| *Final 1.2 260609* | **Charter: Priority Topic Bayes –** *"Same evidentiary standards. Enhanced decision framework."* |
|---|---|

**Objective**:
- Advance the credible and regulatory-relevant use of Bayesian methods in clinical development, e.g. Hybrid Designs, Design priors / Analysis priors, focusing on decision-making under uncertainty and integration into existing frameworks (estimands, trial design, MIDD)
- Enable a balanced application of Bayesian methods as one of the possible approaches available to clinical developers and reviewers, as appropriate.
- Support EMA's change management efforts to embrace Bayesian statistics - closing comments, Amsterdam Workshop June 17, 2025: *Peter Arlett: recommending **a change management approach to help elevate the implementation of Bayesian** approaches so they are embraced and not discarded leading to missed opportunities*).
- Address the gap between conceptual understanding and regulatory acceptance.

**Expected Impact:** • Alignment between industry and regulators, credible use in submissions, acceleration of fit-for-purpose designs.

***This workstream is not focused on methodological advocacy, but on enabling applications of Bayesian approaches with confidence within existing evidentiary standards.***

## Key stakeholders and resources

| | |
|---|---|
| **Priority topic leads** | Mike Krams and Nicky Best |
| **Team** Bayesian & Regulatory Expertise | • Marc Vandemeulebroecke<br>• Jürgen Hummel,<br>• Christian Stock<br>• David Wright<br>• Kert Viele |
| **Target Audience** | • Clinical and statistical reviewers at EMA<br>• Senior scientific leadership and decision-makers within EMA (e.g., MWP, Heads of Office) and other regulatory agencies<br>• Pharmaceutical industry statisticians and clinical development leaders<br>• External academic experts in Bayesian clinical trials<br>• EFPIA / industry regulatory policy groups |

## Scope, Workplan, and Deliverables

**Strategic framing:** Build progressive regulatory engagement
- Move from awareness to confidence to institutional acceptance
- Reduce perceived risk while demonstrating decision-relevant value

**Phase 1 (Q2 2026): De-risking the Concept**
- Conduct Executive Webinar: Regulatory audience. No equations. Type I error reassurance. Real use cases.
- Prepare Two-page regulatory primer (what Bayes can/cannot do; when/how to use Bayes)

**Phase 2 (Q3/4 2026): Build Competence and Trust**
- Full-day workshop (case studies, EMA scenarios, calibration). Examples will demonstrate alignment with existing regulatory standards through side-by-side comparison of Bayesian/Frequentist operating characteristics.
- Monthly case clinics (priors, hierarchical models, platform trials)
- Office hours (low-stakes regulator engagement). *Key principle:* Same bar for Bayes. Just a different framework.

**Decision point (Q4 2026):** Assess regulatory readiness for transition to Phase 3 based on engagement, feedback, and demonstrated understanding.

**Phase 3 (Q1-4/2027): Institutional Embedding**
- Mock Bayesian confirmatory submission (SAP, simulations, sensitivity). This serves as a concrete test of governance readiness and addresses concerns around transparency, reproducibility, and reviewability.
- Joint FDA–EMA workshop
- Draft EMA concept note

***Key Questions:***

1. What is regulatory-ready Bayes? 2. How to meet evidentiary standards? 3. Where is added value vs complexity?

## Key Linkages

- PSI Bayesian SIG
- EFPSI Regulatory Stats WG
- ASA RISW – Sept 2026
- Interactions on FDA Draft Guidance on Bayesian Statistics and ICH E20
- Regulatory initiatives (e.g. Concept paper on Bayes, workshops/webinars)

## High-level timelines

### Key Milestones and Target dates

1. Alignment on charter within EFPSI SML – April 17, 2026 - done
2. Webinar – Sept 30, 2026
3. Two page regulatory primer – June 30, 2026
4. Interaction between EFPSI SML and EMA on phase 2&3 – June 30, 2026
5. Workshop announced – Sept 30, 2026

***Quarterly progress reports at EFPSI SML meetings***