DIA Value, Access & Regulatory Strategy Workshop

25-26 October 2017
Radisson Blu Hotel, Basel, Switzerland

PROGRAMME COMMITTEE

Solange Corriol-Rohou
Senior Director, Global Regulatory Affairs & Policy, Europe
AstraZeneca Global Medicines Development, France

Angelika Joos
Executive Director, Global Regulatory Policy
MSD, Belgium

Katarzyna Kolasa
Senior Project Sales Director, Health Economics & Outcomes Research, Eastern Europe
GE Healthcare, Poland

Mira Pavlovic
Founder
MDT Services, France

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Global HTA & Payment Policy Lead
F. Hoffmann-La Roche, Switzerland

OVERVIEW

All stakeholders, including leaders across industry and health authorities, agree that finding answers to market access is critical to delivering breakthrough medicines to patients. Unfortunately, policies and practices do not always marry up.

This meeting will bring together professionals working with regulatory and value strategies. HTA bodies and regulators are breaking down silos, facilitating access to new promising medicines and increasing efficiencies in assessment processes. Similarly, R&D processes need to adapt to generate the appropriate evidence for registration and reimbursement that should allow patients' timely access to innovative and promising drugs.

Bringing together policy makers, developers, and the patients as end users, to keep you ahead of the game with practical solutions.

DIA shares insights in advance of this conference:

- Webinar: Approvals and Access – Overcoming the Final Hurdle of Drug Development
- Podcast: EBE Plots Path from "Flawed Underfunded Biotech System"

Hear a preview of programme committee member Solange Corriol-Rohou's perspective on evidence generation throughout the life-cycle to support Medicines Adaptive Pathways to Patients (MAPPs) in the IMI project ADAPT SMART Webinar: ADAPT SMART "Putting Stakeholders at the Centre"

WHAT YOU WILL LEARN

- Acceptance of RWD by HTA bodies/payers
- Early access to market
- Definition of unmet medical needs
- Alignment on evidence requirements across EU and between regulators and HTA
- Methodologies for HTA and pricing of products in combination or multiple indications
- Cross-functional multi-stakeholder dialogue to optimize market access and product affordability
- Patient's role in the regulatory and HTA process

WHO SHOULD ATTEND?

- Professionals working in regulatory affairs and HTA/market access
- Professionals involved in drug development, e.g. clinicians, epidemiologists and biostatisticians

Value, Access & Regulatory Strategy

DAY ONE I WEDNESDAY, 25 OCTOBER

08:00 REGISTRATION AND WELCOME COFFEE

08:30 KEYNOTE SPEECH

European Medicines Agency (EMA) Representative invited

09:00 SESSION 1

DIFFERENT PERSPECTIVES ON UNMET MEDICAL NEED

Session Chair:
Claudine Sapede, Global HTA & Payment Policy Lead, F. Hoffmann-La Roche, Switzerland

Addressing unmet medical need is a key criterion for medicine prioritisation and for a molecule to qualify for accelerated regulatory review processes. Yet, there is not a common definition of the term and the interpretations may also vary between stakeholder groups (regulators, HTA bodies/payers, patients, medicine developers...). To what extend and how is unmet medical need considered in reimbursement decision-making? Could we make progress and advance these questions? This is what will be explored in this session.

Speakers:
Federal Joint Committee (G-BA), Germany, Representative invited

Medical Products Agency, Sweden, Representative invited

EURORDIS Representative invited

10:30 COFFEE BREAK

11:00 SESSION 2

ADAPTIVE PATHWAYS AND INNOVATIVE MEDICINES DEVELOPMENT FOR IMPROVED ACCESS

Session Chair:
Solange Corriol-Rohou, Senior Director, Global Regulatory Affairs & Policy, Europe, AstraZeneca Global Medicines Development, France

Adaptive Pathways should enable patients' earlier access to promising treatments in areas of high unmet medical needs. This is certainly a sensitive and controversial concept which value is currently explored through the EMA Adaptive Pathways initiative and the IMI ADAPT SMART project.

