DIA

Value, Access & Regulatory Evidence

Acknowledging the different perspectives and finding the pragmatic way forward

24-25 October 2019 | Swissotel, Basel, Switzerland

Programme Committee

Lucia D'Apote
Director, European Lead, Global Regulatory and R&D Policy, Amgen Ltd., UK

Mira Pavlovic
Associate Professor for regulatory and HTA Science, Portugal & Medicines Development and Training Services, France

Claudine Sapède
Director, Global HTA Policy
Novartis Pharma AG, Switzerland

Anja Schiel
Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA's Biostatistics Working Party, Chair EMA's Scientific Advice Working Party, Norway

Bakhuti Shengelia
Executive Director Global Policy and Healthcare Systems, Novartis, Switzerland

Who Should Attend

This workshop is intended for professionals with an interest in:

- Real World Evidence
- Epidemiology
- Policy
- Regulatory Science

Venue

Swissotel Le Plaza Basel
Messeplatz 25
4005 Basel

WIFI Access

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Overview

This workshop brings together all relevant V&A and Regulatory stakeholders in seeking solutions to the challenges of aligning Regulatory and Reimbursement strategies in Europe. In this quest for, most effective and pragmatic solutions for accelerating availability of medicines to patients in need, the agenda will use case studies and panel discussions. The event will cover the state of play experience from REA pilots and potential development post 2020 surrounding joint clinical assessments (JCAs). We will also discuss current challenges and gaps in PLEG and other implications for Global development programmes, where additional complexities lie.

The workshop builds on the EMA-EUnetHTA work programmes and involves key opinion leaders from both sides.

Objectives

- Through candid and well-informed discussions, help all involved stakeholders find better ways to improve communication in the regulatory-reimbursement ecosystem;
- Identify the limitations in how evidence is used across the entire regulatory-reimbursement and their implications for the patients answer the question of what isn't working perfectly;
- Come up with pragmatic solutions for closing existing evidence gap and their policy implications ("what could we do" and "how we should do");
- Try to find solutions for patients to get access to the medicines they need.

Key Topics

- Parallel consultation, Scientific Advice & Early Dialogue – learnings from the past and shaping the future: Case study & Panel Discussion
- Patient role/involvement
  - How and when in regulatory and HTA assessment process patients could be more effectively engaged?
  - The role of patients in shaping outcome measurements for regulatory and reimbursement purposes
- Uncertainty of evidence - feasibility of delivering an a data package acceptable for both regulators and reimbursement authorities
  - Choice of comparator
  - Surrogate/intermediate endpoints and extrapolating the results
  - Use and acceptability of innovative clinical trial designs (adaptive trials, master protocols, use of historical controls)
- Post-licensing evidence generation (PLEG) - Scope and acceptance revisited
- Suitability of current regulatory & value frameworks for new technology based curative therapies (e.g. cell and gene) in the pipeline
- Regulatory vs Access views – internal alignment within industry functions?
- Unmet medical needs & evidentiary standards

DAY ONE | THURSDAY, 24 OCTOBER 2019

08:00 REGISTRATION

08:30 KEYNOTE

THE IMPACT OF DIGITAL TECHNOLOGIES IN HEALTH

Speaker: **Stephen Moran,** Global Head of Strategy, Novartis, Switzerland

09:15 SESSION 1

OVERVIEW OF EXISTING EVIDENCE GAPS AND MISALIGNMENTS ACROSS REGULATORY AND REIMBURSEMENT ECOSYSTEM – IMPLICATIONS FOR THE PATIENT ACCESS

Session Chair:

Mira Pavlovic, Associate Professor for Regulatory and HTA Science, Portugal & Medicines Development and Training Services, France

The session will set the scene by providing a high level critique of the current state of affairs in the regulatory and reimbursement ecosystem by focusing on evidence gaps and effectiveness of communication between various players. The session will point out the most pressing issues that are amenable to solution, and discuss the implications of those issues for patient access.

