An Active Surveillance, Post-Authorization Study to Assess Tofacitinib Utilization Patterns and to Characterize the Safety of Tofacitinib Use in Patients with Moderately to Severely Active Ulcerative Colitis in the Real-World Setting Using Data from a US Administrative Healthcare Claims Database

First published: 29/06/2020

Last updated: 21/02/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS36041

Study ID

46136

DARWIN EU® study

No

Study countries

United States

Study description

The goal of the study is to understand the patterns of tofacitinib use in the US and to characterize the safety of tofacitinib (all approved formulations) in ulcerative colitis (UC) patients in the post-approval setting. The primary outcome of interest is malignancy, excluding non-melanoma skin (NMSC).

Study status

Ongoing

Research institutions and networks

Institutions

Pfizer

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Andrea Leapley

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 25/10/2019

Study start date

Planned: 30/06/2020

Actual: 30/06/2020

Data analysis start date

Planned: 30/06/2025

Date of final study report

Planned: 30/05/2026

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

Final_A3921347_Tofa UC_US Active Surveillance PASS Protocol_v3_4.10.2020.pdf (498.13 KB)

A3921347_PROTOCOL- UC US ACTIVE SURVEILLANCE_V3.0_30SEP2024.pdf (548.07 KB)

Regulatory

Was the study required by a regulatory body?

No

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

EU RMP category 3 (required)

Other study registration identification numbers and links

A3921347

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Main study objective:

The main objectives are to describe the utilization patterns of tofacitinib in the US with regard to on-label and off-label use, and to estimate the incidence rate of malignancy, excluding non-melanoma skin cancer (NMSC), among adult UC patients who initiate tofacitinib in the course of routine clinical care.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Active surveillance

Study drug and medical condition

Medicinal product name

XELJANZ

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

TOFACITINIB CITRATE

TOFACITINIB

Anatomical Therapeutic Chemical (ATC) code

(L04AF01) tofacitinib

tofacitinib

Medical condition to be studied

Colitis ulcerative

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- 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

11857

Study design details

Outcomes

1. Indication for tofacitinib use, classified as on-label, off-label, or unknown, based on medical events (diagnoses and/or medication use) identifiable around the time of the first ever tofacitinib prescription and,
2. Malignancy, excluding non-melanoma skin cancer in adult UC patients exposed to tofacitinib in the course of routine clinical care, NMSC, Serious infections, Opportunistic infections (e.g. tuberculosis), Herpes zoster (HZ) reactivation, Major adverse cardiac events (MACE), Venous thromboembolic events (VTE, deep venous thrombosis DVT and pulmonary embolism PE), Hepatic events, Progressive multifocal leukoencephalopathy (PML), Gastrointestinal (GI) perforations, Interstitial lung disease (ILD), Surgery for UC, Death

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Data analysis plan

For the drug utilization study, proportions of patients with an on-label, off-label, or unknown tofacitinib indication will be estimated (and described) with corresponding 95% confidence intervals (CIs). For the safety endpoints of interest, descriptive statistics, counts and proportions, cumulative incidence proportions, and incidence rates (number of events per 100 person-years) and associated 2-sided 95% CIs will be calculated as appropriate. Patients with a baseline history of an outcome of interest will be excluded from the calculation of the incidence rate for that particular outcome of interest (e.g. patients with a baseline history of malignancy will be excluded from the calculation of the incidence rate for malignancy). This will be a time to first event analysis based on an index date defined for each cohort with appropriate censoring rules applied (based on therapy switches, end of study, etc.) for those who do not experience an event by end of follow-up period.

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)

Administrative healthcare records (e.g., claims)

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