ADAPTIVE PATHWAYS WORKSHOP

Stockholm • Friday, November 10, 2017 • 9:00–13:00

Adaptive Pathways
Applications for Scientific Insights in Europe

PRESENTED BY:

ICON

Mapi™
An ICON plc Company

Agenda Overview

VENUE: Sturegatan 15,
114 36 Stockholm

The workshop will end with a lunch served at 13:00. If you plan to attend the lunch, please register on our webpage:

http://mapi.bz/apw

9:00–9:10	Welcome Message
9:10–9:30	Introduction AP Concept
9:30–9:55	1st Element: Iterative Model
9:55–10:20	2nd Element: RWE
10:20–10:45	3rd Element: Patient Involvement
10:45–11:00	Questions & Answers
11:00–11:20	Coffee Break
11:20–11:50	Scientific Advice
11:50–12:10	Trends & Rationale
12:10–12:30	Consequences
12:30–13:00	Q&A and Panel Discussion
13:00	Lunch

At today's patient-centered research workshop, experts from across the industry will share their knowledge, insights, and experience on a topic that is vital to a project's success. As leading voices in patient-centered research, ICON and Mapi are happy to present this important workshop to all attendees.

Committed to Improving Health Outcomes

With patient involvement in healthcare decision making increasing, and payer influence on the rise, drug and medical device companies face a growing mandate to produce real world evidence that demonstrates product value and safety. ICON's recent acquisition of the Mapi Group will help address that need.

At ICON and Mapi, we know that research centered on people is crucial to improving world health. Our unrivaled four decades of operational expertise in generating, synthesizing, and analyzing real world evidence and health economics outcomes research has established us as the industry leader and recognized innovator of outcomes research methodologies and sciences.

Our research solutions bridge the distance between life science companies and patients, enabling market access and navigating the complex global regulatory environment. Our commitment runs deep. We are the only health research company with a direct-funded non-profit organization dedicated to improving patient outcomes and quality of life. The Mapi Research Trust supports thousands of independent and academic research programs every year in over 130 countries, offering free and subsidized access to clinical outcomes assessments (COA), their derivatives, and translations.

Presenters Include:

Mia Malmenäs
Director,
Real World Strategy and Analytics, Mapi Group

Nahila Justo
Scientific Director,
Real World Strategy and Analytics, Mapi Group

Karolina Antonova
Head of Strategy,
Läkemedelsindustriföreningen (LIF)

Dr. Will Maier
Chief Scientific Officer, Director Patient-Centered Sciences,
Mapi Group

Dr. Matthew Bending
Director,
Real World Strategy and Analytics, Mapi Group

Prof. Michael Drummond
Professor,
Center for Health Economics, University of York

Dr. Joakim Ramsberg
Principal Secretary,
Commission on Financing, Reimbursement and Pricing of Pharmaceuticals

Chief Scientific Officer,
Swedish Agency for Health and Care Services Analysis

Introduction & Agenda

For the past five to ten years we have witnessed a heightened interest in improving timely access for patients to new medicines. On the one hand, the European Medicines Agency (EMA) started introducing regulatory processes aiming at cutting down lead-time for marketing authorization in indications with high unmet medical needs with the introduction of initiatives such as the Compassionate Use Programs (based on Regulation (EC) No 726/2004), the Conditional Approval Mechanism (based on Regulation (EC) No 507/2006), and the Initiative for Patient Registries launched in September 2015. On the other hand, the United States Food and Drug Administration (FDA) developed four approaches, which were formalized with the adoption of FDA Safety Innovations Act in 2012, known as Priority Review, Breakthrough Therapy, Accelerated Approval, and Fast Track.

Now, the paradigm is rapidly shifting as last year this trend found culminating expressions on both sides of the Ocean when Europe launched the adaptive pathways (AP) approach and the US passed the 21st Century Cures Act. There are elements of similarities and contrasts between the two but the endeavors go in the same direction and require similar efforts from all counterparts; that is:

- a concerted approach during the complete life-cycle of the products (from clinical development to market adoption),
- the need to complement trial data with real-world evidence,
- and the acknowledgement of patients' centeredness.

Focusing on Europe, according to EMA adaptive pathways is a scientific concept for medicine development and data generation which allows for early and progressive patient access to a medicine and it is based on three principles:

- early involvement of patients and health-technology-assessment bodies in discussions on a medicine's development;
- gathering evidence through real-life use to supplement clinical trial data; and
- iterative development, which either means approval in stages, beginning with a restricted patient population then expanding to wider patient populations; confirming the benefit-risk balance of a product, following a conditional approval based on early data (using surrogate endpoints) considered predictive of important clinical outcomes.

Objectives

Today's workshop in Stockholm is aimed at presenting the different elements of the adaptive pathway concept, as well as discussing its implications and expected impact in the adoption of new innovative treatments in Europe.

9:00–9:10 **Welcome Message**
Presented by: Mia Malmenäs, Mapi Group

9:10–9:30 **Introduction AP Concept**
Presented by: Nahila Justo, Mapi Group

9:30–9:55 **1st Element: Iterative Model**
Presented by: Karolina Antonova, Läkemedelsindustriföreningen (LIF)
Challenges and experiences of national implementation of AP

9:55–10:20 **2nd Element: RWE**
Presented by: Mia Malmenäs, Mapi Group
How secondary data such as Nordic and European registries can support AP discussing challenges and opportunities

10:20–10:45 **3rd Element: Patient Involvement**
Presented by: Will Maier, Mapi Group
Discussing both dimensions, the early involvement of patient representatives as an interlocutor in the assessment and the enhanced use of PROs

10:45–11:00 **Questions & Answers**

11:00–11:20 **Coffee Break**

11:20–11:50 **Scientific Advice**
Presented by: Dr. Matthew Bending, Mapi Group
The adaptive pathway development should be iterative and as such plans for demonstration and change of the value within the evolving data set can/needs be discussed with HTAs.

