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

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.

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

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Network

Contact details

Study institution contact

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Study 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

Study start date

Actual: 08/05/2020

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

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

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

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)

Special population of interest

Pregnant women

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

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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

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 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