EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Workshop on the use of Bayesian statistics in clinical development

17 June 2025 , 09:00 – 17:00 (CET/CEST)
Hybrid Meeting - Virtual/ EMA, Amsterdam, Room 1D

The proposed uses for Bayesian statistics in clinical development are increasing, leading to upcoming guidance from the EU Medicines Regulatory Network and ICH.

As part of the published Methodology Working Party 2025-2027 workplan and the Accelerating Clinical Trials in the EU (ACT EU) multi-annual workplan 2025-2026, a workshop on the use of Bayesian statistics in clinical development will be held on **17 June 2025** at EMA premises in Amsterdam.

The workshop aims to engage all stakeholders, including but not limited to patients, healthcare professionals, industry and academia.

The objectives of this workshop are to:

- Discuss the potential benefits and challenges when using Bayesian statistics in clinical development
- Discuss what information might be useful and relevant to include in upcoming guidance documents
- Better understand the trial designs that are being proposed and used, so that future guidance is fit for purpose

Workshop on the use of Bayesian statistics in clinical development – Draft Agenda

17 June 2025, 09:00 – 17:00 (CET/CEST)

Chaired by Peter Arlett (EMA)

08:30 Joining and technical checks

09:00 Welcome and opening remarks

Opening remarks **5′**
Peter Arlett (EMA)

ACT EU and the importance of methodological innovation **5′**
Marianne Lunzer (Clinical Trials Coordination Group chair and ACT EU Steering Group)

Bayesian Statistics in Regulation: Panacea or Pandora's Box? **10′**
Kristin Karlsson (Methodology Working Party Vice-Chair)

09:20 Session 1: Introduction to Bayesian statistics and decision making

Chairs: Frank Pétavy (EMA) and Aysun Cetinyurek Yavuz (MEB-CBG)

Bayesian borrowing in clinical trial test decisions: Frequentist type I error rate and power **15′**
Annette Kopp Schneider (German Cancer Research Center, Heidelberg)

Benefits and challenges of using Bayesian methods to support regulatory decision-making **15′**
Nicky Best (EFPIA/EFSPI)

Q&A **20′**

10:10 Coffee break

10:30 Session 2: Upcoming Guidance – Challenges and Opportunities

Chairs: Martin Posch (Medical University of Vienna) and Juan Jose Abellan (EMA)

EU Concept Paper on the use of Bayesian statistics in clinical development **25′**
Peter van den Ven (MEB-CBG)

ICH E20 – an industry perspective **25′**
Frank Bretz (EFPIA/EFSPI)

ICH E20 – a regulatory perspective **25′**
Frank Pétavy (EMA)

Q&A **30'**

12:15 Lunch break

13:00 Session 3: Use cases of Bayesian statistics I

Chairs: Tobias Fellinger (AGES) and Florian Lasch (EMA)

Bayesian methods without borrowing in ultrarare diseases **15'**
Natalia Muehlemann (EFPIA/EFSPI) and Jan Priel (EFPIA/EFSPI)

Bayesian adaptive design for a practice-changing platform trial in a rare paediatric cancer: The Glo-BNHL Trial **15'**
Lucinda Billingham (University of Birmingham)

Bayesian modelling to handle intercurrent events and facilitate interim decision making **15'**
Nicky Best (EFPIA/EFSPI) and Prashant Dalvi (EFPIA/EFSPI)

Q&A **30'**

14:15 Coffee break

14:30 Session 4: Use cases of Bayesian statistics II

Chairs: Peter van de Ven (MEB-CBG) and Juan Jose Abellan (EMA)

Leveraging information for a key secondary endpoint across adjacent populations **15'**
Simon Wandel (EFPIA/EFSPI) and Anastasia Lesogor (EFPIA/EFSPI)

Bayesian borrowing for pediatric extrapolation: the DINAMO study **15'**
Martin Oliver Sailer (EFPIA/EFSPI) and Igor Tartakovsky (EFPIA/EFSPI)

A Regulator's Perspective on Pediatric Bayesian Methods **15'**
James Travis (FDA)

Q&A **30'**

15:45 Coffee break

16:00 Session 5: Use cases of Bayesian statistics III

Chairs: Kristin Karlsson (Methodology Working Party Vice-Chair) and Manolis Efthymios (EMA)

Bayesian shrinkage methods for routine estimation of subgroup treatment effects **15'**
Björn Bornkamp (EFPIA/EFSPI) and John McMurray (University of Glasgow)

Application of ICH-M15 for Bayesian Modelling - Using a systematic model assessment framework to support design submission and discussion **15'**

Tobias Mielke (EFPIA/EFSPI)

Q&A **15'**

16:45 Closing remarks

Wrap up and key messages **15'**

TBC

About the chairs and speakers

Anastasia Lesogor

EFPIA/EFSPI

Anastasia Lesogor, MD works as Executive Director, Senior Global Program Clinical Head in Global Drug Development at Novartis Pharma. She is responsible for leading the development, registration, approval and life cycle management of RNA-based therapeutics in the atherosclerosis therapeutic area. Anastasia has 20+ years of experience in drug development with a proven track record of successfully leading large multinational teams across all functions and stages of global drug development, which has resulted in multiple global regulatory drug approvals, including new molecular entities and label extensions. Anastasia was trained in internal medicine. She received her post-graduate certificate in genetics at Harvard Medical School.

