Benefit-Risk Assessment from Inception to Maturation: Aligning Regulatory and Industry Goals

Tutorial: February 9 | Meeting: February 10-11 | Bethesda, MD



PROGRAM COMMITTEE

Bennett Levitan, MD, PhD

Director, Department of Epidemiology Janssen Research & Development

Lawrence Liberti, MS, RPh, RAC

Executive Director

Centre for Innovation in Regulatory Science (CIRS)

Marilyn A. Metcalf, PhD

Senior Director, Benefit Risk Evaluation GlaxoSmithKline

Rebecca A. Noel, DrPH, MSPH

Senior Research Scientist, Global Patient Safety Eli Lilly and Company

Register at diahome.org/BenefitRisk

DIA GLOBAL CENTER 21 Dupont Circle, NW, Suite 300 Washington, DC 20036

WORLDWIDE OFFICES

Basel, Switzerland | Beijing, China | Horsham, PA, USA Mumbai, India | Tokyo, Japan

OVERVIEW:

Join FDA, EMA, Health Canada, and other regulatory agencies plus patient, payor, industry, and academic representatives to discuss the progress and application of Benefit-risk assessment throughout the development and post-approval life cycle. The meeting will feature discussion of emerging sponsor and regulator expectations for Benefit-risk assessment during development; practical methods commonly used to characterize benefits, risks, and their balance in the peri-approval period; and communicating complex assessments transparently across the development life cycle.

FEATURED TOPICS:

- Evolving Expectations for Benefit-Risk
- Update on EMA Benefit-Risk Initiatives
- New Canadian Benefit Harm Uncertainty (BHU) Framework
- CIRS Initiative
- IMI PROTECT Initiative
- Expectations from Patient and Payor Perspectives
- Considerations for the Application of Benefit-Risk Assessment During Development
- Peri-Approval Benefit-Risk Assessment
- Post-Approval Benefit-Risk Assessment
- Q&A Session
- Plus Much More

LEARNING OBJECTIVES:

At the conclusion of this meeting, participants should be able to:

- Discuss the emerging expectations and requirements for Benefit-risk assessment and their applications by sponsors and regulators
- Explain when and how to apply methods commonly used to characterize Benefit-risk
- Identify ways to enhance transparency and communication of Benefit-risk decisions





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Type of Activity: Knowledge

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CONTINUING EDUCATION CREDIT ALLOCATION

Tutorial: Making Better Benefit-Risk Decisions Through the Development Life Cycle: IACET: .4 CEUs

Meeting: Benefit-Risk Assessment from Inception to Maturation: Aligning Regulatory and Industry Goals: IACET: 1.3 CEUs

Pharmacv:

Session 3: Widening the Perspective: Reflections from Vital Partners Beyond Industry and Regulators: 1.5 contact hours or .15 CEUs, 0286-0000-14-025-L04-P

Session 6: Post-Approval Benefit-Risk Assessment: 1.5 contact hours or .15 CEUs, 0286-0000-14-026-L04-P

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TUTORIAL | SUNDAY, FEBRUARY 9

12:00–1:00PM TUTORIAL REGISTRATION

1:00-5:00PM TUTORIAL

Making Better Benefit-Risk Decisions through the Development Life Cycle

INSTRUCTORS:

Richard Hermann, MD, MPH

Safety Science Physician AstraZeneca

Ian Hirsch, PhD

Senior Statistical Team Leader AstraZeneca, United Kingdom

Bennett Levitan, MD, PhD

Director, Department of Epidemiology Janssen Research & Development

Zhong Yuan, MD, PhD

Senior Director, Epidemiology Janssen Research & Development

Understanding how to integrate pharmaceutical Benefit-risk processes and methodologies into a coherent whole has become a vital skill for stakeholders. This tutorial will follow the progression of vignettes at three points in the development and post-approval life cycle, showing how a team might utilize various methodologies in context.

- (i) Development: As a team locks its assessment content in place, considers what endpoints to use and roughs out an approach for the assessment when only phase II data is available
- (ii) Peri-approval: As a team uses all data developed during its clinical program to develop Benefit-risk arguments to use in health authority submissions and advisory committee presentations.
- (iii) Post-approval in preparation for a PBRER: Using post-approval data sources, developing and presenting an integrated Benefit-risk assessment.

LEARNING OBJECTIVES:

At the conclusion of this tutorial, participants should be able to:

- Describe how to integrate pharmaceutical Benefit-risk processes and methodologies into a coherent whole
- Recognize and gain familiarity with the utility from different methods for Benefit-risk assessment at different points in the development life cycle



Drug-Induced Injury of Liver, Heart, Kidney, and Skin: Employing Recent Advances to Improve Patient Safety and Speed Up the Pipeline

May 7-9 | Bethesda, MD

Register at diahome.org/DILO









MONDAY, FEBRUARY 10

7:15-8:15AM REGISTRATION AND CONTINENTAL BREAKFAST

8:15-8:30AM WELCOME AND OPENING REMARKS

Rebecca A. Noel, DrPH, MSPH

Senior Research Scientist, Global Patient Safety Eli Lilly and Company

8:30-10:00AM KEYNOTE ADDRESS

Global Views of Benefit-Risk Assessment

SPEAKERS:

