Regulatory Science Forum

28-30 September 2020

PROGRAMME COMMITTEE

Anne Morant
Regulatory Science Consultant representing H. Lundbeck A/S., Denmark

Anthony Humphreys
Head of Scientific Committees Regulatory Science Strategy, EMA, EU

Helga Gardarsdottir
Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands

Inka Heikkinen
Associate Director, Global Regulatory Policy, MSD, Denmark

Marieke De Bruin
Director, Copenhagen Centre for Regulatory Science, University of Copenhagen, Denmark

Marjon Pasmooij
Steering Group Member of the Regulatory Science Network Netherlands, Science Programme Manager, Medicines Evaluation Board, the Netherlands

Sara Torgal
Scientific Programmes Manager, DIA EMEA

Sini Eskola
Director Regulatory Affairs, European Federation of Pharmaceutical Industries and Associations (EFPIA), Belgium

Who Will Attend?

Professionals involved in:

- Regulatory Science
- Regulatory Strategy
- Regulatory Intelligence
- Scientific Advice
- Data analysis
- Policy
- Patient Engagement

Overview

The Regulatory Science Forum is a platform for sharing knowledge derived from regulatory science research and analyses conducted at the academic, industry and regulatory levels, promoting open dialogue between all stakeholders. The Forum will discuss where more research is needed, as well as the best ways to promote the widespread implementation of findings, recommendations, and other results.

Regulatory science informs and investigated issues and challenges related to regulatory approvals, assessment and evaluation. It can help your company to better understand the issues you are facing and find more effective solutions to getting your medicines to patients faster.

Objectives

By attending this meeting, participants will be able to:

- Understand how Regulatory Science is used by the different stakeholders and how it informs decision making and policy;
- Discuss how to maximize this work across all stakeholders;
- Become aware of the Regulatory Science research status in Europe, how to use it in a broader spectrum and how to connect it to the bigger common goals;
- Recognize what Regulatory Science means from the Industry, Regulator & Academia perspectives;
- Discover how Regulatory Science informs and investigates matters and challenges related to regulatory approvals, assessment and evaluation;
- Interact with other stakeholders and explore practical ways to increase that.

Key Topics

- Why is Regulatory Science research important?
- Regulatory Science Training and Network in European Academia
- Maximizing the Value and Impact of Regulatory Science Research
- HTA-Regulatory Cooperation
- Stakeholder & Patient Engagement
- Body of Evidence
- Complex Clinical Trials
- Pharmacovigilance
- Labelling

14:00   LOG-IN AND ORIENTATION

14:10   INTRODUCTION TO THE DIA REGULATORY SCIENCE FORUM

Sara Torgal, Scientific Programmes Manager, DIA EMEA
Helga Gardarsdottir, Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands

14:30   KEYNOTE

Professor Hubert Leufkens, Professor, Pharmaceutical Policy and Regulatory Science, Utrecht University, the Netherlands

14:50   SESSION 1

SETTING THE SCENE - WHY IS REGULATORY SCIENCE RESEARCH IMPORTANT?

Session Chair:
Sini Eskola, Director Regulatory Affairs, European Federation of Pharmaceutical Industries and Associations (EFPIA), Belgium

Regulatory Science is a field looking into developing new tools, standards and approaches to evaluate the efficacy, safety, quality and performance of medicines in order to assess their benefit-risk balance and facilitate a sound and transparent regulatory decision-making. It is the basis for the pharmaceutical regulation to evolve. It can be a fast-moving and exciting research area where for example advances in data analytics, ability to collect the real-world data and turn it into the real-world evidence to support regulatory decision making and rapidly advancing science are transforming the way new treatments are developed. To remain competitive, all regions have to continue to evolve their respective regulatory system to remain at the cutting edge of regulatory science and policy. It also requires collaboration between the regulators, academia, industry and patients. This session explores examples on what major regions and stakeholders are doing to advance their priority topics in regulatory science research and how that contributes to policy making and current practices/regulation.