Numerous questions need to be addressed to make this concept a viable and efficient approach in Europe and beyond. What evidence should be generated, which tools and methods should be used or need to be developed, what criteria to fulfil to enter the adaptive pathways, when to leave the pathways, how to deal with product value uncertainties, how to address the criticisms and what could be the impact on managed entry agreements?

Panel Discussion with Q&A

Panellists:
European Medicines Agency (EMA) Representative invited
National Institute for Health and Care Excellence (NICE) Representative invited
Industry Representative invited
Physician Representative invited

12:30 LUNCH

14:00 SESSION 3

EARLY DIALOGUE BETWEEN REGULATORS AND HTA (AND PAYERS & PATIENTS)

Session Chair:
Mira Pavlovic, Founder, MDT Services, France

After having obtained marketing authorisation, a product may be refused for reimbursement if its relative effectiveness is considered inferior to existing treatments or if the evidence provided is considered inadequate or insufficient. An early dialogue/scientific advice between the sponsor and the Health Technology Assessment (HTA) bodies before the start of the pivotal clinical programme is particularly useful to discuss the evidence to provide and ultimately facilitate HTA process, timely reimbursement decision(s) and market access. For this purpose, sponsors may ask for national or multinational, regulatory and/or HTA advice. The experience gathered so far will be discussed as well as ways of possible improvements.

When receiving guidance from HTA bodies, national authorities apply country/region - specific policies and legal requirements related to the specificities of their own healthcare system. Therefore, respecting HTA needs for evidence generation does not guarantee that a drug will be reimbursed. A topic of specific interest here is the choice of an adequate comparator when the latter differs in different European countries including the use of non-authorised comparators. Possible implications for trial designs will be discussed.

- Experience so far (EMA, some HTA bodies)
- How to improve it (continuous dialogue)
- Early dialogue in PRIME and ADAPTIVE PATHWAYS
- Special topic: choice of comparator and off-label use
  - Differences in acceptance by HTA bodies
  - Implications for trial design

Speakers:
French National Authority for Health (HAS) Representative invited
Industry Representative invited
Academia Representative invited

15:30 COFFEE BREAK

16:00 SESSION 4

PATIENT'S ROLE IN THE REGULATORY AND HTA PROCESS

Session Chair:
EURORDIS Representative invited

Patient relevant endpoints (PRE), patient-reported outcomes (PRO) and patient-centred outcomes (PCO) are frequently used interchangeably, even if their definitions and roles differ considerably.

PRE are clinical endpoints that reflect how a patient feels, functions and survives; they cover broadly morbidity (due to a disease or its treatment), mortality and health-related quality of life and may be assessed both by patients and by physicians; quite often, PRO and PRE are identical. The simultaneous assessment of all PRE versus adequate comparator is a hallmark of relative effectiveness assessment in Europe.

PRO are outcomes that can be reported only by patients themselves due to their subjective nature; they cover simple measures such as symptoms, and more complex measures such as symptom rating scales and multidimensional questionnaires of health-related quality of life.

Patient-centered outcomes and related research (PCOR) are aimed at developing structured patient engagement and centeredness in the comparative effectiveness field as well as in the shared decision making in clinical practice.

This panel will discuss PRO, PRE and PCOR, and their use to support marketing authorisation and reimbursement decisions in Europe.

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Collaboration between patients and decision makers (regulatory, HTA and payers) will be described and discussed. The need for patients and patient representatives well informed and trained in the area of medicines development will be highlighted, and ongoing training initiatives, e.g. the European Patient Academy (EUPATI) will be discussed as relevant actions to allow the informed patient engagement in the medicines development process and clinical use.