GLOBAL REGULATORY PERSPECTIVE

Hans-Georg Eichler, MD, MSc

Senior Medical Officer

European Medicines Agency, European Union

GLOBAL PATIENT PERSPECTIVE

Frank W. Rockhold, PhD

Senior Vice President, Global Clinical Safety and Pharmacovigilance GlaxoSmithKline

10:00-10:30AM REFRESHMENT BREAK

10:30AM-12:00PM SESSION 1

Part 1: Evolving Expectations for Benefit-Risk Assessment

SESSION CHAIR:

Lawrence Liberti, MS, RPh, RAC

Executive Director

Centre for Innovation in Regulatory Science (CIRS)

In this featured two part session global regulatory and industry representatives will provide detailed updates on the key Benefit-risk initiatives.

Update on EMA Benefit-Risk Initiatives

Hans-Georg Eichler, MD, MSc

Senior Medical Officer

European Medicines Agency, European Union

IMI PROTECT Initiative

Juhaeri Juhaeri, PhD

Vice President & Head Pharmacoepidemiology Sanofi

Health Canada's New Benefit Harm Uncertainty (BHU) Framework

Robyn R. Lim, PhD

Senior Science Advisor Office of Legislative and Regulatory Modernization Health Products and Food Branch Health Canada, Canada

12:00-1:30_{PM} LUNCHEON

Global Labeling 2014: Compliance in a Changing Regulatory Environment

Tutorial: April 8 | Meeting: April 9-10 Bethesda. MD

Register by March 18 to Save!



Register at diahome.org/Labeling



1:30-3:00PM SESSION 2

Part 2: Evolving Expectations for Benefit-Risk Assessment

SESSION CHAIR:

Rebecca A. Noel, DrPH, MSPH

Senior Research Scientist, Global Patient Safety Eli Lilly and Company

In this session global regulatory and industry representatives will continue to discuss updates on the key Benefit-risk initiatives. You will have the opportunity to pose questions to the presenters in a panel forum in this session.

CIRS Initiative

Lawrence Liberti, MS, RPh, RAC

Executive Director Centre for Innovation in Regulatory Science (CIRS)

FDA Perspective

Andrea Tan

Operations Research Analyst OSP, CDER, FDA

PANELISTS:

Hans-Georg Eichler, MD, MSc

Senior Medical Officer European Medicines Agency, European Union

Juhaeri Juhaeri, PhD

Vice President & Head Pharmacoepidemiology Sanofi

Robyn R. Lim, PhD

Senior Science Advisor Office of Legislative and Regulatory Modernization Health Products and Food Branch Health Canada. Canada

Lawrence Liberti, MS, RPh, RAC

Executive Director Centre for Innovation in Regulatory Science (CIRS)

Andrea Tan

Operations Research Analyst OSP, CDER, FDA

3:00-3:30PM REFRESHMENT BREAK

3:30-5:00_{PM} SESSION 3

Widening the Perspective: Reflections from Vital Partners Beyond Industry and Regulators

SESSION CHAIR:

Robyn R. Lim, PhD

Senior Science Advisor
Office of Legislative and Regulatory Modernization
Health Products and Food Branch
Health Canada

What do patients consider to be a medicine's most important positive and negative effects and uncertainties? Why do payors decide to reimburse a medicine? This session will reveal some perspectives beyond those of regulators and industry that are vital to achieving a future state in which the health community coordinates itself to best effect. We will explore opportunities for Benefit-risk approaches to incorporate and inform patients' views, improve medicines development, enhance medicines management, and optimize health outcomes.

SPEAKERS:

Payor Perspective

Edmund Pezalla, MD, MPH

National Medical Director for Pharmacy Policy and Strategy Office of the Chief Medical Officer

Patient Perspective

Louise Binder

International Community of Women Living With HIV/AIDS Canada

Linda Wilhelm

Consumer Advisory Council Canadian Arthritis Network, Canada

5:00-5:15PM CLOSING REMARKS

Lawrence Liberti, MS, RPh, RAC

Executive Director

Centre for Innovation in Regulatory Science (CIRS)

5:15-6:15_{PM} RECEPTION





TUESDAY, FEBRUARY 11

7:30-8:15AM REGISTRATION AND CONTINENTAL BREAKFAST

8:15AM-8:30AM OPENING REMARKS

Marilyn A. Metcalf, PhD

Senior Director, Benefit Risk Evaluation GlaxoSmithKline

8:30-10:00AM SESSION 4

Considerations for the Application of Benefit-Risk Assessment during Development

SESSION CHAIR:

Isabelle Stoeckert, PhD

Vice President, Head Global Regulatory Affairs Europe/Canada Bayer Pharma AG

Development of value adding drugs demands a clearly positive Benefit-risk balance during development, approval and life cycle of a medicinal product. Various regulatory relevant documents such as clinical trial applications and Development Safety Update Reports produced by sponsors during the development phase require an explicit statement on Benefit-risk. This session will address the challenges for companies to map out and continuously update and benchmark the expected key benefits and risks, whilst maintaining consistency in the relevant documents provided to the Regulatory Authorities. It will also take into account available regulator 's perspectives on how dialogue on the expected benefit-risk balance could be formalized already during development, e.g., during Scientific Advice/End of Phase II meetings, and what models or frameworks could be acceptable in this context. Patient centric drug development demands the elicitation of the patient 's perspective at an early stage, preferably prior to finalizing the Phase III design, and concrete examples for potential approaches will be provided.