EMA Regulatory Science Strategy 2025
Anthony Humphreys, Head of Scientific Committees Regulatory Science Strategy, EMA, EU

Panel discussion with Q&A, with the participation of:
Anthony Humphreys, Head of Scientific Committees Regulatory Science Strategy, EMA, EU
Gerald Dal Pan, Director, Office of Surveillance & Epidemiology, FDA's Center for Drug Evaluation and Research, USA
Professor Hubert Leufkens, Professor, Pharmaceutical Policy and Regulatory Science, Utrecht University, the Netherlands
Susan Longman, Head Regulatory Affairs Region Europe, Novartis Pharma AG, Switzerland

16:00   BREAK

16:15   SESSION 2

COMPLEX CLINICAL TRIALS

Session Chair:
Marjon Pasmooij, Steering Group Member of the Regulatory Science Network Netherlands, Science Programme Manager, Medicines Evaluation Board, the Netherlands

CTFG Recommendation Paper on the Initiation and Conduct of Complex Clinical Trials
Massimiliano Sarra, Permanent official and CTFG Secretary, Italian Medicine Agency (AIFA), Italy

EPFIA perspective on Complex Clinical Trials
Nick Sykes, Senior Director, Worldwide Safety & Regulatory, Pfizer, UK

Panel discussion with Q&A

17:15   BREAK

17:30   SESSION 3

NANOSIMILARS

Session Chair:
Jon de Vlieger, Coordinator NBCD Working Group and Director Business Development, Lygature, the Netherlands

Identification of regulatory needs for nanomedicines
Susanne Bremer-Hoffmann, European Commission Joint Research Centre (JRC), Italy

The EU regulatory landscape of non-biological complex drugs (NBCDs) follow-on products: Observations and recommendations
Kevin Klein, Utrecht Institute for Pharmaceutical Sciences (UIPS), Division of Pharmacoepidemiology and Clinical Pharmacology, Utrecht University, the Netherlands

EMA's approach to the Evaluation of Medicines containing Nanotechnology
Falk Ehmann, Chair of Innovation Task Force, European Medicines Agency, EU

Panel discussion with Q&A

18:30   WRAP-UP

18:40   END OF DAY ONE

14:00   **WELCOME**

Sara Torgal, Scientific Programmes Manager, DIA EMEA
Helga Gardarsdottir, Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands

14:10   **SESSION 4**

BODY OF EVIDENCE

Session Chair:

Anne Vinther Morant, Regulatory Science Consultant representing H. Lundbeck A/S., Denmark

The 'Body of Evidence' session will revolve around generation of substantial evidence of clinical efficacy to support approval of new medicines. With focus on EMA and FDA, the presenters will share results of their regulatory science research on topics such as approvals based on a single pivotal trial; demonstration of clinical relevance; as well as pre- and post-approval evidence pertaining to use of expedited pathways.

Systematic analysis of evidence supporting expedited approvals by the EMA 2011-2018 and fulfilment of post-marketing obligations
Petra Sevcikova, Honorary Senior Researcher, Population Health Sciences Institute, Newcastle University, UK

Clinical Value Of Oncology Medicines In Europe: Is It Different For Conditionally Approved Medicines?
Lourens Bloem, PhD Candidate, Division of Pharmacoepidemiology and Clinical Pharmacology, Utrecht Institute for Pharmaceutical Sciences, Utrecht University, the Netherlands

Providing Substantial Evidence with a single pivotal trial: Discussion of Regulatory trends in the EU and US
Katrine Schultz-Knudsen, Regulatory Science & Advocacy Department, H. Lundbeck A/S, Denmark

Clinical relevance: an analysis of EMA and FDA Advisory Groups in support of new Marketing Authorisation applications between 2011 and 2015
João Duarte, Director, Chief of Staff & Regulatory Science, Global Regulatory Affairs, Alexion Pharmaceuticals, France

