DIA
Value, Access & Regulatory Strategy Workshop

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

PROGRAMME COMMITTEE

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Global HTA & Payment Policy Lead
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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.

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

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
- Patient organisations
- Biotech companies, drug developers including SMEs
- Decision makers (Ministries of Health etc.)

Value, Access & Regulatory Strategy Workshop

DAY ONE I WEDNESDAY, 25 OCTOBER

08:00 REGISTRATION AND WELCOME COFFEE

08:30 KEYNOTE SPEECH

ACCELERATING ACCESS TO MEDICINES – OVERCOMING THE DEVELOPMENTAL, REGULATORY AND REIMBURSEMENT CHALLENGES

Richard Barker, Founding Director, Centre for the Advancement of Sustainable Medical Innovation (CASMI), United Kingdom

09:00 SESSION 1

DIFFERENT PERSPECTIVES ON UNMET MEDICAL NEED

Session Chair: **Claudine Sapède,** 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.

A Patient's Perspective

François Houÿez, Treatment Information and Access Director, Health Policy Advisor, EURORDIS, France

A Regulator's Perspective

Michael Berntgen, Head of Product Development Scientific Support Department, European Medicines Agency (EMA), European Union

An HTA Body's Perspective

Antje Behring, Team Lead, Early Benefit Assessment, Pharmaceuticals Department, Federal Joint Committee (G-BA), Germany

Panel Discussion with Q&A

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:

Jacoline Bouvy, Scientific Adviser, Science Policy and Research Programme, National Institute for Health and Care Excellence (NICE), United Kingdom

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

Richard Barker, Founding Director, Centre for the Advancement of Sustainable Medical Innovation (CASMI), United Kingdom

Rosa Giuliani, Consultant in Medical Oncology, S. Camillo-Forlanini Hospital, Italy; European Society for Medical Oncology (ESMO), Switzerland

12:30 LUNCH

14:00 SESSION 3

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

Session Chair: **Mira Pavlovic**, Founder, Medicines Development and Training (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
- 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

HTA Bodies Cooperation on Early Dialogues: Recent Advances

François Meyer, Advisor to the President, International Affairs, French National Authority for Health (HAS), France

Enhancing the Dialogue Beyond RCTs

Byron Jones, Executive Director, Statistical Methodology and Consulting Group, Novartis Pharma, Switzerland

The Imperfect Study Design – How to Deal with It?

Stefan Lange, Deputy Director, Institute for Quality and Efficiency in Health Care (IQWiG), Germany

15:30 COFFEE BREAK

16:00 SESSION 4

PATIENT'S ROLE IN THE REGULATORY AND HTA PROCESS

Session Chair: **François Houÿez**, Treatment Information and Access Director, Health Policy Advisor, EURORDIS, France

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. 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
- Patients as experts: role, training, committees
- Suitability of data from new sources - How to include it into the decision making process

Patient-Reported Outcomes Data Acquisition: Is It Worth It?

Olivier Chassany, Professor, Director, Patient-Reported Outcomes & Clinical Endpoints Research, University Paris-Diderot, France

State of Play of Patients Engagement in Medicines Development and Research

Beatriz Silva Lima, Professor of Pharmacology, University of Lisbon, Portugal; NDA Advisory Board Member

Patient Driven Data – What's in a Definition?

Elin Haf Davies, Founder/CEO, aparito, United Kingdom

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**
  Julie Lepin, Vice President, Global Regulatory Affairs, Therapeutic Area Head, Oncology and InVitro Diagnostics, Merck, United States

Panel Discussion with Q&A

- **Regulator's Perspective:** What is convincing evidence? What is the focus of the regulatory benefit/risk decision? How are patient preferences considered?
  Michael Berntgen, Head of Product Development Scientific Support Department, European Medicines Agency (EMA), European Union
- **HTA Body's Perspective:** What is evidence? Alternative solutions?
  Antje Behring, Team Lead, Early Benefit Assessment, Pharmaceuticals Department, Federal Joint Committee (G-BA), Germany
- **Patient's Perspective:** What does fast patient access mean? Risks, early access and trade-offs.
  François Houÿez, Treatment Information and Access Director, Health Policy Advisor, EURORDIS, France
- **Physician's Perspective:** Between fast access and evidence but closest to the patient – how can physicians help with early access?
  Rosa Giuliani, Consultant in Medical Oncology, S. Camillo-Forlanini Hospital, Italy; European Society for Medical Oncology (ESMO), Switzerland

10:30 COFFEE BREAK

11:00 SESSION 6

USE OF REAL-WORLD EVIDENCE TO SUPPORT ACCESS

Session Chair: **Katarzyna Kolasa**, Principal Senior Consultant, Market Access, Straub Medical, Switzerland

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.

Panel Discussion with Q&A

Following questions will be addressed:

- How Can RWD Uncover Real Unmet Medical Needs in R&D Process?
- Shall We Rethink RWD in the Era of Health's Digitalization?
- Can We Move from RWD to Real World Decision Making?
- How to Redefine RWD to Ensure Its Usability for R&D and P&R Decision Making Processes?

Panellists:

François Meyer, Advisor to the President, International Affairs, French National Authority for Health (HAS), France

Radek Wasiak, Vice President and General Manager, Real-World Evidence and Meta Research, Evidera, United Kingdom

Vladimir Zah, Chief Executive Officer, ZRx Outcomes Research, Canada; Research Member, ISPOR Health Science Policy Council, Serbia

Michael Schröter, Head of Personalised Reimbursement Models, Global Pricing and Market Access, F. Hoffmann-La Roche, Switzerland

Stephane Regnier, Head of HEOR Excellence, Global Patient Access, Novartis Pharmaceuticals, Switzerland

12:30 LUNCH

14:00 SESSION 7

RELATIVE EFFICACY VERSUS RELATIVE EFFECTIVENESS – THE FUTURE

Session Chair: **François Meyer**, Advisor to the President, International Affairs, French National Authority for Health (HAS), France

- Current challenges in the assessment of relative efficacy/effectiveness
- The need for comparative data
- Role of EUnetHTA moving forward
- Cooperation between EMA and EUnetHTA: current status and perspectives

Panellists:

Leeza Osipenko, Head of Scientific Advice, National Institute for Health and Care Excellence (NICE), United Kingdom

Kristin Svanqvist, Head of Unit for HTA and Reimbursement, Norwegian Medicines Agency (NoMA), Norway

Michael Berntgen, Head of Product Development Scientific Support Department, European Medicines Agency (EMA), European Union

15:30 END OF CONFERENCE

Continuing Education

SwAPP and SGPM Credits

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This workshop has been accredited with 11.00 credits.

Evaluation

Your comments and views on the content and organisation of the event are highly valued. The evaluation form will be available online: http://bit.ly/2i13Ckm

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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.

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 basel@diaglobal.org or call +41 61 225 51 51.

About DIA

DIA is the global connector in the life sciences product development process. Our association of thousands of 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 headquartered in Washington, DC, USA with the EMEA office in Basel, Switzerland, and additional regional offices in Horsham, Pennsylvania, USA; Tokyo, Japan; Mumbai, India; and Beijing, China.