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

Last updated: 31/05/2023

Study Finalised

Administrative details

EU PAS number

EUPAS18435

Study ID

27619

DARWIN EU® study

No

Study countries

 Australia

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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

Ng Ho Yin (Patrick)

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 topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Data collection methods:

Secondary use of data

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

Medicinal product name

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

3.000

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

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)

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