15:20   **BREAK**

15:35   **SESSION 5**

STAKEHOLDER & PATIENT ENGAGEMENT

Session Chair:

Marjon Pasmooij, Steering Group Member of the Regulatory Science Network Netherlands, Science Programme Manager, Medicines Evaluation Board, the Netherland

Patient Involvement in Regulatory Procedures and Impact on Regulatory Decision Making
Sharon Gorman, Director, Global Regulatory Policy and Intelligence, Pfizer, UK

Views of European Drug Development Stakeholders on Treatment Optimization and Its Potential for Use in Decision-Making
Robbe Saesen, PhD researcher, Pharmaceutical and Pharmacological Sciences, KU Leuven, EORTC, Belgium

Stakeholder Perspectives on Implementing Precision Medicine in Diabetic Kidney Disease
Elisabeth Bakker, PhD Student, Clinical Pharmacy and Pharmacology, University Medical Centre Groningen, the Netherlands

Study to support the evaluation of the EU Orphan Regulation
Thyra de Jongh, Principal Consultant, Technopolis Group, the Netherlands

16:45   **BREAK**

16:55   **SESSION 6**

REGULATORY SCIENCE TRAINING AND NETWORK IN EUROPEAN ACADEMIA

Session Chair:

Beatriz Silva Lima, NDA Advisory Board and Universidade de Lisboa, Portugal

An outline of relevant training/research integrated actions within European (academic) institutions in the domain of regulatory Science. This session will further discuss next steps in further developing regulatory science as a discipline considering emerging technological and scientific advances.

RegSci Training actions in Denmark
Marie Louise de Bruin, CORS, University of Copenhagen, Denmark

RegSci Training actions in Portugal and beyond
Beatriz Silva Lima, NDA Advisory Board and Universidade de Lisboa, Portugal

Joint University and Agency RegSci training in the Netherlands
Marjon Pasmooij, Science Programme Manager, Medicines Evaluation Board, Utrecht, the Netherlands

EUPATI Training
Annemiek van Rensen, EUPATI Netherlands, the Netherlands

Panel discussion with Q&A

18:00 **BREAK**

18:10 **SESSION 7**

PHARMACOVIGILANCE

Session Chair:

Helga Gardarsdottir, Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands

17 Signals from a national spontaneous reporting system; What regulatory action do they lead to?
Florence van Hunsel, Head Signal Detection, Netherlands Pharmacovigilance Centre Lareb, the Netherlands

Do labels of drugs with the same mechanism of action describe the same adverse drug reactions? A case study on TNF- inhibitors
Lotte Minnema, PhD Candidate, University of Utrecht & Pharmacovigilance Assessor, Department Pharmacoepidemiology and Clinical Pharmacology, Medicines Evaluation Board, the Netherlands

The Quality and Robustness of Evidence used to support Regulatory Pharmacovigilance Decisions
Saad Shakir, Professor, Drug Safety Research Unit (DSRU), UK

19:10 **WRAP-UP**

19:20 **END OF DAY TWO**

14:00 **WELCOME**

Sara Torgal, Scientific Programmes Manager, DIA EMEA
Helga Gardarsdottir, Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands

14:10 **SESSION 8**

INTERFACE BETWEEN AUTHORISATION AND HTA

Session Chair:

Inka Heikkinen, Associate Director, Global Regulatory Policy, MSD, Denmark

Regulatory and HTA/payers underpin their respective assessment and decision-making on increasing novel evidence sources. This session will discuss the interface and gaps between authorisation and reimbursement and the consequences for the predictability of decision making. So that patients can gain more timely access to beneficial treatments.

