

# 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:** 15/05/2024

Study

Ongoing

## Administrative details

### PURI

<https://redirect.ema.europa.eu/resource/47773>

### 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.

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## Study status

Ongoing

## Research institution 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

Amit Vilas Thorat

Study contact

[AmitVilas.Thorat@pfizer.com](mailto:AmitVilas.Thorat@pfizer.com)

### Primary lead investigator

Elke Binder

Primary lead investigator

## Study timelines

### **Date when funding contract was signed**

Planned:

31/08/2021

Actual:

02/09/2021

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### **Study start date**

Planned:

30/04/2022

Actual:

11/03/2022

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### **Date of final study report**

Planned:

30/08/2025

## 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 Amendment 1 \(clean\) V2.0\\_ 23 September 2021\\_Redacted.pdf\(3.12 MB\)](#)

## Regulatory

**Was the study required by a regulatory body?**

No

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

Not applicable

## Methodological aspects

### Study type

### Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Disease epidemiology

Effectiveness study (incl. comparative)

**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**

Other

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**Non-interventional study design, other**

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

## Study drug and medical condition

**Name of medicine**

Xeljanz

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**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)

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**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

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## 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

### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No