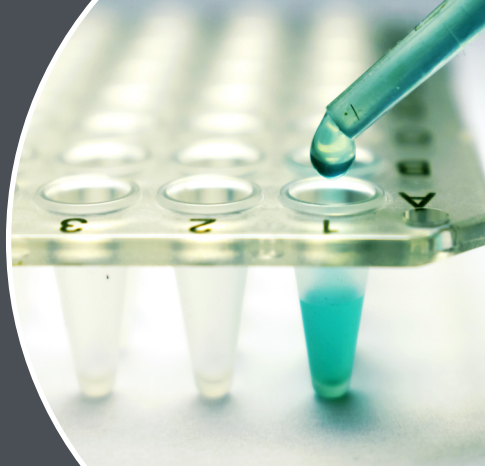
DIA VIRTUAL MEETING

2nd Joint DIA-EUCOPE Workshop on ATMPs, Innovative Gene and Cell Therapies in the EU

27-28 May 2020



PROGRAMME COMMITTEE

Maren von Fritschen
DIA Regional Advisory Council Chair, AddOn Pharma, Germany

Alexander Natz
Secretary General, EUCOPE, Belgium

Thomas Bols
Head of Government Affairs and Public Policy, EMEA & APAC, PTC Therapeutics, Inc., Switzerland

Sara Torgal
Scientific Programmes Manager, DIA, Switzerland

Overview

This workshop on cell and gene therapies aims to meet the needs of the developers of advanced therapies to discuss a range of topics that will help them make more informed decisions on regulatory strategies and evidence packages - as well as market access challenges - which are key topics for cell and gene therapy developers.

It will feature speakers from regulators, payers, patients, industry and academic organisations. Participants will benefit from the direct interaction with these stakeholders as a part of the main programme and during the networking breaks.

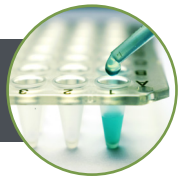
Key Topics

- Development challenges and solutions for cell and gene therapies
- Regulatory tools and pathways, incl. early interaction with decisions
- Hospital exemption issues
- Post-licensing evidence with examples from country-level initiatives
- HTA and value assessment for curative therapies with high upfront fee
- Case study for a launch in Europe

Who Will Attend

- R&D, regulatory and access professionals from organisations developing cell and gene therapies
- Regulators, payers and patients who are impacted by or participating the decisions or policies related to cell and gene therapies

DAY ONE | WEDNESDAY, 27 MAY 2020



09:00 LOG-IN AND CONNECT

09:30 SESSION 1

DEVELOPMENT OF ADVANCED THERAPY MEDICINAL PRODUCTS (ATMPS) – CHALLENGES AND OPPORTUNITIES FROM INDUSTRY AND AUTHORITY PERSPECTIVE

Session Chair:
Maren von Fritschen, DIA Regional Advisory Council Chair, AddOn Pharma, Germany

- **Industry's perspective on ATMP development**
  Andrea Braun-Scherhag, Vice President, Head Regulatory Affairs Europe, Ultragenyx, Switzerland
  - o Overview of development programmes for cell and gene therapies
  - o Practical experiences and key recommendations
  - o Challenges of ATMP development during COVID-19 pandemic
- **EMA's perspective on ATMP development**
  Ana Hidalgo-Simon, Head of Advanced Therapies, European Medicines Agency, EU
  - o Scientific Advices for ATMPs – identified issues
  - o Benefit of Prime scheme for ATMPs
  - o Update on EMA's approach during COVID-19 pandemic
- **Regulatory Assessor's perspective on ATMP development**
  Ilona G. Reischl, CAT Vice-Chair, Clinical Trials Assessor Federal Office for Safety in Health Care (BASG)
  - o Structured product development and caveats
  - o Legal interface issues: IVDs & combined ATMPs