Evolution of Policies and Standards for Handling the Evidence for Decision Making – Selected "Hot Topics" to Set the Scene
Michael Berntgen, Head of Product Development, Scientific Support Department, European Medicines Agency, EU

Existing Gaps in the Use of Evidence across Regulatory and Reimbursement Agencies, Misalignment of Standards and Practices and their Implications for Patient Access
Niklas Hedberg, Chair EUnetHTA Executive Board & Chief Pharmacist Dental and Pharmaceutical Benefits Agency, TLV, Sweden

Reimagining the Use of Evidence across Regulatory and Reimbursement Continuum – What are the Promising Ways Forward?
Edith Frenoy, Director Market Access, EFPIA, Belgium

Panel Discussion with Q&A

10:45 COFFEE BREAK

11:00 SESSION 2

PARALLEL SCIENTIFIC ADVICE

Session Chair:

Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA's Biostatistics Working Party, Chair EMA's Scientific Advice Working Party, Norway

In this session, the different Stakeholder experiences with Parallel Scientific Advice will be presented. The Speakers will reflect on their learnings as well as discuss how such learnings can translate into improvements that can be proposed for the future. This session also sets the scene for the SA role-play in session 3 where participants are encouraged to take different Stakeholder perspectives when discussing different theoretical products.

Regulatory Perspective
Michael Berntgen, Head of Product Development, Scientific Support Department, European Medicines Agency, EU

HTA Body Perspective
Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA's Biostatistics Working Party, Chair EMA's Scientific Advice Working Party, Norway

Industry Perspective
Judith Creba, Executive Director, EU Regulatory Strategy Novartis, Switzerland

Panel Discussion with Q&A

12:30 LUNCH BREAK

14:00 SESSION 3

PARALLEL SCIENTIFIC ADVICE & EARLY DIALOGUE - ROLE PLAY ON MINI SCIENTIFIC PARALLEL ADVICE

Session Chair:

Giovanni Tafuri, Senior Scientific Officer, EUnetHTA, Netherlands

Anja Schiel, Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA's Biostatistics Working Party, Chair EMA's Scientific Advice Working Party, Norway

This session is a role-play exercise where the Participants are encouraged to take different Stakeholders perspectives and discuss hypothetical products. By standing in someone else's shoes, we hope that the challenges related to try to find the most optimal solution to satisfy all Stakeholders requests become clear.

| 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 ONE | THURSDAY, 24 OCTOBER 2019

Panel Discussion with the participation of:

Industry Perspective
Vanessa Elisabeth Schaub, Global Access Senior Health Systems Strategy Leader HTA & Reimbursement, F. Hoffmann-La Roche, Switzerland

Payers Perspective
Erik Sagdahl, Advisor – Pharmaceutics and Health Economics, Norwegian Medicines Agency (NoMA), Norway

Regulatory Perspective
Peter Mol, Member, SAWP, Chair of EMAs Registry Task Force, University Medical Center Groningen, Netherlands

Patients Perspective
Matteo Scarabelli, Patient Engagement Manager in HTA, EURORDIS, France

16:00 SESSION 4

POST LICENSING EVIDENCE GENERATION – WHAT IS IT AND WHAT IS ITS PURPOSE?

Session Chair:
Niklas Hedberg, Chair, EUnetHTA Executive Board & Chief Pharmacist, Dental and Pharmaceutical Benefits Agency, TLV, Sweden

How the PLEG Landscape Has Changed in the Past Decade – EUnetHTA perspective
Irena Guzina, Medical Assessor, Coordinator of EUnetHTA WP5B PLEG Activities, HAS, France

Current Challenges and Gaps in PLEG and Their Implications for Regulatory and Reimbursement Decision Making
Peter Mol, Member, SAWP, Chair of EMAs Registry Task Force, University Medical Center Groningen, Netherlands

Utilising Different Data Sources for Post-Licensing Evidence – Opportunities and Challenges
Michael Berntgen, Head of Product Development, Scientific Support Department, European Medicines Agency, EU

PLEG: The Perspective of Pharmaceutical Developers
Catherine Cohet, Senior Epidemiology Expert, Clinical R&D, GSK, Belgium

What Will the Future Bring: Panel Discussion with Q&A

17:30 NETWORKING RECEPTION - SPONSORED BY OUR PARTNERCLINERION LTD (*HELVETIA 2*)

SHORT TALK: GATHERING OF REAL WORLD EVIDENCE IN EUROPE USING MULTIPLE DATA SOURCES IN A CHALLENGING ENVIRONMENT

Lenka Kellermann, Managing Director, OncologyInformationService, Germany
Ian Rentsch, Chief Executive Officer, Clinerion Ltd, Switzerland

18:30 END OF DAY ONE

DAY TWO | FRIDAY, 25 OCTOBER 2019

08:00 REGISTRATION

08:30 SESSION 5

POST-LAUNCH EVIDENCE GENERATION (PLEG) – WORKSHOP

Session Chair:
Niklas Hedberg, Chair, EUnetHTA Executive Board & Chief Pharmacist, Dental and Pharmaceutical Benefits Agency, TLV, Sweden

This session will provide the opportunity for the audience to actively participate through small group discussions and share experience on a selection of themes related to PLEG.

