A Prospective Active Surveillance Study to Monitor the Real-World Safety of Ritlecitinib Among Adolescents with Alopecia Areata

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Study Planned

Administrative details

EU PAS number

EUPAS1000000421

Study ID

1000000421

DARWIN EU® study

No

Study countries

- ☐ Canada
- ☐ European Union
- ☐ United Kingdom
- ☐ United States

Study description

This study is designed to actively monitor growth and bone development, including bone fractures, and maturation and pubertal development associated with exposure to ritlecitinib in adolescents 12-17 years old in the post-approval setting. Neurotoxicity, an important potential risk, will also be evaluated.

Study status

Planned

Research institutions and networks

Institutions

Pfizer

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Institution

CorEvitas

Contact details

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Study contact

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Primary lead investigator

Sarah MacDonald

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 11/06/2024

Actual: 11/06/2024

Study start date

Planned: 31/03/2026

Data analysis start date

Planned: 31/03/2026

Date of interim report, if expected

Planned: 30/09/2026

Date of final study report

Planned: 31/03/2037

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer 100%

Study protocol

B7981092_PROTOCOL_V2.0_12JUL2024.pdf(725.01 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Other study registration identification numbers and links

B7981092

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:

Secondary use of data

Study design:

This will be a prospective observational cohort study of adolescents with severe AA who receive ritlecitinib and those in the comparator cohort, including those exposed to other approved systemic medications for the treatment of AA in adolescents.

Main study objective:

Among adolescent participants with AA who are treated with ritlecitinib and, separately, among adolescent participants in the comparator cohort, including those exposed to other approved systemic medications for the treatment of AA in adolescents, to:

• Estimate growth and bone development metrics;
• Estimate maturation and pubertal development metrics;
• Estimate the incidence of bone fractures; and
• Estimate the incidence of neurotoxicity events

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

LITFULO

Name of medicine, other

ritlecitinib tosylate

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

RITLECITINIB

Anatomical Therapeutic Chemical (ATC) code

(L04AF08) ritlecitinib

ritlecitinib

Medical condition to be studied

Alopecia areata

Population studied

Short description of the study population

Adolescents 12-17 years old receiving ritlecitinib for Alopecia areata (AA) and adolescents in the comparator cohort, including those exposed to other approved systemic medications for the treatment of AA in adolescents.

Age groups

Adolescents (12 to < 18 years)

Special population of interest

Other

Special population of interest, other

Adolescents

Estimated number of subjects

1000

Study design details

Setting

CorEvitas International Adolescent AA Registry is a prospective, multicentre, observational registry initiated to evaluate treatment outcomes in adolescents with severe AA. Sites include dermatological and hair-loss clinics throughout the EU, as well as the UK, the US, and Canada that have a high volume of treated adolescent patients with AA are identified and asked to participate, targeting up to 65 study sites.

Comparators

Participants without previous exposure to ritlecitinib who initiate another systemic medication for the treatment of AA, including non-ritlecitinib JAK inhibitors, at the time of registry enrolment or within 6 months prior to enrolment, will be assigned to the comparator cohort.

Participants with severe AA in the opinion of the provider at the time of registry enrolment who are unexposed to systemic medications for AA within the 6 months prior to, or at the time of, registry enrolment and who are not prescribed a systemic treatment for AA at registry enrolment, will also be included in the comparator cohort.

Outcomes

Growth outcomes will include the change from baseline to end of follow-up in participant's height standard deviation score (SDS) and weight SDS measures. Other descriptive measurements of growth will also be reported including: standing height, height percentiles, height velocity, height velocity SDS, weight, weight percentiles, body mass index (BMI), BMI percentiles, and BMI SDS. To evaluate bone development, the occurrence of bone fractures will be assessed. Maturation and pubertal development will be measured. Neurotoxicity events will be reported.

Data analysis plan

For the primary analyses, descriptive statistics for each outcome of interest will be computed separately by exposure cohort. For growth and bone development metrics, mean and SD will be presented for continuous metrics related to height, weight, and BMI, including change from baseline to end of follow-up in standing height, height percentile, height SDS, height velocity SDS, weight, weight percentile, weight SDS, BMI, BMI percentile, and BMI SDS. The proportion of participants whose height SDS at the end of follow-up is more than 1 or 2 standard deviations lower than their baseline height SDS will also be reported.

Tanner staging, a categorical maturation and pubertal development metric, will be described using counts and percentages by exposure cohort, and by sex within each exposure cohort. The cumulative incidence and IR of bone fractures and neurological adverse events, overall and by type, will be estimated within each exposure cohort.

Outcomes of interest will be compared between exposure cohorts as exploratory analyses. Conventional linear regression models will be fit on the PS-matched sample for continuous outcomes, including change in height SDS,

change in weight SDS, age at PHV, and age at Tanner stage progression. To compare bone fractures and neurological adverse events, generalised linear regression models will be fit on the PS-matched sample.

In order to increase sample size and statistical power, the primary and exploratory analyses are proposed to be performed on a pooled sample of participants from Europe (UK and EU countries) and North America (US and Canada).

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

CorEvitas International Adolescent AA Registry

Data sources (types)

Disease 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

Unknown