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

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### 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: 18/06/2025

# 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)

### 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

### 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

### 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