- Patient's role in regulatory and HTA process
  - Regulator and HTA point of view
  - Patient point of view
- Patients as experts: role, training, committees
- Suitability of data from new sources (eHealth, mobile phone apps) - How to include it into the decision making process

Speakers:
Paris Diderot University Representative invited
University of Lisbon Representative invited
Industry Representative invited

17:30 NETWORKING RECEPTION

18:30 END OF DAY ONE

DAY TWO | THURSDAY, 26 October

08:00 REGISTRATION AND WELCOME COFFEE

09:00 SESSION 5

EVOLUTION OF PRODUCT DEVELOPMENT TO RESPOND TO THE FUTURE NEEDS

Session Chair:
Angelika Joos, Executive Director, Global Regulatory Policy, MSD, Belgium

Industry is responding to society's call for innovative medicine development addressing high unmet medical needs. As a consequence, the traditional medicine development paradigm is undergoing fundamental changes. For example, new immuno-oncology medicines are developed using new scientific methodologies, such as platform trials and single arm studies to accelerate the clinical development and make new treatment options available to patients as quickly as possible. The clinical development programs progress with unprecedented speed and the complexity of the programs is increasing. They often include platform trials testing multiple compounds to identify the best target or combination therapy trials that could potentially offer cures.
The panel will be invited to discuss the impact of the new scientific methodologies on the current regulatory and value assessment frameworks:

- How to make clinical development more efficient to support early access to market
- Managing multiple/new indications
- Product used in combination with other product
- How to evaluate the value of multiple indications and the combination

Setting the Scene: Introduction to new development realities – multi-indication/ combination therapies, the respective clinical trial setting and challenges in patient access

- **Industry Perspective**

Panel Discussion with Q&A

- **Regulators' Perspective:** What is convincing evidence? What is the focus of the regulatory benefit/risk decision? How are patient preferences considered?
- **Payer/ HTA Perspective:** What is evidence? Alternative solutions?
- **Patients' Perspective:** What does fast patient access mean? Risks, early access and trade-offs.
- **Physicians' Perspective:** Between fast access and evidence but closest to the patient – how can physicians help with early access?
- **Payers' Perspective:** How can payers facilitate patient access to combination products?

10:30 COFFEE BREAK

11:00 SESSION 6

USE OF REAL-WORLD EVIDENCE TO SUPPORT ACCESS

Session Chair:
Katarzyna Kolasa, Senior Project Sales Director, Health Economics & Outcomes Research, Eastern Europe, GE Healthcare, Poland

How to get a full insight into the unmet medical needs and create a meaningful research & development (R&D) program as well as a credible value proposition? What makes a successful pricing & reimbursement (P&R) story in the era of the growing healthcare budget constraints? In the session, we will discuss whether the growing opportunities of real world data (RWD) can ease these challenges. In the era of digitalisation of healthcare and burst of patient's stories sharing on social media, more and more new types of RWD are becoming available. The question is how to leverage on them in a credible and robust manner in order to develop new RWD driven R&D and P&R decision making processes.

How Can RWD Uncover Real Unmet Medical Needs in R&D Process?
Academia Representative invited

Shall We Rethink RWD in the Era of Health's Digitalization?
Regulator invited

Can We Move from RWD to Real World Decision Making?
Industry Representative invited

Panel Discussion with Q&A: How to Redefine RWD to Ensure its Usability for R&D and P&R Decision Making Processes?

12:30 LUNCH

14:00 SESSION 7

RELATIVE EFFICACY VERSUS RELATIVE EFFECTIVENESS – THE FUTURE

Session Chair:

French National Authority for Health (HAS) Representative Invited

- What could come next from the collaboration between HTA bodies?
- Future role of EMA
- Role of EUnetHTA moving forward

Speakers:

French National Authority for Health (HAS) Representative invited
European Commission Representative invited

15:30 END OF CONFERENCE

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Conference Venue

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Continuing Education

SwAPP and SGPM Credits

DIA meetings and training courses are approved by the SwAPP (Swiss Association of Pharmaceutical Professionals) Commission for Professional Development (CPD) and SGPM (Swiss Society of Pharmaceutical Medicine) and are honoured with credits for pharmaceutical medicine. All meeting and training course participants are eligible for applicable credits.

This workshop has been accredited with 11.00 credits.

