

# Study of Operating characteristic of Bayesian methods that borrow treatment effects

**First published:** 07/06/2023

**Last updated:** 24/06/2025

Study

Finalised

## Administrative details

### EU PAS number

EUPAS105257

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### Study ID

106787

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### DARWIN EU® study

No

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### Study countries

 France

 United States

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### Study description

While the appropriateness of Bayesian methods in the paediatric context is largely recognised, most existing methods refer to borrowing control arm data only. From a regulatory perspective, an important issue with Bayesian methods is that they potentially do not control the Type I Error. Many simulation studies have been performed within the last decade in order to evaluate the operating characteristics, e.g. regarding Type I Error inflation and possible gains with respect to power, associated with Bayesian methods. Moreover, these methods are known to be potentially sensitive to small changes in underlying parameters, such as a weight parameter which determines how much information is borrowed from the historical data, and could potentially leads to different conclusions. Considering the limitations associated with these methodologies, an extension towards borrowing treatment effect, instead of historical control data only, is considered. The primary aim of this Tender is to thoroughly evaluate the available Bayesian methods for borrowing information on treatment effects in the context of paediatric evaluations. More specifically, specific objectives of this project include: - Review and evaluate available and published methods for dynamic borrowing via a structured and critical review (not limited to paediatric extrapolation) - Provide a clear mathematical specification of each model identified, articulating the interpretation of underlying parameters, and define the relevant operating characteristics (OCs) and performance metrics to evaluate for a given context - Evaluate and compare (via simulations) performance of such methods along the pre-defined OCs in various common scenarios - Synthesize and disseminate overall conclusions and recommendations

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### **Study status**

Finalised

## **Research institutions and networks**

## Institutions

### Quinten Health

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

Daniel Lee New York, USA

## Contact details

### Study institution contact

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Study contact

[b.amzal@quinten-health.com](mailto:b.amzal@quinten-health.com)

### Primary lead investigator

Billy Amzal

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 25/01/2023

Actual: 25/01/2023

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### **Study start date**

Planned: 25/01/2023

Actual: 25/01/2023

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### **Date of final study report**

Planned: 13/02/2025

Actual: 27/02/2025

## Sources of funding

- Other

## More details on funding

European Medicines Agency

## Study protocol

[EMA - ROC02 - D3 - Study Protocol.pdf](#) (352.28 KB)

## Regulatory

### **Was the study required by a regulatory body?**

Yes

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### **Is the study required by a Risk Management Plan (RMP)?**

Not applicable

## Methodological aspects

### Study type

**Study type:**

Not applicable

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**Scope of the study:**

Other

**If 'other', further details on the scope of the study**

Assessment of Bayesian statistical methods

**Main study objective:**

(1) Review and evaluate methods for dynamic borrowing (2) Provide a clear mathematical specification of each model identified (3) Evaluate and compare (via simulations) performance of such methods along the pre-defined OCs in various common scenarios (4) Synthesize and disseminate overall conclusions and recommendations

## Population studied

**Age groups**

- Preterm newborn infants (0 - 27 days)
- Term newborn infants (0 - 27 days)
- Infants and toddlers (28 days - 23 months)
- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)

- Adults (85 years and over)
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### **Estimated number of subjects**

0

## Study design details

### **Data analysis plan**

Not applicable

## Documents

### **Study report**

[EMA\\_Study\\_Report\\_D4\\_final.pdf](#) (2.12 MB)

### **Study, other information**

[EMA\\_preliminary\\_study\\_plan\\_D2\\_final.pdf](#) (226.57 KB)

## Data management

### ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

## **Data sources (types)**

Other

Published literature

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## **Data sources (types), other**

Literature Review: Pubmed. Simulation Study

# Use of a Common Data Model (CDM)

## **CDM mapping**

No

# Data quality specifications

## **Check conformance**

Unknown

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## **Check completeness**

Unknown

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## **Check stability**

Unknown

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## **Check logical consistency**

Unknown

# Data characterisation

## **Data characterisation conducted**

No