutical Industries and Associations

Welcome & Introduction to EFPIA MQEG Biomanufacturing Satellite Session

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






CASSS CMC Strategy Forum EU

Virtual Session – Oct. 18, 2021

Presentation Outline

1. Welcome & a few facts about EFPIA
2. Highlights of EFPIA MQEG Biomanufacturing team's achievements & Outlook
3. Agenda of this year's Biomanufacturing Satellite Session at CASSS

REPOSITIONING INDUSTRY AS A PARTNER IN HEALTHCARE

#WeWontRest

About EFPIA


50
staff members
38
member companies
36
member associations
18
SMEs
35
partners in research
14
Vaccines Europe
An industry for healthy lives
Members

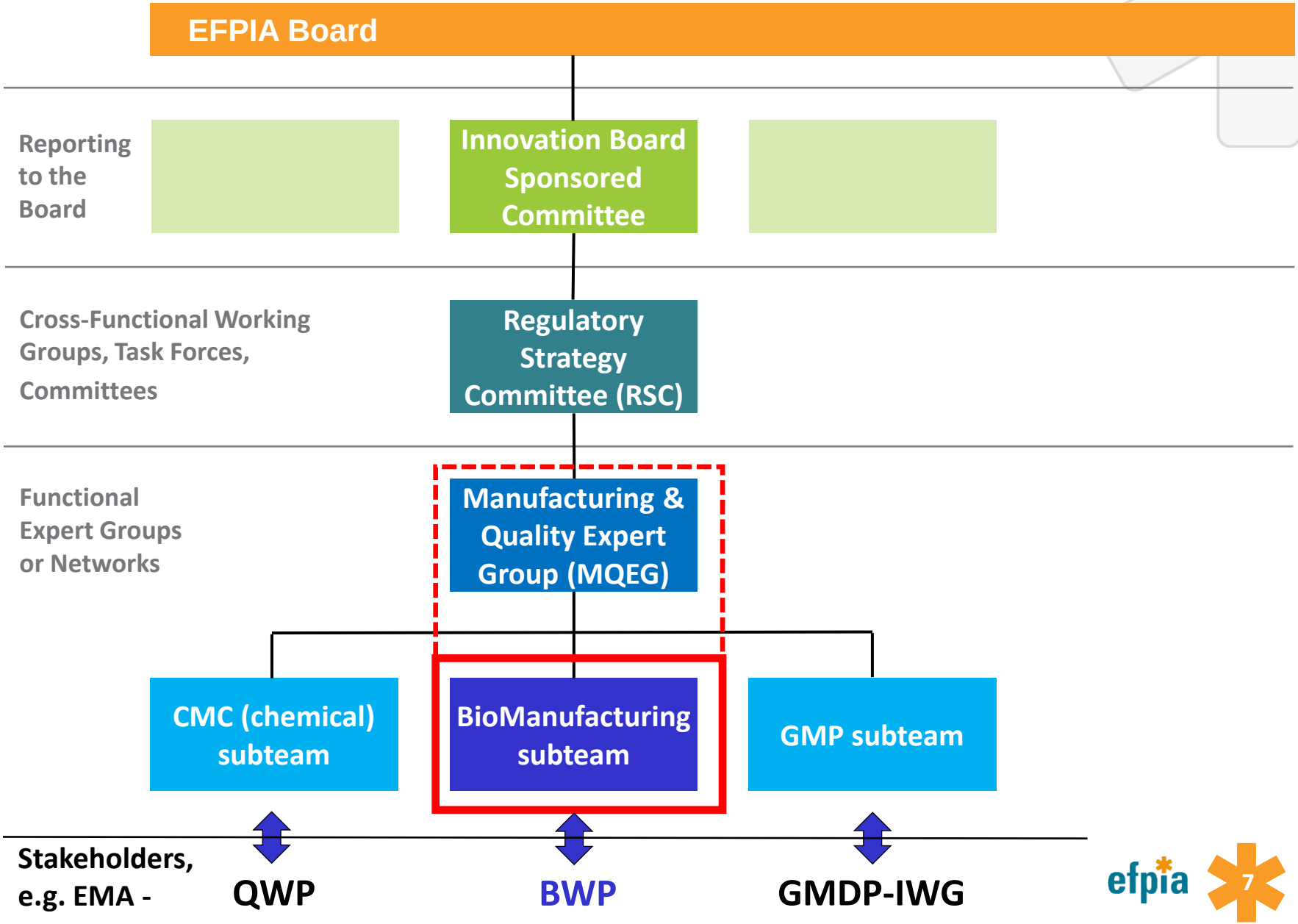
EFPIA integral to ICH successes

efpia leadership

Our mission

EFPIA's mission is to create a collaborative environment that enables our members to innovate, discover, develop and deliver new therapies and vaccines for people across Europe, as well as contribute to the European economy.

Simplified EFPIA Governance & MQEG strategic set-up



A joint effort: EFPIA MQEG COVID-19 CMC white paper