Annette Kopp- Schneider

German Cancer Research Center, Heidelberg

Prof. Dr. rer. nat. Annette Kopp-Schneider received a doctorate in mathematics and computer science from Rheinisch-Westfälische Technische Hochschule Aachen, Germany and the Habilitation in medical biometry at the Ruprecht-Karls-Universität Heidelberg, Germany. She leads the division of Biostatistics at the German Cancer Research Center (DKFZ). Her involvement as biostatistician for the pediatric oncology trial series INFORM2 at the KiTZ Hopp Children's Cancer Center Heidelberg has started her interest in Bayesian clinical trial design.

Aysun Cetinyurek Yavuz

MEB-CBG

Aysun Cetinyurek Yavuz is a biostatistician and she works as a methodology assessor at the Dutch Medicines Evaluation Board (MEB-CBG). Her expertise and interests include meta-analysis, Bayesian borrowing methods, use of external controls, and trials in rare diseases. Aysun is seconded from the Radboud University Medical Center where she works as a senior researcher and involved in teaching of clinical trials course and provides consultancy to cardiology group. She also supervises two PhD students performing research on the use of RWD/RWE in regulatory decision making.

Björn Bornkamp

EFPIA/EFSPI

Björn Bornkamp studied Statistics in Dortmund and now works in the Statistical Methodology Group at Novartis in Basel, where he provides consulting to statisticians and clinical teams on topics related to dose-finding studies, subgroup analyses, Bayesian statistics as well as estimands and causal inference.

Efthymios Manolis

EMA

Efthymios Manolis has served as a scientific officer at the European Medicines Agency (EMA) since 2007. He is the EMA lead in the methodology domain Modelling and Simulation (M&S) specialised interest area. Additionally, he is an expert in the ICH M15 Expert Working Group (EWG). Prior to his tenure at the EMA, Efthymios worked as a modeller in the industry. Efthymios has authored numerous articles in peer-reviewed journals.

He earned his pharmacy degree from the National University of Athens, Greece, in 2002, followed by an MSc in PK/drug metabolism and modelling from Paris XI University, France, in 2003.

Florian Lasch

EMA

Florian Lasch is a Biostatistician with a degree in mathematics and a PhD from Hannover Medical School. Florian works as a Biostatistics Specialist at the European Medicines Agency (EMA), providing scientific support throughout all stages of marketing authorisation assessments, and leads the EMA Estimands Implementation Group.

Frank Bretz

EFPIA/EFSPI

Frank Bretz is a Distinguished Quantitative Research Scientist at Novartis. He has supported the methodological development in various areas of drug development, including dose finding, estimands, multiple testing, and adaptive designs. He was a member of the ICH E9(R1) Expert Working Group on 'Estimands and sensitivity analysis in clinical trials' and currently serves on the ICH E20 Expert Working Group on 'Adaptive clinical trials'.

Frank Pétavy

EMA

Frank Pétavy is a mathematician and biostatistician by training. He has worked 14 years in all phases of clinical development in the Pharmaceutical Industry before moving to EMA in 2012 where he was Head of Methodology until last year. He is currently seconded part-time to the Dutch medicines Agency MEB where he acts as a statistical assessor in Scientific Advice and Marketing Authorisation Applications. He has a wide experience in the development and review of methodological, clinical and quality guidelines at both European and international levels. In particular, he is deputy topic lead of the ICH E20 expert working group on behalf of European regulators. Frank does not see himself as a frequentist or Bayesian, and aims to foster scientific-based medicine.

Igor Tartakovsky

EFPIA/EFSPI

Igor Tartakovsky works at Boehringer Ingelheim headquarters in Germany as a Global Clinical Program Lead in the therapeutic area "Cardio Renal Metabolism". Igor is board-certified in paediatric medicine and specialized in paediatric rheumatology. He has over 25 years of experience in clinical drug development in the fields of anticoagulation, diabetology, paediatrics, and rheumatology in the pharmaceutical industry and clinical field combined.

