# An evaluation of early use patterns to assess the effectiveness of Xeljanz® (tofacitinib citrate) in rheumatoid arthritis: A retrospective non-interventional database study of observational data embedded within Optimising Patient outcome in Australian RheumatoLogy - Quality Use of Medicines Initiative (OPAL-QUMI) (OPal study of xeljanz Effectiveness in RA - OPERA)

**First published:** 03/04/2017

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Study Finalised

## Administrative details

**PURI**

https://redirect.ema.europa.eu/resource/27619

**EU PAS number**

EUPAS18435

**Study ID**

27619

**DARWIN EU® study**

No

**Study countries**

☐ Australia

**Study description**

Xeljanz® (tofacitinib citrate) is a potent, selective inhibitor of the Janus kinase (JAK) family of kinases with a high degree of selectivity against other kinases in the human genome. It was approved for use in Australia in Feb 2015 and included in the Pharmaceutical Benefits Scheme (PBS) (reimbursement) in Oct 2015. Limited data exist to describe the characteristics and outcomes in patients who receive Xeljanz® in the real world setting. To permit assessment of general treatment patterns, clinical effectiveness, adherence and patient reported outcomes among RA patients being treated with Xeljanz in the post-approval setting, Pfizer will support a database study utilizing data collected within the Optimising Patient outcome in Australian RheumatoLogy (OPAL) network, a clinician driven point of care observational data management consortium. The OPAL network is made up of Australian private-practice rheumatologists who agree to share a clinical record system for data gathering. Several studies have already been published based on data collected from this combined cohort. This protocol outlines operational and analytical aspects of a database study within the OPAL network to describe treatment patterns and patient characteristics of Xeljanz-treated patients. It will also describe effectiveness of and adherence to Xeljanz in real-world Australian clinical practice. The study will describe baseline characteristics of patients initiating treatment, their clinical and patient-reported outcomes, and any observed

safety outcomes. The analyses will be based on enrolled incident users of Xeljanz for RA treatment. Similar data will also be collected for patients treated with biologic disease-modifying anti-rheumatic drugs (bDMARDs) to provide context about clinical management of RA in real-world Australian clinical practice. This study does not aim to perform formal comparisons between Xeljanz and bDMARDs.

**Study status**

Finalised

## Contact details

**Study institution contact**

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Study contact

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**Primary lead investigator**

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Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Planned: 20/04/2016

Actual: 20/04/2016

**Study start date**

Planned: 19/05/2017

Actual: 09/05/2017

**Data analysis start date**
Planned: 19/05/2017

Actual: 09/05/2017

**Date of interim report, if expected**
Planned: 14/07/2017

**Date of final study report**
Planned: 01/03/2019

Actual: 21/10/2021

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Pfizer

## Study protocol

A3921292 NIS Protocol.pdf(582.93 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 type list

**Study topic:**

Human medicinal product

Disease /health condition

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

**Data collection methods:**

Secondary use of data

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**Main study objective:**

To understand the patterns of treatment, clinical effectiveness, patient-reported outcomes and treatment adherence among Australian adult patients with RA treated with tofacitinib. Similar data will also be collected for patients treated with bDMARDs to provide descriptive information about clinical management of RA in real-world Australian clinical practice.

## Study Design

**Non-interventional study design**

Cohort

Other

**Non-interventional study design, other**

Retrospective study

## Study drug and medical condition

**Name of medicine**

XELJANZ

**Medical condition to be studied**

Rheumatoid arthritis

## Population studied

**Short description of the study population**

Patients aged 18 years or older diagnosed with rheumatoid arthritis (RA), received treatment with tofacitinib or a bDMARD and have at least 1 year of follow-up identified from the OPAL registry.

**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)

**Special population of interest**

Other

**Special population of interest, other**

Patients with rheumatoid arthritis

**Estimated number of subjects**

3000

## Study design details

**Outcomes**

(1) To describe tofacitinib treatment patterns among Australian adult patients with RA. (2) To assess the clinical effectiveness of tofacitinib, as defined by disease severity markers and percentage of patients reaching targeted treatment goals, in Australia. (3) To assess patient reported outcomes and treatment adherence in Australian adult patients with RA who are prescribed tofacitinib. (1) To describe bDMARD treatment patterns among Australian adult patients with RA. (2) To assess patient reported outcomes and treatment adherence in Australian adult patients with RA who are prescribed bDMARDs. (3) To describe the safety profile of Australian adult patients with RA who have been prescribed tofacitinib.

**Data analysis plan**

Patients meeting the inclusion and exclusion criteria described above will be categorised into one of two mutually exclusive drug cohorts, based on the type of DMARD received (tofacitinib or bDMARDs). All continuous variables will be summarised using n (non-missing sample size), mean, standard deviation, median, minimum and maximum. The frequency and percentages (based on the non-missing sample size) or observed levels will be reported for all

categorical measures. Descriptive summaries will be produced for each data cut, providing there is sufficient data available, and again at the final analysis. All summaries are descriptive and there are no comparative analyses being undertaken, therefore, no adjustments for multiple data cuts and multiple endpoints are required. Patients who discontinue their index treatment (tofacitinib or bDMARD) will continue to be followed for a period of 1 year.

## Documents

**Study results**

A3921292 Non-Interventional Study Report Abstract 23 August 2021_Redacted.pdf(247.21 KB)

**Study report**

A3921292 Non Interventional Study Report 07 June 2021_Redacted.pdf(3.71 MB)

# Data management

## Data sources

**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**

No