- **Members of Biomanufacturing subteam closely involved in important output of EFPIA MQEG: *White Paper on CMC development, manufacture and supply of pandemic COVID-19 therapies and vaccines***

EFPIA ✱ Date: 8 June 2020 Draft ☐ Final ✱

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EFPIA White Paper on CMC development, manufacture and supply of pandemic COVID-19 therapies and vaccines

Executive Summary

This paper provides focused recommendations for CMC and GMP approaches to support the development of new COVID-19 pandemic medicines.

In considering how to address the challenge of expediting the development of new medicines, EFPIA has worked with regulators for a number of years[1,2,3] on innovative CMC approaches and principles that can facilitate rapid science and risk-based development of new high-quality medicines. Building on these earlier interactions, EFPIA intends that this paper can be used by regulators and companies to implement such accelerated CMC approaches for the development and supply of COVID-19 medicines.

SOME HIGHLIGHTS – SEPT. 2020

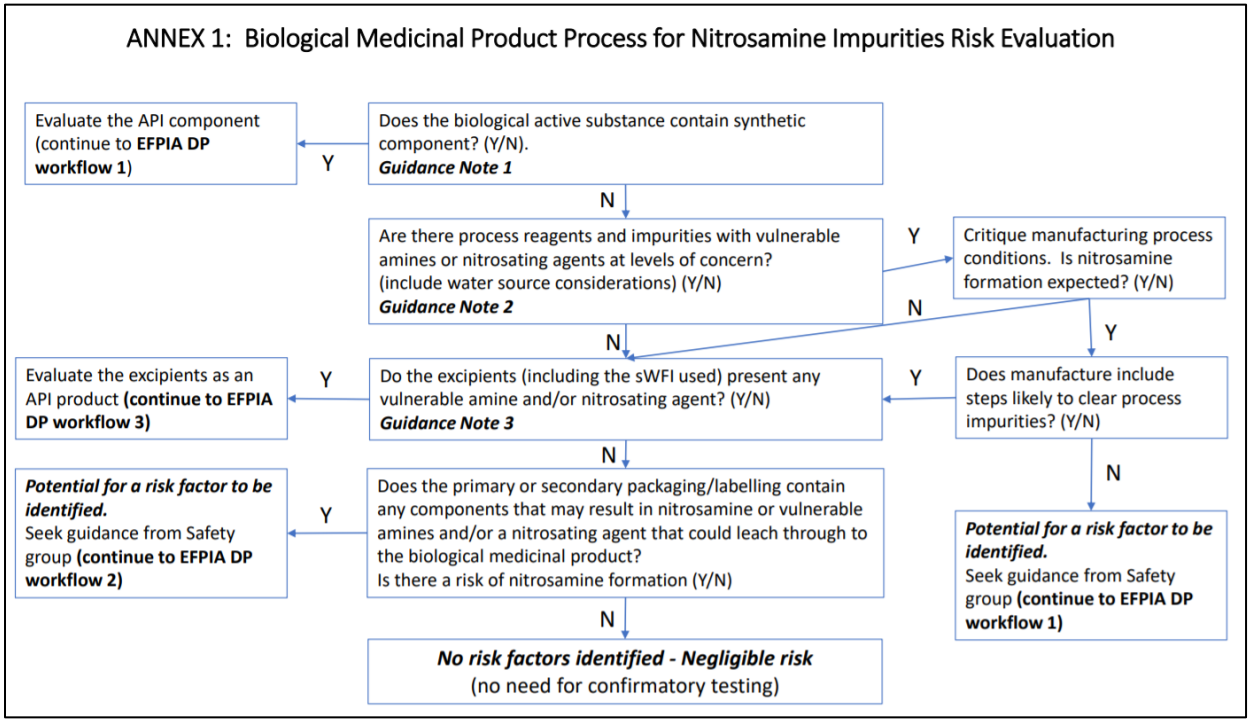
ATMP CMC/GMP Team within MQEG Biomanufacturing delivered EFPIA ICH Q-topic proposal to ICH QDG