James Travis

FDA

James Travis is a master mathematical statistician in the Division of Biometrics II in the US FDA Center for Drug Evaluation and Research. He is the technical lead for the pediatric and maternal health statistical scientists and has been supporting pediatric drug development since joining the Pediatric Review Committee in 2017. He has worked extensively on pediatric extrapolation, including reviewing a range of applications that implemented Bayesian methods for this purpose

Jan Priel

EFPIA/EFSPI

Jan Priel earned his doctoral degree in Mathematics from University of Hamburg (Germany). He is a Principal Innovative Statistics Consultant in the Strategic Consulting group in Cytel. His specialties include Bayesian approaches to adaptive trial design and statistical modeling. As a statistical consultant, he has supported clients across a wide range of indications, including oncology, Alzheimer's disease, cardiovascular disease, sepsis, vaccines, neurological disorders, genetic disorders, immunological disorders, autoimmune disorders, mental health disorders, liver disease, and medical diagnostics, spanning from early Phase 1 to late Phase 3 stages of drug development.

John McMurray

University of Glasgow

Professor John McMurray is currently Professor of Medical Cardiology and honorary Consultant Cardiologist at the Queen Elizabeth University Hospital, Glasgow. He is and has been involved in many guideline committees, including chairing the Task Force for 2012 ESC Guidelines on heart failure and was a member of the 2016 and 2021 Task Forces. Professor McMurray is an Associate Editor for JACC-Heart Failure and is also a member of the editorial board of several other journals. He is the Congress Programme Committee Chair for the European Society of Cardiology 2023/2024 and European Society of Cardiology Board Member.

Juan Jose Abellan

EMA

Dr. Juan Jose Abellan is a mathematician and statistician by training. He has worked as a statistician in various roles in Public Offices, Academia and the Pharmaceutical Industry. He has worked in several scientific areas including Epidemiology and Clinical Drug Development, and he has made a number of contributions in those fields in peer-review scientific journals. He is currently working for the European Medicines Agency (EMA) based in the Netherlands. His main interests focus on methods for the generation of sound evidence to support regulatory decision making around the efficacy, effectiveness and safety of medicines.

Kristin Karlsson

Methodology Working Party Vice-Chair

Kristin Karlsson is currently employed as a senior assessor of pharmacometrics at the Swedish Medical Products Agency and is the vice-chair of the EMA Methodology Working Party. Kristin Karlsson has been part of the pharmacometrics community for 20 years and has experience with modelling and simulation within regulatory assessment, academic research, and the pharmaceutical industry. Kristin Karlsson has an MSc in Chemical Engineering and earned a PhD in Pharmacometrics at Uppsala University, Sweden. Furthermore, Kristin is the rapporteur of the ICH M15 expert working group (Model Informed Drug Development), and a previous member of the ICH E11A (paediatric extrapolation) expert working group and a former Swedish delegate of the EMA Paediatric Committee (PDCO).

Lucinda (Cindy) Billingham

University of Birmingham

Lucinda (Cindy) Billingham is a Professor of Biostatistics at the University of Birmingham. She has worked for thirty years as a Biostatistician at their Cancer Research UK Clinical Trials Unit and is now Director of Biostatistics for the Unit working with a large group of Biostatisticians on an extensive portfolio of early and late phase trials. She has expertise in the design and analysis of trials in rare cancers, application of Bayesian methods in trials, early phase trial design, statistical methods for the simultaneous analysis of quality of life and survival data and the evaluation of biomarkers in trials for stratified medicine.

Marianne Lunzer

AGES

Marianne is a Medical Doctor currently working as a safety assessor in the clinical trials department at Austrian Agency for Health and Food Safety (AGES) and Clinical Trials Coordination Group (CTCG) chair. She has been a Clinical Trials Facilitation and Coordination Group (CTFG) alternate since 2017 and a CTIS MS PO since 2019. Marianne also served as pharmacovigilance assessor (2008-2017) and was an alternate member of the Pharmacovigilance Risk Assessment Committee (PRAC).

Martin Posch

Medical University of Vienna

Martin Posch is Professor of Medical Statistics at the Medical University of Vienna and Head of the Center for Medical Data Science. His research focuses on innovative clinical trial designs, including platform trials, adaptive group sequential designs, and trials with multiple objectives. He is involved in several EU funded research consortia on complex trial design and analysis such as IHI RealiseD, IMPROVE, and SHARE-CTD, and has contributed to IMI EU-PEARL. From 2011 to 2012, he served as a statistical expert at the European Medicines Agency, contributing to guideline development and the assessment of study designs, and is currently a member of the EMA Methodology European Specialised Expert Community (ESEC).

Martin Oliver Sailer

EFPIA/EFSPI

Oliver Sailer is a Statistician with a PhD in Statistics from TU Dortmund University. He joined Boehringer Ingelheim in 2010 and worked as a trial and project Statistician in Oncology and Biosimilars. In 2017, he joined the Methodology Statisticians team where he provided consultation to trial and project Statisticians on various topics related to clinical trial development. Currently, he is the Chapter Head of a team of clinical data scientists and also co-leads a capability team that focuses on Bayesian borrowing for clinical trials.

