Prospective, Registry-Based Observational Cohort Study of Ritlecitinib Safety in Pregnancy

First published: 10/09/2024

Last updated: 19/06/2025

Study | Planned

Administrative details

EU PAS number

EUPAS1000000296

Study ID

1000000296

DARWIN EU® study

No

Study countries

United States

Study description

Research question: Is there an increased risk of adverse maternal and/or infant outcomes in individuals with AA exposed to ritlecitinib during pregnancy?
Primary objective: To estimate the prevalence of major congenital malformation [MCM] births (primary outcome) among pregnant individuals with alopecia areata [AA] who are (1) exposed to ritlecitinib (exposed cohort) and (2) unexposed to ritlecitinib (comparator cohort).
Secondary objectives: 1. To estimate the prevalence of the following secondary outcomes in the 2 cohorts: SAB, elective termination, pregnancy complications (pre-eclampsia, eclampsia), stillbirth, preterm birth, SGA, minor congenital malformation, infant postnatal growth deficiency, and infant developmental delay. 2. To estimate the RR of each of the study outcomes in the exposed versus unexposed cohorts, if sample size permits.
Study design: This registry-based, prospective observational cohort study will enroll and follow pregnant individuals in the US, including individuals with AA exposed to ritlecitinib during pregnancy and individuals with AA unexposed to ritlecitinib during pregnancy. This study will be a new, product-based pregnancy registry. Participation in the registry is voluntary and participants can withdraw their consent to participate at any time. Data will be collected from enrolled pregnant individuals and the HCPs involved in their care or the care of their infants.
The primary study outcome is MCM and the secondary outcomes are SAB, elective termination, pregnancy complications (pre-eclampsia, eclampsia), stillbirth, preterm birth, SGA, minor congenital malformation, postnatal growth deficiency, and infant developmental delay. The main measures of effect are the prevalence of each outcome in the 2 study cohorts and, if sample size permits, the RR of each outcome, comparing the cohorts.

Study status

Planned

Research institutions and networks

Institutions

Pfizer

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Pharmaceutical Product Development (PPD)

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Ian Stryker ian.stryker@pfizer.com

Study contact

ian.stryker@pfizer.com

Primary lead investigator

Monica Bertoia

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 10/06/2024

Actual: 10/06/2024

Study start date
Planned: 31/10/2024

Data analysis start date
Planned: 28/02/2029

Date of interim report, if expected
Planned: 28/02/2030

Date of final study report
Planned: 30/06/2035

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer 100%

Study protocol

B7981095_RITLECITINIB PREGNANCY REGISTRY PROTOCOL_V2.0_15JUN2024 clean.pdf (813.28 KB)

B7981095_RITLECITINIB PREGNANCY REGISTRY PROTOCOL_V3.0_12FEB2025.pdf (867.21 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Other study registration identification numbers and links

B7981095

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Primary data collection

Study design:

This study is a prospective, registry-based observational cohort study. This study will be a new, product-based pregnancy registry. Data will be collected from eligible participants in the US.

Main study objective:

Primary objective: To estimate the prevalence of MCM births among pregnant individuals with AA who are (1) exposed to ritlecitnib and (2) unexposed to ritlecitnib.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

LITFULO

Study drug International non-proprietary name (INN) or common name

RITLECITINIB

Anatomical Therapeutic Chemical (ATC) code

(L04AF08) ritlecitinib

ritlecitinib

Medical condition to be studied

Pregnancy

Alopecia areata

Population studied

Short description of the study population

Two cohorts of pregnant individuals with AA in the US. The study cohorts will include individuals exposed to ritlecitinib during pregnancy and individuals unexposed to ritlecitinib during pregnancy who are enrolled in the registry.

Age groups

- **Paediatric Population (< 18 years)**
  - Preterm newborn infants (0 – 27 days)
  - Term newborn infants (0 – 27 days)
  - Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Special population of interest

Pregnant women

Estimated number of subjects

400

Study design details

Setting

The study population will be derived from eligible individuals in the US enrolled in the pregnancy registry.

Outcomes

Outcomes of interest

Primary: Major congenital malformation (MCM)

Secondary: Spontaneous abortion (SAB), elective termination, pregnancy complications (pre-eclampsia, eclampsia), stillbirth, preterm birth, SGA, minor congenital malformation, infant postnatal growth deficiency, and infant developmental delay.

Data analysis plan

The analysis population will include valid participants who were prospectively enrolled in the registry, are not exposed to teratogens or investigational medications during pregnancy, and who are not considered lost to follow up. Patient characteristics will be summarized with descriptive statistics for each cohort. The number of observations, median, mean, standard deviation, minimum, and maximum will be reported for each continuous variable. The frequency and percentage per category will be reported for each categorical variable. Prevalence of the outcomes of interest will be calculated according to the conventions described in the protocol. In general, the prevalence of each

outcome will be calculated by dividing the number of cases of the outcome by the appropriate denominator for that particular outcome, based on clinical knowledge. For most outcomes, the analysis population (denominator) will be the number of pregnant individuals with pregnancy outcome data, the number of live births, or the number of infants with follow-up data at the timepoint of interest, as appropriate; however, for some outcomes, the analysis population (denominator) will be restricted based on certain relevant factors. Comparative analyses will be conducted for each outcome if sample size permits. Crude (unadjusted) RRs (and corresponding 95% CIs) will be calculated using Exact methods. Adjusted RRs will be calculated using generalized linear models (binomial family) with a log (RR) link and weighted by IPTW. The Clopper-Pearson method will be used to derive 95% CIs. IPTW will be calculated using propensity scores estimated from propensity score models. Each individual's propensity score will be estimated using a logistic regression model with exposure status as the outcome (dependent variable). Detailed methodology for summary and statistical analysis of the data collected in this study will be documented in a statistical analysis plan

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), other

This will be a new, product-based pregnancy registry conducted by PPD (part of Thermo Fisher Scientific).

Data sources (types)

Other

Data sources (types), other

Primary data collection

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

Unknown