- We will look from our desired situation, to our present one and then focus on the way to go between these two.
- Several different aspects of PLEG will be covered such as legal and technical aspects as well as methodological issues etc.

10:00 COFFEE BREAK

10:30 SESSION 6

JOINT CLINICAL ASSESSMENT (REA)

Session Chair:
Claudine Sapède, Director, Global HTA Policy, Novartis Pharma AG, Switzerland

In this session panellists will discuss the state of play, experience from REA pilots and potential development post 2020 surrounding joint clinical assessments (JCAs) - a key component of the joint work across European HTA agencies. What good quality means for stakeholders in relation to clinical HTA methodological standards for JCAs? What practical as well as policy implications this may have for stakeholders and sustainability of EU cooperation? During the workshop session the audience will also be invited to share its perspective and experience.

Panel Discussion with the participation of:

Anja Schiel, Anja Schiel, Senior Adviser/Statistician, Unit for HTA and Reimbursement Norwegian Medicines Agency (NoMA), Chair EMA's Biostatistics Working Party, Chair EMA's Scientific Advice Working Party, Norway
Edith Frenoy, Director Market Access, EFPIA, Belgium
Michael Berntgen, Head of Product Development, Scientific Support Department, European Medicines Agency, EU
Anne Willemsen, Project Manager, Joint Production Co-Lead Partner – Pharmaceuticals, the National Health Care Institute (ZIN), Netherlands
Kalitsa Filioussi, Market Access Director Hematology, Novartis Oncology-Europe, Switzerland
Industry Speaker Invited

12:00 LUNCH

13:00 SESSION 7

JOINT CLINICAL ASSESSMENT (REA) - WORSHOP

Session Chair:
Claudine Sapède, Director, Global HTA Policy, Novartis Pharma AG, Switzerland

This session will provide the opportunity for the audience to actively participate through small group discussions and share experience on a selection of themes related to JCA. Panellists from session 6 will join to facilitate support the group

- Uptake of REA Reports
- Interface between JCA and National HTA Agencies Assessments
- Methodological Quality Standards and Implications for Evidence Generation Development
- Industry Preparedness for JCA Dossier Submission

14:30 COFFEE BREAK

15:00 SESSION 8

SUITABILITY OF CURRENT REGULATORY AND HTA FRAMEWORKS FOR NEW HIGH-VALUE CURATIVE TECHNOLOGIES

Session Chair:
Lucia D'Apote, Director, European Lead, Global Regulatory and R&D Policy, Amgen Ltd, UK

Panel Discussion on:

- How Regulators and HTA can collaborate more closely to define mutual evidence requirements for better patient access?
- Are the current regulatory and HTA frameworks suitable for the new curative technologies – where are the gaps?
- How health systems define value proposition for new curative technologies?

With the participation of:

Niklas Hedberg, Chair, EUnetHTA Executive Board & Chief Pharmacist, Dental and Pharmaceutical Benefits Agency, TLV, Sweden
Flora Giorgio, Head of Sector, Unit B4- Medical Products: Quality, Safety, Innovation, DG SANTE European Commission, EU
Michael Berntgen, Head of Product Development, Scientific Support Department, European Medicines Agency, EU
François Houyez, Treatment Information and Access Director, Health Policy Advisor European Organisation for Rare Diseases (EURODIS), Belgium
Ansgar Hebborn, Head – Global HTA & Payment Policy, F. Hoffmann-La Roche AG, Switzerland

16:15 END OF WORKSHOP

EVALUATION

We value your feedback on the content and organisation of this conference. Please complete the electronic survey after the conference

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No paper copies of the presentations will be provided.

NOTE: If a presentation is not available, the speaker either did not agree to publish it or did not provide us with the presentation. Updated versions of the slides will be made available shortly after the conference.

CERTIFICATE OF ATTENDANCE

A Certificate of Attendance will be sent to all attendees electronically after the conference. Please note certification requires full attendance. For more information please liaise with our DIA Contact Centre on Basel@DIAglobal.org or call +41 61 225 51 51.