Bayesian Statistics in Regulatory Decision-Making: Panacea or Pandora's Box?

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Vice-chair Methodology Working Party

LÄKEMEDELSVERKET
SWEDISH MEDICAL PRODUCTS AGENCY

Methodology Working Party (MWP)

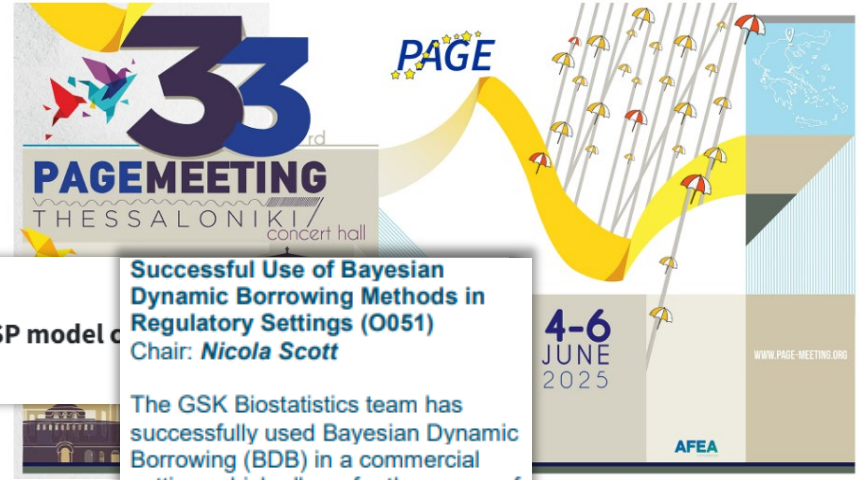
A forum to bring methodological experts together with the objective to strengthen methodological aspects in assessment and regulatory science.

- Modelling and simulation
- Biostatistics
- Pharmacokinetics
- Pharmacogenomics
- Diagnostics
- Real world evidence
- AI
- Data science

MWP supports the work of all Committees and provides methodological guidance across the medicine's development lifecycle.

MWP aims to leverage the cross-disciplinary expertise to support methodological innovation in global drug development and support advice and interpretation of complex methodology across (clinical) drug development.

What is the buzz all about?



PSI 2025

8 - 11 JUNE 2025

Borrowing Strength or Buying Trouble? Using External Data in Regulatory Context (O006)
Chair: ***Olivier Collignon***

Developing Treatments for Rare Pediatric Diseases Using Bayesian Extrapolation - ***Björn Bornkamp***

Improving Outlier Detection in Subgroup Analysis using Bayesian Predictive Cross-validation Models - ***Wilmar Igl***

Bayesian shrinkage estimation for subgroup analysis in clinical trials: Examining the critical aspects - ***Björn Bornkamp***

III-068 Paulo Paneque Galuzio

Bayesian optimization as an efficient tool for QSP model c

Thursday 9:50-11:45

Successful Use of Bayesian Dynamic Borrowing Methods in Regulatory Settings (O051)
Chair: ***Nicola Scott***

The GSK Biostatistics team has successfully used Bayesian Dynamic Borrowing (BDB) in a commercial setting, which allows for the re-use of external data, synthesising new and existing data to increase efficiency whilst maintaining rigorous standards for regulatory decision making.

A Bayesian-NLME approach identifies patients at risk of delayed MTX

data is provided

Wednesday 15:30-17:00

Thinking beyond the norm: how to (fairly) evaluate Bayesian Dynamic Borrowing Designs - ***Gaëlle Saint-Hilary***

III-029 Francois Mercier

{jmpost}: An R package for Bayesian joint TGI-OS models

Thursday 9:50-11:45

III-033 Anna Mikhailova

A Bayesian Workflow for Minimal PBPK Modeling: A Cas

Thursday 9:50-11:45

Bayesian Dynamic Borrowing
Chair: ***Julia Saperia***

Unexpected results and challenges when using mixture priors for Bayesian borrowing (O031) - ***Darren Scott***

Non-monotonic power in Bayesian dynamic borrowing: insights and practical remedies (O035) - ***Gianmarco Caruso***

Biased borrowing or borrowing bias? Leveraging Bayesian borrowing and quantitative bias analysis for robust comparative effectiveness insights (O049) - ***Grace Hsu***

Frequentists United: A Safe Space for Embracing Bayes (T003) - ***Patrik Atkinson***

methodology to regulators and stakeholders. The acceptance of Bayesian approaches by regulators is a big step forward, widely acknowledged within the industry and beyond.

The award presentation took place at the PSI annual conference in Amsterdam, where Nicky Best and Andrea Callegaro collected the award on behalf of the Biostatistics

Regulatory Hot topics session (PL3)

The Regulatory Hot topic session will focus on tw
adaptive design. ***Peter van de Ven*** (Dutch Medici
decision making. ***Armin Koch*** (Hannover Medica
implications and key considerations within the ne
Q&A segments allowing for direct engagement wi
(Novartis).

atistics in regulatory decision making; 2) ICH E20 draft g
y Best (GSK) will present on Bayesian statistics in regu
Khadija Rantell (MHRA) will provide their perspectives
attendees will have the chance to actively participate, w

III-030 Assil Merlaud

Characterizing the organ-specific association between tumor dynamics and overall survival across cancer types and studies: a Bayesian meta-regression

Thursday 9:50-11:45

III-099 Anne Ravix

Exploring Bayesian TDM: a simulation-based proof of concept

neonates

Thursday 9:50-11:45

Need for guidance on Bayesian methods

- Application of Bayesian methods mentioned in several guidance documents, e.g. ICH E9, ICH E11A, ACT EU Q&A on complex trials
- Support innovative methodology
- Understand strengths and pitfalls
- Metrics for how to assess uncertainty
- Regulatory aspects of interpretation of Bayesian statistics

⇨Concept Paper on the use of Bayesian statistics in clinical development

Concluding remark

The aim of today's workshop is to have a dialog on the potential use of Bayesian statistic in regulatory-decision making