

BAYESIAN METHODS
FOR BIOMEDICAL RESEARCH
—
**Part III: Bayesian applications in medical
research**

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ISPED summer school
at the University of Bordeaux

June 10th, 2026

Introduction

Examples of Bayesian applications

3 different real-world use cases in biomedical sciences:
illustrations where the Bayesian approach can be particularly useful

⚠ **Disclaimer:** this course is NOT

- ~~a meta-analysis short course~~
- ~~an adaptive design in clinical trials short course~~

Post-mortem re-analysis
of an under-powered randomized trial

Original analysis of EOLIA

EOLIA (Combes *et al.*, NEJM, 2018):

- randomized clinical trial
- evaluation of a new treatment for severe acute respiratory distress syndrome
- outcome: mortality rate after 60 days
- 249 patients:
 - 125 controls
 - ⇒ mechanical ventilation (conventional treatment)
 - 124 treated
 - ⇒ ECMO (extracorporeal membrane oxygenation – new(er) treatment)

Frequentist analysis:

⇒ Relative Risk of death at 60 days for ECMO compared to control: 0.76

$CI_{95\%} = [0.55, 1.04]$

p-value = 0.09

Bayesian re-analysis of EOLIA data

Goligher *et al.* (*JAMA*, 2018)

	Group	
	ECMO	Control
group size n	124	125
number of deaths at 60 days	44	57

Observed data in the EOLIA trial

Your turn !



Read EC Goligher *et al.*
Extracorporeal Membrane
Oxygenation for Severe Acute
Respiratory Distress Syndrome and
Posterior Probability of Mortality
Benefit in a Post Hoc Bayesian
Analysis of a Randomized Clinical
Trial, *JAMA* 320(21): 2251, 2018.
[DOI:10.1001/jama.2018.14276]

Practical: exercise 6

Bayesian meta-analysis

What is a meta-analysis

“An analysis of analyses”

⇒ a single quantitative summary of studies answering the *same research question*

Ex: medical therapies effects are often evaluated in multiple different studies.

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Ex: medical therapies effects are often evaluated in multiple different studies.

⇒ pool individual observations from multiple studies ?

- ⚠ potential differences in the pooled experiments
- ⚠ only aggregated summary statistics estimates (“effect sizes”) available – alongside uncertainty (e.g. standard errors)

Study Heterogeneity

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Often, different studies used different populations

⇒ potential extra-variability

+ different sample sizes ⇒ also impact the estimate and its variability

Meta-analysis random effects model

Common approach for meta-analysis:

$$y_i \sim \mathcal{N}(\theta_i, \sigma_i^2)$$

$$\theta_i \sim \mathcal{N}(\mu, \tau^2)$$

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Hierarchical generalization of the fixed effect model:

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⇒ between study variability: $y_i \sim \mathcal{N}(\mu, \sigma_i^2 + \tau^2)$

Hierarchical generalization of the fixed effect model:

$$y_i \sim \mathcal{N}(\mu, \sigma_i^2)$$

⇒ assume same average effect for each study

Bayesian meta-analysis in practice

Meta-analysis: a perfect usecase for Bayesian analysis ?

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Meta-analysis: a perfect usecase for Bayesian analysis ?

- few observations
- informative *prior*
- sequential

Going further

Scientific literature search

⚠ FIRST (!) exhaustive search of the scientific literature

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Evidence synthesis

Meta-analysis \in evidence synthesis

e.g. meta-regression, mechanistic modeling, ...

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Still active research domains:

- random effects model will down-weight studies with larger sample sizes
 - Serghiou & Goodman, *JAMA*, 2018

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- random effects model will down-weight studies with larger sample sizes
 - Serghiou & Goodman, *JAMA*, 2018
 - a bug or a feature ?*

Your turn !



Read ND Crins *et al.* Interleukin-2 Receptor Antagonists for Pediatric Liver Transplant Recipients: A Systematic Review and Meta-Analysis of Controlled Studies, *Pediatric Transplantation* 18(8):839, 2014. [DOI:10.1111/petr.12362]

Practical: exercise 7

**BONUS content:
CRM dose-escalation**

Continuous Reassessment method

CRM [O'Quigley *at al.*, 1990]

Objective: identify the optimal dose

(i.e. Minimum Efficient Dose or Maximum Tolerated Dose)

⇒ select iteratively the dose for the next (batch of) recruited patient(s)
based accumulating observations from previously included patients

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based accumulating observations from previously included patients

- 😊 treat each patient ethically (dose best supported by the current evidence)
- 😊 *prior* knowledge
- 😊 sequential Bayesian: online update of the posterior

Your turn !



Read F Kaguelidou *et al.*
Dose-Finding Study of Omeprazole
on Gastric pH in Neonates with
Gastro-Esophageal Acid Reflux Using
a Bayesian Sequential Approach,
PLOS ONE 11(12):e0166207, 2016.
[DOI:10.1371/journal.pone.0166207]

Practical: BONUS exercise 9