What is the role of integrated scientific advice in adaptive pathways and the key HTA questions and issues considered?

11:50–12:10 **Trends & Rationale**
Presented by: Prof. Michael Drummond, University of York
Accelerated Access Programs for Innovative Drugs: Have they achieved their objectives and what next?

12:10–12:30 **Consequences**
Presented by: Dr. Joakim Ramsburg, Swedish Agency for Health and Care Services Analysis
Consequences downstream: the payer perspective

12:30–13:00 **Questions & Answers and Panel Discussion**

13:00 **Lunch**

Presenters

Dr. Joakim Ramsberg

Principal Secretary,
Commission on Financing, Reimbursement and Pricing of Pharmaceuticals

Chief Scientific Officer,
Swedish Agency for Health and Care Services Analysis

Joakim Ramsberg, Ph.D., is Principal Secretary in the Government commission on Financing, reimbursement and pricing of pharmaceuticals. He is project director and chief scientific officer at Vårdanalys (The Swedish Agency for Health and Care Services Analysis), where he has lead projects that e.g., evaluate national clinical guidelines, examine disease registries, and assess government reforms to increase access and strengthen psychiatric care. He is a lecturer at the Department of Population Medicine, Harvard Pilgrim Healthcare Institute and Harvard Medical School where he also was the 2015-16 Swedish Harkness Fellow in Health Care Policy and Practice.

Mia Malmenäs

Director
Real World Strategy & Analytics, Mapi Group

Mia Malmenäs has over 17 years of experience from the pharmaceutical industry analyzing and designing clinical trials, retrospective data sources and observational studies, and validation of Patient Reported Outcomes/Clinical Outcome Assessments. She has published over 10 journal articles and been an author on more than 15 posters. Her publications reflect her research interests in retrospective data analysis as well as study design methodology. Currently, Mia is co-chairing one work stream in the ISPOR Medication Adherence and Persistence Special Interest Group and she has chaired 3 workshops and led one course within the same organization.

Nahila Justo

Scientific Director
Real World Strategy and Analytics, Mapi Group

Nahila Justo is a PhD candidate at the Karolinska Institute and has a MBA from the Stockholm School of Economics (Sweden), an MPhil in European Affairs from the University of Salamanca (Spain) and a MSc in Economics from the Inter-American Development Bank and the Di Tella Institute (Argentina). Nahila also has undertaken post-graduate courses in Epidemiology and Biostatistics at the IECS Argentina (Instituto de Efectividad Clínica y Sanitaria) and holds a MA in International Relations and a BA in Political Science from National University of Rosario, Argentina. She currently serves as Scientific Director at Mapi Real World Evidence Strategy and Analytics.

Karolina Antonov

Head of Strategy
Läkemedelsindustriföreningen (LIF)

Karolina Antonov is Head of Strategy at Läkemedelsindustriföreningen (LIF), the trade association for the research-based pharmaceutical industry in Sweden. With about 85 members and associate companies, LIF represents approximately 80 percent of the total sales of pharmaceuticals in Sweden. LIF's primary responsibilities are pricing and reimbursement issues, but is involved in many of the activities within pharmaceutical strategy such as managing introductions, adapting pathways, and RED issues. LIF is also a member of the European trade association EFPIA, which works to improve access to medicines for all patients in Sweden.

Dr. Will Maier

Chief Scientific Officer, Director Patient-Centered Sciences
Mapi Group

Mapi's Chief Scientific Officer, Dr. Will Maier provides scientific leadership to Mapi's research and consulting services across a range of scientific areas. Dr. Maier has more than 20 years of drug development and commercialization experience. He served as Senior Director of Epidemiology at GlaxoSmithKline and Elan Pharmaceuticals and led research groups conducting observational research to support reimbursement, marketing and drug safety investigations of pharmaceuticals. Dr. Maier is a member of the EMA's European Network of Centres for Pharmacoepidemiology and Pharmacovigilance.

Dr. Matthew Bending

Director, Head of HTA, Strategy & Communication
Real World Strategy & Analytics, Mapi Group

Dr. Matthew Bending is an experienced health economist with over 12 years consultancy experience and is Head of the HTA, Strategy and Communications team and Director or Real World Strategy & Analytics. Matthew has let the development of early payer engagement services in Mapi and has experience of more than 25 HTA scientific advice engagements across a range of diseases areas. Matthew provides senior leadership for large projects on strategic market access and Health Technology Assessments (HTAs) for pharmaceutical, vaccine and medical device companies.

Prof. Michael Drummond

Professor
Center for Health Economics, University of York

Michael Drummond is Professor of Health Economics and former Director of the Centre for Health Economics at the University of York and part of the team from the University of York that produces independent Technology Assessment Reports for NICE. Professor Drummond has authored three major textbooks and co-authored more than 400 publications in peer-reviewed scientific journals. Professor Drummond has consulted for the World Health Organization, the European Union, and numerous major pharmaceutical companies. He has served the Medicines Commission in the United Kingdom, the Board of the International Society of Technology Assessment in Health Care (ISTAHC) and the Board of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR).

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Thank You For Attending

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