Panel discussion with the presenters

10:50-11:05 BREAK

11:05 SESSION 2

HOSPITAL EXEMPTION – EUROPEAN LEVEL DISCUSSIONS AND NATIONAL PRACTICES

Session Chair:
Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Belgium

Speakers:
Pietro Sternini, Head of Market Access Europe, Atara Biotherapeutics, Switzerland
Ilona G. Reischl, Clinical Trials Assessor Federal Office for Safety in Health Care (BASG)
Michela Gabaldo, Head Alliance Managment & Regulatory Affairs, Fondazione Telethon, Italy

Discussion with the Audience

11:55-12:10 BREAK

12:10 SESSION 3

REGISTRIES SUPPORTING REAL WORLD EVIDENCE (RWE)

Session Chair:
Claudio Santos, SVP Global Medical Affairs, PTC Therapeutics Inc. USA

- Global development plan – changes in CT on the horizon?
- Real-world evidence to inform decision making of cell and gene therapies

Claudio Santos, SVP Global Medical Affairs, PTC Therapeutics Inc. USA

- EMA Approach on Post-licensing Evidence Collection and Registries for Decisions Making

Núria Semis-Costa, Risk Management Specialist at European Medicines Agency, EU

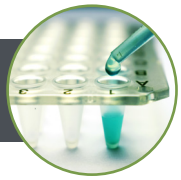
13:10 WRAP UP DAY ONE

13:30 END OF DAY ONE

Disclosure Policy

Unless otherwise disclosed, DIA acknowledges that the statements made by speakers are their own opinion and not necessarily that of the organisation they represent, or that of the DIA. Speakers and agenda are subject to change without notice. Recording during DIA sessions is strictly prohibited without prior written consent from DIA.

DAY TWO | THURSDAY, 28 MAY 2020



08:30 LOG-IN AND CONNECT

09:00 SESSION 4

SCENE SETTING

Session Chair:
Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Belgium

- Political developments
- Market developments
- Payer perspectives
- Developments on contracting

09:30-09:40 BREAK

09:40 SESSION 5

CROSS-BORDER HEALTHCARE AND INNOVATIVE PAYMENT MODELS

Session Chair:
Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Belgium

Speaker:
Michela Gabaldo, Head Alliance Managment & Regulatory Affairs, Fondazione Telethon, Italy
Christophe Hilbert, Manager, Market Access, Pricing and Reimbursement, Europe, Bluebird Bio, Switzerland

Panel discussion with Q&A

10:25-10:40 BREAK

10:40 SESSION 6

PRICING & HTA CHALLENGES FOR ATMPS

Session Chair:
Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Belgium

HTA methods - implications for ATMPs
Andrew Olaye, EMEA Market Access, Orchard Therapeutics, UK

Do we need innovative payment models for ATMP reimbursement in Europe?
Etienne Jousseame, Head Market Access Cell and Gene Europe at Novartis, France

Discussion with the audience and the presenters

11:40-11:50 BREAK

11:50 SESSION 7

RESOLVING HTA/PAYER UNCERTAINTIES WITH REAL WORLD EVIDENCE

Session Chair:
Karen M Facey, Senior Research Fellow, University of Edinburgh, UK

Speakers:
Sreeram Ramagopalan, Global Head Real World Data (Market Access), F. Hoffmann-La Roche Ltd, Switzerland
Karen M Facey, Senior Research Fellow, University of Edinburgh, UK

Panel Discussion:
Martin Wenzl, Health Policy Analyst, Organisation for Economic Co-operation and Development, France
Adrian Jonas, Associate Director for Data and Analytics, NICE, UK
Christos Sotirelis, EMA Patient Expert, UK
Sreeram Ramagopalan, Global Head Real World Data (Market Access), F. Hoffmann-La Roche Ltd, Switzerland

13:05 CLOSING REMARKS

Thomas Bols, Head of Government Affairs and Public Policy, EMEA & APAC, PTC Therapeutics, Inc., Switzerland

13:20 END OF WORKSHOP