Non-interventional post-authorization effectiveness study to assess long-term outcomes of Nintedanib treatment in patients with systemic sclerosis associated interstitial lung disease (SSc-ILD) (Nintedanib long-term outcomes in patients with SSc)

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Study | Planned

Administrative details

PURI
https://redirect.ema.europa.eu/resource/106793

EU PAS number
EUPAS106792

Study ID
106793

DARWIN EU® study
No

Study countries
Argentina
Armenia
Austria
Belgium
Brazil
Bulgaria
Canada

China
Croatia
Czechia
Denmark
Dominican Republic
Egypt
Estonia
Finland
France
Germany
Greece
Hungary
Iran, Islamic Republic of
Ireland
Israel
Italy
Japan
Lithuania
Malta
Moldova, Republic of
Montenegro
Netherlands
New Zealand
Norway
Poland
Portugal
Romania
Russian Federation
Serbia
Slovenia
South Africa
Spain
Sweden
Switzerland
Türkiye
Ukraine
United Kingdom
United States

Study description

The main objective of this post-authorization effectiveness study is to assess the long-term effectiveness of Ofev® on a composite outcome of time to forced vital capacity (FVC) decline, lung transplantation (indicating end-stage ILD), or mortality, in addition, to other outcomes of interest in patients with SSc-ILD using data from the EUSTAR registry. The primary study objectives will be to assess long-term treatment effectiveness of Ofev® based on a composite outcome of time to FVC decline, time to lung transplantation, or death. The secondary study objectives will be: - To assess long-term treatment

effectiveness of Ofev® based on FVC decline, - To assess long-term treatment effectiveness of Ofev® based on time to lung transplantation (indicating end-stage ILD), - To assess long-term treatment effectiveness of Ofev® based on time to death, - To assess patterns of disease progression based on changes in FVC decline (overall and by treatment group) in patients with SSc-ILD, - To assess effects of other concomitant or previous therapies on a composite outcome of FVC decline, time to lung transplantation, or death (this does not correspond to an outcome but will be incorporated into the analyses), - To assess long-term effect of Ofev® on quality of life (QoL), - To assess the safety and effectiveness profile of Ofev® in the subset of patients with a diagnosis of pulmonary hypertension (PH) at baseline, and - To assess the incidence rates of 1) major bleeding (defined as requiring intervention or hospitalization), 2) gastrointestinal perforation, 3) thromboembolism (arterial or venous) by treatment group.

Study status
Planned

Research institution and networks

Networks

Eustar – European Scleroderma Trials & Research Group

Contact details

Study institution contact
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Study contact

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Primary lead investigator
Francesco Del Gado
Primary lead investigator

Study timelines

Date when funding contract was signed
Planned:

01/06/2023

Study start date
Planned:
01/08/2023

Data analysis start date
Planned:
01/08/2033

Date of interim report, if expected
Planned:
31/12/2025

Date of final study report
Planned:
31/12/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Boehringer-Ingelheim

Regulatory

Was the study required by a regulatory body?
Yes

Is the study required by a Risk Management Plan (RMP)?
Not applicable

Methodological aspects

Study type

Study type list

Study type:
Non-interventional study

Main study objective:
The main objective of this post-authorization effectiveness study is to assess the long-term effectiveness of Ofev® on a composite outcome of time to forced vital capacity (FVC) decline, lung transplantation (indicating end-stage ILD), or mortality, in addition, to other outcomes of interest in patients with SSc-ILD using data from the EUSTAR registry.

Study Design

Non-interventional study design
Cohort

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code
(L01EX09) nintedanib

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
2000

Study design details

Outcomes
To assess long-term treatment effectiveness of Ofev® based on a composite outcome of time to FVC decline, time to lung transplantation, or death. To assess long-term treatment effectiveness of Ofev® based on FVC decline, time to lung transplantation, time to death, QoL, patterns of disease progression based on changes in FVC decline, effects of concomittant or previous therapies, safety and effectiveness profile in patients with pulmonary hypertension, and incidence rate of major bleeding, gastrointestinal perforation,

and thromboembolism.

Data analysis plan
This ten-year study will include a two-year pilot analysis followed by analyses updated biennially. Descriptive statistics will be used to describe patient characteristics at baseline, primary and secondary outcomes. Univariate and bivariate distributions of exposures, outcomes, and relevant covariates will be summarized using frequencies and percentages for categorical variables and summary measures for continuous variables. Comparative analyses, if feasible, will be conducted using a Cox proportional hazards regression model with time-varying exposure to estimate HRs and 95% CIs. Ofev® treated patients will be propensity score matched to Ofev non-users to balance variables that are strongly prognostic for the outcomes. All primary and secondary outcomes will be analyzed, if feasible, using the aforementioned descriptive and comparative methods for the SSc-ILD sub-population with PH. Descriptive analyses of the safety outcomes will also be conducted by treatment group.

Data management

Data sources

Data source(s), other
European Scleroderma Trials and Research (EUSTAR) registry Switzerland

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