ICH New Topic Proposal Template

1. Topic Title
Proposal to Introduce ICH Guidance on the Development and Manufacture of Advanced Therapy Medicinal Products
2. ICH Topic Description
Type of Harmonisation Action: New guideline ☒ ☐ Category of Harmonized Procedure: Quality ☒ ☐☐☐
Brief statement of perceived problem (caused by lack of harmonisation): Advanced therapy medicinal products (ATMPs) are medicines that are composed of the following classes of products; • Gene therapy medicinal products contain recombinant genes that lead to a therapeutic, prophylactic or diagnostic effect, usually to treat a variety of diseases, including genetic disorders, cancer or long-term diseases • Somatic cell therapy medicinal products contain unmanipulated cells or cells and tissues which have been manipulated to treat, diagnose or prevent diseases • Tissue engineered products contain cells or tissues that have been modified so they can be used to repair, regenerate or replace human tissue Because of the novelty, complexity and heterogeneity of ATMPs, insufficient guidance documents are available to date. ATMPs are not in the scope of current ICH guidance documents but developers apply the guidelines intended for biologics wherever possible although ATMPs differ in several important aspects from traditional biologics.

MQEG Biomanufacturing team representatives act as integral part of EFPIA response to the Nitrosamines issue

- Timely publication of *N-Nitrosamine Impurities in Biological Medicinal Products* paper and associated workflows



Continued Publication activities of MQEG Drug-Device CMC team: «Platform-device» reflection paper

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EFPIA Reflection Paper on Integral Drug-Device Combination Product Platform Approach

Date: 16/04/2021 Version: Final

Executive Summary

This paper describes how Quality by Design[1] (QbD) principles combined with use of prior knowledge and a Design of Experiments[2] (DoE) approach may be employed to create a design space of device and drug variables that constitutes an integral drug-device platform. These ICH Q8 (R2) [1], Q9 [2] and Q10 [3] concepts based on risk management are also largely consistent with ISO14971 [4]. Once the platform design space[3] has been approved by Regulatory agencies then a new product residing within the platform design space should have a reduced or no need of review for certain nonproduct-specific data that pertains to the device constituent part.

* Full version: see link

OUTLOOK 2021/22

Selected MQEG-Biomanufacturing Strategic Topics

- **ICH Q5A(R2) Quality of biotechnological products: viral safety**
  - Support development of Step 1 consensus guideline
- **Pharmacopoeial Monograph Strategy for complex Biologics**
  - Continue to explore performance-based monographs concept
- **Antibody-drug conjugates (ADCs)**
  - Finalize industry concept paper
- **Clonality, characterisation & viral safety of cell lines (NGS & other new technologies)**
  - Develop industry concept paper

To be discussed later in this session

- **Polysorbates (link to particle issues, ChP etc.)**
  - Develop/ finalize two reflection papers (control strategy & best-practice)
- **MAM as a QC tool (new topic)**
  - Develop industry reflection paper
- **Collaborate with EFPIA MQEG Mobile (autonomous/portable) manufacturing team**
  - Further complement existing first reflection paper

EFPIA MQEG BIOMANUFACTURING SATELLITE SESSION 2021

Agenda 1/3

14:00 – 17:50 **EFPIA MQEG Satellite Session**
Session Chairs: Karoline Bechtold-Peters, *Novartis Pharma AG*, Helen Newton, *MSD* and Fionnuala O'Driscoll, *Eli Lilly Kinsale Limited*

14:00 – 15:00 **Introduction and Concept Paper Updates**

14:00 – 14:10 **Welcome and Introduction**
Markus Goese, F. Hoffmann-La Roche Ltd.