Natalia Muehlemann

EFPIA/EFSPI

Natalia Muehlemann, MD, MBA, has over 20 years of experience in the Life Sciences across several therapeutic areas and combines medical, statistics, data science, and strategic expertise, enhancing value through evidence generation, advanced analytics, and stakeholder engagement. In her position of VP at Cytel, Natalia focuses on the integration of adaptive and innovative designs into clinical development strategies. Prior to Cytel, Natalia was Global Category Head, Acute Care – Oncology – Devices at Nestle Health Sciences. Natalia also acted as an Expert Jury member for the European Commission's European Innovation Council and SMEs Executive Agency and other investor forums.

Nicky Best

EFPIA/EFSPI

Nicky Best is VP and Head of Statistics and Data Science Innovation at GSK and has over 10 years' experience in the Pharmaceutical Industry. She has received several awards for her work on Bayesian methods in clinical trials, epidemiology and the pharmaceutical industry, including the RSS/PSI award for Statistical Excellence in the Pharmaceutical Industry and the Royal Statistical Society Bradford Hill Medal and Greenfield Medal. Before joining the pharmaceutical industry, Nicky was Professor of Statistics and Epidemiology at Imperial College London. Her academic research focused on development and application of Bayesian methods in health and social science; she has over 100 peer-reviewed publications and she is co-developer of the BUGS Bayesian software packag

Peter Arlett

EMA

Peter Arlett is Head of the Data Analytics and Methods Taskforce at the European Medicines Agency. In this role he leads on operations and transformation on clinical evidence at the EMA including clinical trials, real world evidence, safety reporting and data science including AI. He is Chair of the EMA Data Board, Co-Chair of the HMAEMA Network Data Steering Group, Co-chair of the EMA AI Coordination Group, Co-chair of the Vaccine Monitoring Platform Steering Group, and Member of the ACT EU Steering Group. Prior to taking up this role in 2020, he held leadership roles within the EMA in the areas of pharmacovigilance, epidemiology, and risk management. Prior to starting at EMA in 2008, Peter worked on new legislation and international collaboration for the European Commission, was the UK delegate to the European Committee for Human Medicinal Products, and was an assessor and manager at the UK's MHRA. He has a medical degree from University College London,and began his career as a hospital physician in Oxford and London. In addition to his role at EMA, Peter is Honorary Professor at the London School of Hygiene and Tropical Medicine. He is also a Fellow of the Royal College of Physicians of Edinburgh and of the Faculty of Pharmaceutical Medicines of London.

Peter van de Ven

MEB-CBG

Peter van de Ven is a seconded methodology assessor at the Dutch Medicines Evaluation Board (MEB-CBG) and a member of the temporary drafting group for the EMA concept paper on Bayesian methods in clinical development. He is an associate professor in clinical trial methodology and team leader at the University Medical Center Utrecht (UMCU). At UMCU, he and his team provide statistical expertise in the design and analysis of clinical trials and perform research on various innovations in clinical trial methodology.

Prashant Dalvi

EFPIA/EFSPI

Prashant is a trained physician with a MD in Clinical Pharmacology and holds diplomas in pharmaceutical medicine (FPM) and oncology (ICR). Over the last 14 years, he has worked in clinical development in a variety of settings with roles in industry, CRO and the UK national regulatory authority (MHRA). Over the last 3 years, he has worked as a clinical lead at GSK as part of Immunology and Respiratory teams on programmes across pre-clinical and clinical phases.

Simon Wandel

EFPIA/EFSPI

Simon Wandel holds a master in Statistics and a PhD in Medical Statistics/Epidemiology (both from University of Bern). He joined Novartis in 2010, where he held roles with increasing responsibility, before being appointed Global Group Head Biostatistics for atherosclerotic cardiovascular disease in 2021. He has a genuine interest in statistical methods for clinical trials, with a slight bias towards Bayesian approaches.

Tobias Fellinger

AGES

Tobias Fellinger is a statistician in the methodology and statistics group at the Austrian Medicines and Medical Devices Agency (AGES). He holds a masters degree in statistics from the University of Vienna. He previously worked at the Institute of Medical Statistics in the Center for Medical Data Science of the Medical University of Vienna.

Tobias Mielke

EFPIA/EFSPI

Tobias Mielke works as internal statistical consultant and methodological statistician at Johnson & Johnson. In his role, he actively supports drug development teams across multiple therapeutic areas in assessing and implementing innovative design and analysis methodology with particular focus on adaptive designs and multiplicity. Tobias joined Johnson & Johnson in 2018 from ICON Clinical Research, where he supported development of ADDPLAN DF, a software for the design and analysis of adaptive dose-finding trials using MCPMod. Tobias main scientific interest is in optimization of experimental designs to efficiently and effectively inform decision making in presence of uncertainty.