About DIA

DIA is the global connector in the life sciences product development process. Our association of more than 18,000 members builds productive relationships by bringing together regulators, innovators, and influencers to exchange knowledge and collaborate in an impartial setting. DIA's network creates unparalleled opportunities for exchange of knowledge and has the inter-disciplinary experience to prepare for future developments.

The dedicated efforts of DIA staff, members and speakers enable DIA to provide a comprehensive catalogue of conferences, workshops, training courses, scientific publications and educational materials. DIA is a global community representing thousands of stakeholders working together to bring safe and effective products to patients.

DIA is an independent, non-profit organisation has its Global Center in Washington, DC, USA with the European office in Basel, Switzerland, and additional regional offices in Horsham, Pennsylvania, USA; Tokyo, Japan; Mumbai, India; and Beijing, China

For more information, visit www.DIAglobal.org
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Evaluation

We value your feedback on the content and organisation of this conference. The electronic survey will be sent to you after the conference.

Access Presentations

As a benefit of your registration, presentations are made available on www.diaglobal.org.

- Presentations are made available to full conference attendees only (Attendee, Exhibitor, Speaker, and Press badges).
- To access presentations, go to My Presentation Downloads and log in when prompted
- 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 their presentation. Updated versions of the slides will be made available shortly after the conference.

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Certificate of Attendance

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

Group Discounts

Register 3 individuals from the same company and receive a 50% discount for a 4th! All 4 individuals must register and prepay at the same time without exception. DIA will apply the value of the lowest applicable fee to this discounted registration; it does NOT include fees for optional events or DIA membership. You may substitute group participants of the same membership status at any time; however, administrative fees may be incurred.

Group registration is not available online and only available for the industry rate.

To take advantage of this offer, please print the registration form for each of the four registrants from your company. Include the names of all four group registrants on each of the forms and return them together to DIA.

For groups of 5 or more individuals, please contact Zsofia.Molnar@DIAglobal.org for a custom group rate.

REGISTRATION FORM | ID# 17123

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DIA

Early-bird discount and Advance rate

To qualify for the discount, registration form and accompanying payment must be received by the dates below. Early-bird/Advance rate applies to industry members only.

Early bird discount: register by 02 August 2017 **€ 1'230.00** ❑

Advance rate: register by 13 September 2017 **€ 1'330.00** ❑

CATEGORY	Member *	Non-Member*
Industry	€ 1'430.00 ❑	€ 1'585.00 ❑
Government/Charitable/Non-profit/Academia (Full-Time)	€ 715.00 ❑	€ 870.00 ❑

If DIA cannot verify your membership upon receipt of registration form, you will be charged the non-member fee . Group discount/SME rates available. Special rates for students and patient representatives on offer, subject to avaibility. Please contact DIA EMEA for more information.
Registration fee includes: refreshments, lunches, reception and meeting materials.

*All fees are subject to the applicable VAT. Payment due 30 days after registration and must be paid in full by commencement of the event.

TOTAL AMOUNT DUE: € _____

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Payments must be net of all charges and bank charges must be borne by the payer. **If you have not received your confirmation within five working days, please contact DIA Europe, Middle East and Africa.**

By signing below, I confirm that I agree with DIA's Terms and Conditions of booking. These are available from the office or online by clicking **here.**

Date	Signature

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- Industry (Member/Non-member) € 200.00
- Academia/Charitable/Government/Non-profit (Full-time) (Member/Non-member) € 100.00

For cancellations after this date, or if the delegate fails to attend the meeting, no refund of fees will be given and be responsible for the full registration fee. DIA EMEA reserves the right to alter the venue and dates if necessary. If an event is cancelled, DIA EMEA is not responsible for airfare, hotel or other costs incurred by registered attendees. Registered attendees are responsible for cancelling their own hotel and travel reservations.

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You may transfer your registration to a colleague prior to the start of the event but membership is not transferable. Substitute attendees will be responsible for the non-member fee, if applicable. Please notify the DIA EMEA office of any such substitutions as soon as possible.

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The DIA Europe, Middle East & Africa Contact Center will be pleased to assist you with your registration from Monday to Friday between 08:00 and 17:00 CET.

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