# Novel statistics to handle rare diseases and small sample sizes using Bayesian techniques: Application to Multiple Sclerosis (MS) and Systemic Lupus Erythematosus (SLE) in pregnancy

**First published:** 04/10/2021

**Last updated:** 27/03/2024

Study Ongoing

## Administrative details

**EU PAS number**

EUPAS43420

**Study ID**

43421

**DARWIN EU® study**

No

**Study countries**

Finland

France

Germany

Italy

Norway

Spain

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**Study description**

The aim of the study is to evaluate the safety to the mother and child of medications that are used by women suffering from SLE or MS during pregnancy using novel statistical methods. The ConcePTION platform will be used to analyse observational data from multinational cohort studies based on population based secondary data sources in Europe. The following studies will be undertaken: a. Identification of the most appropriate algorithm to identify MS and SLE in women of child bearing age and childhood infections in children in selected data sources on the ConcePTION platform. b. Evaluation of the utilisation trends and patterns of medications in women with SLE and MS (a) of childbearing age and (b) in pregnant women before, during and after pregnancy c. Evaluation of the safety to the mother and child of medications that are used by women suffering from SLE or MS during pregnancy. Analytic results from each data sources will be combined using novel meta-analytic techniques to overcome the issue of small numbers that will occur due to rare exposures and outcomes being studied. d. Evaluation of the increased risk of the occurrence of a major congenital malformation associated with first trimester exposure to medications that are used by women suffering from SLE or MS during pregnancy. EUROmediCAT data will be used to perform case-malformed analyses and to compile case series of congenital anomaly cases to identify possible safety indications.

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**Study status**

Ongoing

## Research institutions and networks

# Institutions

## CHU de Toulouse - Hôpital des Enfants

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

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

Institution

## Pharmacoepidemiology and Drug Safety Research Group (PharmaSafe), University of Oslo

Norway

**First published:** 19/10/2016

**Last updated:** 06/11/2025

Institution | Educational Institution | ENCePP partner

## Drugs and Pregnancy, Finnish Institute for Health and Welfare (THL)

Finland

**First published:** 17/03/2010

**Last updated:** 20/03/2024

Institution | Educational Institution | Laboratory/Research/Testing facility | ENCePP partner

## Ulster University

United Kingdom (Northern Ireland)

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

**Last updated:** 20/03/2024

Institution | Educational Institution

## University of Bordeaux

France

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

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

Institution | Educational Institution

## The Foundation for the Promotion of Health and Biomedical Research of Valencia Region (FISABIO)

Spain

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

**Last updated:** 31/10/2025

Institution

University of Swansea Wales, UK, University of Ferrara Emilia Romagna, Italy, Ulster University North Ireland, UK, Institute for prevention Research and epidemiology Leipniz, Germany, University of Bordeaux France, The Foundation for the Promotion of Health and Biomedical Research of Valencian Region Spain

## Networks

### ConcepTION

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

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Network

## Contact details

**Study institution contact**

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**Primary lead investigator**

Christine Damase-Michel

**Primary lead investigator**

## Study timelines

**Date when funding contract was signed**

Planned: 01/04/2021

Actual: 01/04/2021

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**Study start date**

Actual: 08/05/2020

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**Date of final study report**

Planned: 31/03/2024

## Sources of funding

- EU institutional research programme

## More details on funding

IMI ConcePTION

## Regulatory

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

No

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

Not applicable

# Methodological aspects

## Study type

## Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Drug utilisation

**Main study objective:**

The ultimate aim of the study is to use novel methods of analysis concerning the synthesis of information from disparate data sources to provide data about medications that are used by women suffering from rare diseases such as SLE or MS, on their safety in pregnancy to the mother and child.

## Study Design

**Non-interventional study design**

Case-control

Cohort

## Study drug and medical condition

**Medical condition to be studied**

Multiple sclerosis

## Population studied

**Age groups**

- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)

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**Special population of interest**

Pregnant women

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**Estimated number of subjects**

5.989.000

## Study design details

**Outcomes**

• Major congenital malformations -, Maternal : - Preeclampsia -Gestational diabetes Pregnancy : • Still birth • Spontaneous abortions • Live births •

Elective terminations • Ectopic pregnancy Birth/Neonatal : • SGA • LGA • Malformations split by organ system • Pre-term birth • Neonatal stroke • Neonatal death • Neonatal infections Children : - Death - Infections

**Data analysis plan**

-Algorithm Identification: Validity judged by consistency of estimated prevalence rates within registries across time and by comparison with published prevalence rates. -Utilisation study: Prevalence of MS and SLE drug utilisation compared between pregnant women and matched non-pregnant women using conditional logistic regression. -Safety study : Medication safety analysed by comparing women with SLE or MS who are exposed to a specific medication with women with SLE or MS who are unexposed . In addition, comparaison to women without either MS or SLE to estimate the association of each disease with adverse pregnancy outcomes. Unadjusted logistic regression models, then a range of adjusted models will be run to account for confounders and effect modifiers. Analytic results from each data sources will be combined using novel meta-analytic techniques to overcome any issues of small numbers occurring. -EUROmediCAT study : Case-malformed analyses(logistic regression)

# Data management

## Data sources

**Data source(s)**

EFEMERIS

German Pharmacoepidemiological Research Database

ARS Toscana

EUROmediCAT central database

**Data source(s), other**

Emilia Romagna GPs drug prescription, EFEMERIS, Drugs and Pregnancy Finland, GePaRD, ARS, EUROmediCAT

**Data sources (types)**

Administrative healthcare records (e.g., claims)

Drug dispensing/prescription data

Other

**Data sources (types), other**

Exposure registry

## Use of a Common Data Model (CDM)

**CDM mapping**

No

## Data quality specifications

**Check conformance**

Unknown

**Check completeness**

Unknown

**Check stability**

Unknown

**Check logical consistency**

Unknown

# Data characterisation

**Data characterisation conducted**

No