Benefit-Risk Approaches as Enabler in Developing Value Adding Drugs

Leo Plouffe Jr. MD, CM, FACOG

Vice President

Head of Risk Management, Global Pharmacovigilance Bayer HealthCare Pharmaceuticals, Inc

Early Dialogue during Development on Expected Benefit-Risk Balance

Bruno Flamion, MD, PhD

NDA Regulatory Advisory Board
Past Chair of the Scientific Advice Working Party,
European Medicines Agency (EMA)
Past Chair of the Committee for Reimbursement of Medicines, Belgium
Professor of Physiology and Pharmacology
University of Namur, Belgium

How Do We Achieve More Patient Centric Drug Development

Pamela Berry

Director, Patient Reported Outcomes GlaxoSmithKline

10:00-10:30AM REFRESHMENT BREAK

10:30AM-12:00PM SESSION 5

Peri-Approval Benefit-Risk Assessment

SESSION CHAIR:

Bennett Levitan, MD, PhD

Director, Department of Epidemiology Janssen Research & Development

The culmination of drug and device development entails health authority assessment of a treatment's Benefit-risk balance. Sponsors' application are increasingly expected to defend whether a treatment's benefits outweigh its risks, accounting for not just clinical data but the nature of the medical condition, alternative treatments and risk mitigation plans. The growing body of regulatory and industry structured Benefit-risk frameworks provide some guidance for this process, but sponsors are struggling with questions on whether and how to prespecify a Benefit-risk approach, the balance between qualitative and quantitative approaches for the assessment, whether and how to incorporate patient perspectives, and how to concisely communicate a Benefit-risk argument. This session will review principles and examples from health authority submissions, describing methods that have been successful and the processes and practical concerns sponsors need to address to conduct them.

SPEAKERS:

Structured Benefit-Risk Assessment as an Inherent Part of a Dossier

Conny Berlin, MS

Global Head, Quantitative Safety Function Novartis Pharma AG

Regulatory Perspective

Joyce Korvick, MD

Deputy Director Division of Gastrointestinal Products OND, CDER, FDA

Patient Perspective

K. Kimberly McClearly

Director, Strategic Initiatives FasterCures/A Center of the Milken Institute



12:00-1:30рм

LUNCHEON

1:30-3:00PM

SESSION 6

Post-Approval Benefit-Risk Assessment

SESSION CHAIR:

Qi Jiang

Executive Director

Amgen Inc.

This session will address the ongoing collection and use of Benefit-risk information for specific applications such as PBRERS and PSURs, as well as more general safety surveillance of postmarketed products. Information will be relevant for mature products, vaccines, and devices.

Adaptive Licensing

John G. Ferguson, MD

Vice President, Global Head of Pharmacovigilance and Medical Safety **Novartis Vaccines**

Benefit-Risk Balance Assessment **During the Lifecycle of Medicinal Products**

Robert Ball, MD, MPH

Deputy Director Office of Surveillance and Epidemiology CDER. FDA

Capturing Benefits and Risks in the Post-approval Phase — **Practical and Regulatory Challenges**

Marilyn A. Metcalf. PhD

Senior Director, Benefit Risk Evaluation GlaxoSmithKline

3:00-3:30PM **REFRESHMENT BREAK**

3:30-5:00_{PM} **SESSION 7**

Panel Discussion

SESSION CHAIR:

Paul D. Huckle, PhD, Mpharm, RPh

Chief Regulatory Officer & Senior Vice President Global Regulatory Affairs GlaxoSmithKline

This session will consist of an open discussion of critical topics. Participants will have the opportunity to ask leading industry and regulatory experts questions on Benefit-risk assessment.

PANELISTS:

Robyn R. Lim, PhD

Senior Science Advisor Office of Legislative and Regulatory Modernization Health Products and Food Branch Health Canada, Canada

Andrea Tan

Operations Research Analyst OSP, CDER, FDA

Robert Ball, MD, MPH

Deputy Director Office of Surveillance and Epidemiology CDER, FDA

Bruno Flamion, MD, PhD

NDA Regulatory Advisory Board Past Chair of the Scientific Advice Working Party, European Medicines Agency (EMA) Past Chair of the Committee for Reimbursement of Medicines, Belaium Professor of Physiology and Pharmacology

University of Namur, Belgium

K. Kimberly McClearly Director, Strategic Initiatives FasterCures/A Center of the Milken Institute

5:00-5:15_{PM} **CLOSING REMARKS**

Bennett Levitan, MD, PhD

Director, Department of Epidemiology Janssen Research & Development

5:15PM **MEETING ADJOURNED**



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