Decision-making under uncertainty: comparing regulatory and health technology assessment reviews of medicines in the US and Europe
Rick Vreman, PhD Candidate, Division of Pharmacoepidemiology and Clinical Pharmacology, Utrecht University, the Netherlands

Managing product approval and reimbursement requirements from the developer's side, current challenges and future opportunities
Magdalini Papadaki, Associate Director, Regulatory Affairs International, MSD, UK

Interface between Authorisation and HTA
Michael Berntgen, Head of Scientific Evidence Generation Department, EMA, EU

ZIN/MEB Pilot collaboration – Lessons learned & Link to Regulatory Science
Pauline Pasman, Project leader MEB-ZIN, Dutch National Health Care Institute (ZIN), the Netherlands

Panel discussion with Q&A, with the additional participation of:
Wim Goettsch, Special Advisor HTA, Dutch National Health Care Institute (ZIN), the Netherlands
Edith Frenoy, Director Market Access, HTA Policy Lead, EFPIA, Belgium
Ansgar Hebborn, Head European Access Policy Affairs, F. Hoffmann-La Roche AG, Switzerland

15:30 **BREAK**

15:45 **SESSION 9**

LABELLING

Session Chair:

Anne Vinther Morant, Regulatory Science Consultant representing H. Lundbeck A/S., Denmark

The 'Labelling' session will include presentations of regulatory science research focusing on how clinical evidence of efficacy and safety translates into label claims for communication to health care providers and patients. Three different angles will be covered: Patient Experience Data, Real-World Evidence, and post-approval label changes related to safety. A brief panel discussion will to provide the opportunity for discussion and Q&As.

Introduction of safety-related changes to the drug label following large post-marketing cardiovascular outcome trials
Fatima Tarrahi, Masterstudent intern, Cardiovascular Department, CBG-MEB (Medicines Evaluation Board), the Netherlands

Reflection of Real-World Evidence in the product information
Maren Koban, Associate Director, Global Regulatory And Scientific Policy, Merck Helthcare KGaA, Germany

FDA's Patient-Focused Drug Development Initiative: Does Patient Experience Data Translate into Drug Label Claims?
Anders Blædel Lassen, Senior Director, Patient Insights. H. Lundbeck A/S., Denmark

16:45	**BREAK**

16:55	**SESSION 10**

NEXT STEPS TOWARDS MAXIMIZING THE VALUE AND IMPACT OF REGULATORY SCIENCE RESEARCH

Session Chair:
Professor Hubert Leufkens, Professor, Pharmaceutical Policy and Regulatory Science, Utrecht University, the Netherlands

Regulatory Science Research is an important driver for improvement of the Drug Regulatory System. Translation of the scientific findings to regulatory practice is a crucial step. This session will bring together the end-users of the system and those conducting regulatory science research. Discussions and debate will focus on value creation: How to identify areas in need of regulatory science research? How to best translate findings? How to maximize impact?

Panel discussion with Q&A, with the participation of:
Anthony Humphreys, Head of Scientific Committees Regulatory Science Strategy, EMA, EU
Peter Mol, Principal Clinical Assessor, University Medical Center Groningen; Member SAWP; CBG-MEB (Dutch Medicines Evaluation Board), the Netherlands
Helga Gardarsdottir, Associate Professor in Drug Regulatory Sciences, Utrecht University, the Netherlands
Virginie Hivert, Therapeutic Development Director, Eurordis, France
Alan Morrison, Vice President Regulatory Affairs Intl, MSD (EFPIA Chair for Regulatory Strategy Committee)
Michael Nguyen, M.D., FDA Sentinel Program Lead, Deputy Director, Regulatory Science Staff/OSE/CDER/FDA, CDR, US Public Health Service, USA

17:55	**WRAP-UP**

18:10	**END OF THE FORUM**

| Disclosure Policy

| 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 that has its Global Center in Washington, DC, USA and the Europe, Middle East and Africa office in Basel, Switzerland. DIA has additional regional offices in Horsham, Pennsylvania, USA; Tokyo, Japan; Mumbai, India; and Beijing, China. For more information, visit www.DIAglobal.org or call DIA EMEA: +41 61 225 51 51.