EMA/DIA Statistics Forum: The Role of Observational Data in Assessing the Benefits and Risks of Drugs

Course # 17593
1 December 2017
European Medicines Agency, London, United Kingdom

PROGRAMME CHAIR

Jim Slattery
Statistician, Surveillance and Epidemiology, Pharmacovigilance Department, European Medicines Agency (EMA), EU

PROGRAMME COMMITTEE

Sigrid Behr
Group Head Quantitative Safety & Epidemiology, Novartis, Switzerland

Hans Ulrich Burger
Global Head Biostatistics Neuroscience and Ophthalmology, F. Hoffmann-La Roche, Switzerland

Stephen Evans
Professor of Pharmacoepidemiology, London School of Hygiene and Tropical Medicine, UK

Jürgen Kübler
Quantitative Scientific Consulting, Germany

Andrew Thomson
Statistician, European Medicines Agency (EMA), EU

Richardus Vonk
Head of Research and Clinical Sciences Statistics, Bayer, Germany

DETAILS OF THE INFORMATION DAY

Location:

European Medicines Agency
30 Churchill Place
Canary Wharf
London E14 5EU,
United Kingdom

OVERVIEW

Analyses of observational data have played a central role in assessing post authorisation drug safety for many years. However, systematic collection of clinical practice records in electronic form has increased the range and quality of possible analyses and the use of such data in other areas of research. This raises important questions for those designing and conducting the research and those attempting to base regulatory decisions on the results.

Topics include:

- How to select the most reliable methods from the diverse range of study designs used in observational analyses
- Which types of questions can be addressed using observational data alone and which require interventional techniques and, in particular, randomisation of alternative treatments
- How the observational data can best be used to enhance and extend the current methods used in clinical research
    - o Follow-up through routine clinical data collection
    - o Value of additional non-randomised control data: concurrent or historical
- How to assess research based on observational studies and, in particular, how to pre-specify criteria of 'success' in an observational study in a way that would allow confident design of a research programme to meet regulatory requirements

WHO SHOULD ATTEND

Statisticians and researchers involved in drug research based on observational data such as Real-World Data Specialists, Statisticians in R&D, Epidemiologists, and PRAC members

EUROPEAN MEDICINES AGENCY
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08:30 WELCOME COFFEE

09:00 SESSION 1

INTRODUCTION
Chair:
Jim Slattery, Statistician, Surveillance and Epidemiology, Pharmacovigilance Department, European Medicines Agency (EMA), EU

Controlled clinical trials are considered as gold standard in drug development and this session will explore the potential role of observational evidence in a regulated environment.

Taking the totality of evidence into account in a systematic way for decision making at all stages of drug development, the session discusses innovative approaches to combine information from randomised trials and observational studies.

Observational data may be considered the best available / achievable information in a number of situations; recent examples and experiences for the use of observational data will be presented.

Need for Observational Evidence
Anja Schiel, Senior Adviser / Statistician, Unit for HTA and reimbursement, Department for Pharmacoeconomics, Norwegian Medicines Agency, Norway; Chair of EMA's Biostatistics Working Party

What Do We Need to Be Able to Do to Formalise Use of Observational Evidence?
Rob Hemmings, Unit Manager, Statistics and, Pharmacokinetics Unit, MHRA, UK; Chair of EMA's Scientific Advice Working Party

FDA Priorities and Action Plan Regarding Observational Data
Mark Levenson, Director, Division of Biometrics VII, Office of Biostatistics, CDER, FDA, USA

10:00 COFFEE BREAK

10:30 SESSION 2

METHODS/STRENGTH OF EVIDENCE - PART 1
Chair:
Sigrid Behr, Group Head Quantitative Safety & Epidemiology, Novartis, Switzerland

Randomised controlled trials are sometimes not feasible or not sufficient to fully assess the benefits and risks of a new drug. But how can observational data be incorporated in the pre- and post-approval data generation plan? This session will discuss frameworks for the use of observational data and provides guidance on how to enhance the reliability.

