A Longitudinal, Retrospective, Multi-centre Observational Study to Evaluate Effectiveness, Persistence, Treatment Patterns and Safety of Australian Patients Receiving Early Access to Tofacitinib

First published: 29/11/2021

Last updated: 17/09/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS41439

Study ID

47773

DARWIN EU® study

No

Study countries

☐ Australia

Study description

This study will examine the disease characteristics and outcomes in ulcerative colitis patients granted access to tofacitinib in Australia using deidentified patient information.

There are over 300 ulcerative colitis patients who have been treated with tofacitinib since its TGA approval. The vast majority of patients who have been granted early access have failed previous biologic therapy.

There has been limited data presented on the response of patients to tofacitinib after previously failing anti-TNF and anti-integrin therapy.

Study status

Ongoing

Research institutions and networks

Institutions

Pfizer

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Institution

Mater Hospital Brisbane, St Vincent's Hospital Victoria, Austin Health Victoria, St John of God Western Australia, St Vincent's Hospital Victoria,

Concord Hospital Sydney, Eastern Health Victoria, Gold Coast Health Queensland, Royal Melbourne Hospital Victoria

Networks

Mater research

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Elke Binder

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/08/2021

Actual: 02/09/2021

Study start date
Planned: 30/04/2022

Actual: 11/03/2022

Date of final study report
Planned: 31/10/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

A3921405 final approved protocol.pdf (2.47 MB)

A3921405_Non-Interventional Study Protocol (REDACTED)_V3.0_15AUG2024.pdf (522.34 KB)

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 topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

Examine the disease characteristics and outcomes in ulcerative colitis patients granted access to tofacitinib in Australia

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

A longitudinal, retrospective, non-interventional. multi-center observational study

Study drug and medical condition

Name of medicine

XELJANZ

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

TOFACITINIB

Anatomical Therapeutic Chemical (ATC) code

(L04AF01) tofacitinib

tofacitinib

Medical condition to be studied

Colitis ulcerative

Population studied

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

160

Study design details

Outcomes

- Clinical response rate (decrease in the partial Mayo score by 2 or partial Mayo score <2) at end of induction (week 8-12) and week 16,
- Remission rate at end of induction (week 8-12) / week 16
- Proportion of patients on tofacitinib completing induction therapy (8 or 16 weeks)
- Persistence of response up to week 52 Resolution of rectal bleeding at end of induction
- Endoscopic healing Cessation of steroids
- Steroid free remission at end of induction and at week 48
- Change in CRP & cholesterol (LDL/HDL) levels
- Colectomy rate

Data analysis plan

Descriptive summary statistics for continuous variables will be used to describe the study population including number of participants, mean, standard deviation, median and range.
Categorical variables will be described with frequency counts and percentages.
Pre-specified groups for statistical analysis include remitters, responders, and non-responders at the specified time points.
P values will be calculated from chi-square for categorical variables and t-tests for continuous variables with normal distribution and non-parametric testing for non-normally distributed data.
Kaplan-Meier survival analyses will be used to analyse individual predictors and display survival curves. Cox proportional hazards regression will be used to analyse multivariable survival data.

Documents

Study, other information

A3921405 Non Interventional Study Abstract V2.0 23 September 2021_Redacted.pdf (1.33 MB)

A3921405 Non-Interventional Protocol Study Abstract 26 May 2021_.pdf (79.68 KB)

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 sources (types)

Electronic healthcare records (EHR)

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