14:10 – 14:20 **Polysorbate Degradation and Mitigation / Control Strategy Considerations**
Klaus Wuchner, *Cilag AG, Switzerland* and Linda Yi, *Biogen, United States*

14:20 – 14:30 **Agile Manufacturing – Autonomous and Gloveless Aseptic Work Chambers**
Karoline Bechtold-Peters, *Novartis Pharma AG, Switzerland*

14:30 – 14:40 **Considerations on Multi-Attribute Method (MAM) by LC-MS for QC Tool**
Thomas Pohl, *Novartis Pharma AG, Switzerland*

14:40 – 15:00 **Panel Discussion – Questions and Answers**
Chat Moderator: Helen Newton, *MSD* and Fionnuala O'Driscoll, *Eli Lilly Kinsale Limited*
Question Moderator: Markus Goese, *F. Hoffmann-La Roche Ltd.*

Panelists:
Brigitte Brake, *BfArM- Federal Institute for Drugs and Medical Devices*
Karoline Bechtold-Peters, *Novartis Pharma AG*
Thomas Pohl, *Novartis Pharma AG*
Klaus Wuchner, *Cilag AG*
Linda Yi, *Biogen*

15:00 – 15:10 **Mini-break**

Agenda 2/3

15:10 – 15:45	**Biological Hot Topic: Rapid Microbiological Analytical Methods**
15:10 – 15:15	**Introduction** Helen Newton, *MSD*
15:15 – 15:25	**Real-time Environmental Monitoring** TBD - *Novartis Pharma AG, Switzerland*
15:25 – 15:35	**Rapid Sterility Focus and Validation** Miriam Guest, *AstraZeneca, United Kingdom*
15:35 – 15:45	**Rapid-fire Questions and Answers** **Question Moderator:** Helen Newton, *MSD*
15:45 – 16:15	**Biological Hot Topic: Host Cell Proteins**
15:45 – 15:50	**Introduction** Fionnuala O'Driscoll, *Eli Lilly Kinsale Limited*
15:45 – 15:55	**Case Study on Lipolytic Enzymes and HCP Removal** Rachel Chen, *Biogen, United States*
15:55 – 16:05	**Immunogenicity of HCPs** Vibha Jawa, *Bristol-Myers Squibb Company, United States*
16:05 – 16:15	**Rapid-fire Questions and Answers** **Question Moderator:** Fionnuala O'Driscoll, *Eli Lilly Kinsale Limited*
16:15 – 16:25	**Mini-break**

Agenda 3/3

16:25 – 17:50 **Biological Hot Topic: mRNA – Cancer Therapy Applications and Associated Challenges**

16:25 – 16:30 **Introduction**
Karoline Bechtold-Peters, *Novartis Pharma AG*

16:30 – 16:45 **Therapeutic Application of mRNA in Cancer Therapy**
TBD - someone from BioNTech

16:45 – 17:00 **Regulatory Considerations on mRNA Products in Cancer Therapy**
Marcel Hoefnagel, *MEB-Medical Evaluations Board, Netherlands*

17:00 – 17:15 **mRNA Manufacturing Aspects – Novel Printing Methods**
TBD - someone from *CureVac*

17:15 – 17:30 **Challenges of Formulation of mRNA Products**
Gerrit Borchard, *University of Geneva, Switzerland*

17:30 – 18:00 **Panel Discussion – Question and Answers**
Chat Moderator: Helen Newton, *MSD* and Fionnuala O'Driscoll, *Eli Lilly Kinsale Limited*
Question Moderator: Karoline Bechtold-Peters, *Novartis Pharma AG*

Panelists:
Gerrit Borchard, *University of Geneva*
Marcel Hoefnagel, *MEB-Medical Evaluations Board*
TBD - someone from *BioNTech*
TBD - someone from *CureVac*

18:00 **Adjourn Day One**

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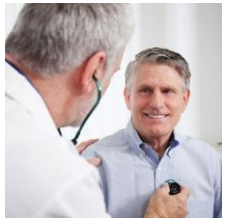
Thank you – any questions?



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