Threshold Crossing - A Useful Way to Incorporate Historical Data in Pivotal Trials?
Martin Posch, Head, Center for Medical Statistics, Informatics, and Intelligent Systems Medical University of Vienna, Austria

Aspects of Observational Study Design that Enhance Reliability
Olaf Klungel, Professor Pharmacoepidemiologic Methods, Pharmacoepidemiology & Clinical
Pharmacology, Utrecht University, Netherlands

The Importance of Prospective Observational Research in the Clinical Development of New Treatments
Mark Belger, Principal Research Scientist, European Statistics Group, Eli Lilly and Co. Ltd, UK

Identifying the Best Approach for Real-World Evidence Generation
Nicolle Gatto, Senior Director, Group Lead, WSS Epidemiology, Pfizer Inc., USA

12:00 LUNCH

13:00 SESSION 3

METHODS/STRENGTH OF EVIDENCE - PART 2
Chair:
Hans Ulrich Burger, Global Head Biostatistics Neuroscience and Ophthalmology, F. Hoffmann-La Roche, Switzerland

The totality of evidence into account in a systematic way for decision making at all stages of drug development will be discussed as well as innovative approaches to combine data from randomised trials and observational data.

Recently Developed Designs of Late-Phase Clinical Trials
Jonathan Alsop, Founder, Numerus, UK

Embedding Randomised Clinical Trials into Clinical Practice
Mario Ouwens, Statistical Science Director, AstraZeneca, Sweden

Cross Design Synthesis Method of Data from Randomised Clinical Trials and Observational Studies
Christoph Gerlinger, Senior Director, Statistics, Bayer, Germany

14:30 COFFEE BREAK

15:00 SESSION 4

EXAMPLES
Chair:
Andrew Thomson, Statistician, Biostatistics & Methodology Support Office, European Medicines Agency (EMA), EU

Observational data where considered the best available / achievable information in a growing number of situations will be looked at. This session will also present recent examples and experiences for the use of observational data.

Observational Methods and Post-Authorisation Studies: Lessons Learned from Bosentan Risk Management
Erwan Muros-Le Rouzic, Associate Director Epidemiology, Actelion, Switzerland

An Example of a Single Arm Pivotal Study in a Rare Disease
Carol Reid, Associate Director, Biostatistics, Roche Products Ltd, UK

Do Dynamic Borrowing Methods Control the Risk of Using Historical Data?
David Dejardin, Biostatistician, F. Hoffmann-La Roche, Switzerland

16:00 SESSION 5

WHERE NEXT? PANEL DISCUSSION
Chair:
Jürgen Kübler, Quantitative Scientific Consulting, Germany

While much progress has been achieved over the past years, there is significant uncertainty about the role of observational data in drug development. An expert panel will discuss the current status and the way forward.

Panellists:
Sigrid Behr, Hans-Ulrich Burger, Stephen Evans, Rob Hemmings, Olaf Klungel, Mark Levenson, Anja Schiel, Jim Slattery

17:00 END OF FORUM

REGISTRATION FORM

ID #17593

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Industry	600.00 EUR ❑
Government/Academia/Charitable/Non-Profit (full time)	300.00 EUR ❑

*Registration fee includes: refreshments, sandwich lunch and delegate material

Payment is due 30 days after registration and must be paid in full by commencement of the event.

HOTEL INFORMATION

Participants are kindly requested to make their own hotel reservation.

ATTENDEE DETAILS

Please complete in block capital letters or attach the attendee's business card here.

❑ Prof ❑ Dr ❑ Ms ❑ Mr

Last Name

First Name

Company

Job Title

Address

Postal Code City

Country

Telephone

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Email*

*(Required for confirmation)

DIA reserves the right to include your name and affiliation on the attendee list.

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By signing below, I confirm that I agree with DIA EMEA Terms and Conditions of booking. These are available from the office or on http://www.diaglobal.org/EUTerms

Date	Signature

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- Industry (Member/Non-member) € 200.00
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The DIA Europe, Middle East & Africa Contact Centre Team will be pleased to assist you with your registration from Monday to Friday between 08:00 and 17:00 CET.
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