Observational Cohort Study of Ritlecitinib Safety in Pregnancy within a US Claims Database

First published: 21/05/2025

Last updated: 21/05/2025

Study Planned

Administrative details

EU PAS number

EUPAS1000000553

Study ID

1000000553

DARWIN EU® study

No

Study countries

United States

Study description

The purpose of this study is to assess the safety of ritlecitinib when used in pregnancy in terms of risk of major congenital malformations (MCMs), spontaneous abortion, pregnancy termination, stillbirth, pregnancy-related hypertension, gestational diabetes, pre-eclampsia, eclampsia, small for gestational age (SGA) births, preterm birth, and serious infection in the first year of life.
This non-interventional study (NIS) is designated as a PASS and is a postmarketing requirement for the FDA.

Study status
Planned

Research institutions and networks

Institutions

Pfizer

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Carelon Research

Optum USA

Contact details

Study institution contact

Mwedusasa Mtenga Mwedusasa-bety.Mtenga@pfizer.com

Study contact

Mwedusasa-bety.Mtenga@pfizer.com

Primary lead investigator

Monica Bertoia

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 18/08/2023

Actual: 18/08/2023

Study start date

Planned: 01/03/2029

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

Study protocol

B7981096_PROTOCOL_V3.0_12FEB2025.pdf (736.51 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

B7981096

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Study design:

Cohort study within two US-based health insurance claims databases. Three cohorts will be identified: pregnancies exposed to ritlecitinib, a treated comparator cohort exposed to other approved alopecia areata (AA) therapies, and a second treated comparator cohort exposed to other AA therapies.

Main study objective:

Primary objectives:

1. To estimate the prevalence of major congenital malformation (MCM) livebirths among pregnant individuals with AA who are (1) exposed to ritlecitinib; (2) unexposed to ritlecitinib but exposed to other approved treatments for AA; and (3) unexposed to ritlecitinib but exposed to other treatments for AA.
2. To estimate the relative prevalence of MCM livebirths in the ritlecitinib-exposed cohort versus the two comparator cohorts.

Secondary objectives:

1. To estimate the prevalence of the following secondary outcomes in the study cohorts: spontaneous abortion, pregnancy termination, stillbirth, pregnancy-related hypertension, gestational diabetes, pre-eclampsia, eclampsia, small for gestational age (SGA) birth, preterm birth, and serious infection in the first year of life.
2. To estimate the relative prevalence of each of the secondary outcomes in the ritlecitinib-exposed cohort versus the two comparator cohorts, if sample size permits.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name, other

Ritlecitinib

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

RITLECITINIB

Anatomical Therapeutic Chemical (ATC) code

(L04AF08) ritlecitinib

ritlecitinib

Population studied

Short description of the study population

Pregnancies among individuals with alopecia areata (AA) with an estimated conception date (ECD) between 23 June 2023 and 03 August 2033 with follow-up for outcomes through 23 June 2034.

Special population of interest

Pregnant women

Study design details

Setting

The base population will include all pregnancies among individuals with AA identified during the study period within the US-based health insurance claims databases.

Comparators

The approved treated comparator cohort (Cohort 2) will include eligible pregnancies exposed to a medication indicated for the treatment of severe AA other than ritlecitinib, and not exposed to ritlecitinib or other JAK inhibitors not approved for the indication of severe AA. Pregnancies may be eligible for this cohort if they are also exposed to other, non-approved, treatments for AA (non-JAK inhibitors), including those used to define Cohort 3.

A second treated comparator cohort (Cohort 3) will include pregnancies exposed to medications or procedures reported in the literature as being used for treatment of extensive or severe AA, but which have not received FDA approval for that indication. Treatments included in this cohort need not have demonstrated effectiveness for the treatment of AA; rather, they are often used in real world practice.

Outcomes

The primary outcome is validated major congenital malformation. The secondary outcomes are spontaneous abortion, pregnancy termination, stillbirth, pregnancy-related hypertension, gestational diabetes, pre-eclampsia, eclampsia, small for gestational age birth, preterm birth, and serious infection in the first year of life.

Data analysis plan

For interim reports all analyses will be descriptive, including the number of observations, mean, standard deviation, median, interquartile range, and range for all continuous variables and counts and percentages for each binary or

categorical variable.
The interim report will additionally include claims-identified outcome counts aggregated across cohorts (in order to blind cohort membership).
Validation of MCMs via medical record review will not be performed until the final report analysis.

For the final report, prevalence and 95% confidence intervals (CIs) for each of the study outcomes will be estimated separately for each study cohort and each database.
For MCM, the final analyses will be restricted to confirmed cases. Prevalence estimates and comparative analyses will be restricted to pregnancies with an observed pregnancy outcome in the claims data.

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
Optum Research Database (ORD); The Healthcare Integrated Research Database

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