Non-interventional prospective study in patients with pulmonary fibrosis treated with nintedanib participating in a patient support program in Spain, to describe patient satisfaction with the program and to monitor quality of life. BALANCE Study

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Last updated: 04/07/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000550

Study ID

1000000550

DARWIN EU® study

No

Study countries

☐ Spain

Study description

Interstitial Lung Disease (ILD) are a heterogeneous group of diseases characterized by excessive deposition of extracellular matrix components within the pulmonary interstitium, leading to architectural distortion, irreversible pulmonary dysfunction, and early mortality.

Idiopathic pulmonary fibrosis (IPF) is the archetypal and most frequent fibrotic ILDs (accounts for 20% of them). In recent years, the term progressive pulmonary fibrosis (PPF) -also referred as progressive fibrosis ILD- has emerged. It refers to patients with ILDs -other than IPF- who develop pulmonary fibrosis of known or unknown cause, who present worsening respiratory symptoms, and physiological/ radiological evidence of progression.

Nintedanib reduces disease progression in IPF and PPF slows the decline in lung function. However, diarrhea is reported by 66.9% to 75.7% of nintedanib-treated and it is the main cause of discontinuation.

Patient support program (PSP) has been developed to help patients better manage their disease and adherence to treatments and it could be very helpful for these patients. Despite that, there is no evidence regarding patients' satisfaction and quality of life.

The aim of this study is to describe patients' satisfaction with PSP Balance Program, QoL and depression symptoms, dosing pattern, disease symptoms, adverse events and nintedanib discontinuation (both permanent and non-permanent) from study inclusion to 12 months of follow-up.

Study status

Ongoing

Research institutions and networks

Institutions

Boehringer Ingelheim

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Institution

Contact details

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Primary lead investigator

Beatriz Román

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 15/01/2025

Actual: 15/01/2025

Study start date

Planned: 25/06/2025

Actual: 23/06/2025

Data analysis start date
Planned: 23/11/2026

Date of interim report, if expected
Planned: 23/11/2025

Date of final study report
Planned: 01/03/2027

Sources of funding

- Pharmaceutical company and other private sector

Regulatory

Was the study required by a regulatory body?

No

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

Not applicable

Other study registration identification numbers and links

1199-0575

Methodological aspects

Study type

Study topic:

Disease /health condition

Study topic, other:

Pulmonary Fibrosis

Study type:

Non-interventional study

Scope of the study:

Evaluation of patient-reported outcomes

Data collection methods:

Primary data collection

Study design:

This is an observational and non-interventional study based on newly collected data with the cohort of IPF and PPF (other than IPF) patients participating in the PSP Balance Program. The study will not interfere with the pre-defined procedures of the PSP and will merely observe and and collect PROs.

Main study objective:

Primary objective:
To describe patient satisfaction with PSP Balance Program at study inclusion.

Secondary objectives:

Secondary objectives related to Program satisfaction:
- To describe the change in patient satisfaction with PSP Balance Program at 6 and 12 months after the inclusion in the study.

Secondary objectives related to treatment management and adverse events:
- To evaluate patient dosing pattern of nintedanib at 6 and 12 months after the inclusion in the study.
- To describe nintedanib management during the study period.

Secondary objectives related to patient reported outcomes:
- To describe the quality of life at baseline and 12 months after the inclusion in the study.
- To describe depression and anxiety symptoms at baseline and 12 months after the inclusion in the study.
- To describe the disease symptoms at baseline and 12 months after the inclusion in the study.
- To determine the number of hospitalization and visits to emergency service related to Interstitial Lung Disease from baseline to 12 months.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

OFEV

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

NINTEDANIB

Anatomical Therapeutic Chemical (ATC) code

(L01EX09) nintedanib

nintedanib

Population studied

Short description of the study population

This study will include patients with IPF and PPF (other than IPF) participating in the PSP Balance Program.

The study duration will be from the start of the recruitment period (which will last approximately 6 months) to the last follow-up visit of the last patient (approximately 18 months of total study period).

Patients will be followed-up at 6 months and at 12 months. Eligible patients will be recruited according to the following inclusion and exclusion criteria:

Inclusion criteria:

Adults (≥18 years old at baseline).

Patients included in the PSP Balance Program.

Ability to read and speak Spanish correctly according to the investigator criteria.

Agree to participate and sign informed consent at baseline.

Exclusion criteria:

Suspicion or diagnosis of any relevant cognitive impairment at the discretion of the investigator.

Age groups

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Study design details

Setting

In this study there is one unique study site, which is the following:

Evidenze Health España S.L.U.

Passatge de Ferrer i Vidal, 8, Sant Martí. 08005 Barcelona

This study site has already been selected as it will be the site providing the PSP Balance Program services.

3 study visits are planned in this study:

- Baseline
- 6 months (+/- 1 month)
- 12 months (+/- 1 month)

All study visits will coincide with routine PSP Balance Program visits, that means, no new visits will be schedule for this study. Following the PSP Balance Program criteria, if 6 or 12 months study visits could not be performed face to face, it will be done by phone. Baseline study visit will always coincide with one face-to-face PSP Balance Program visit.

All visits will be performed by study investigators. After the study visit, all study documents obtained during the visit (e.g., ICF, questionnaires, (S)AE form), will be sent to the study site for processing (data entry into the eCRF) and archival.

The Principal Investigator (PI) will delegate the collection of the study informed consent form and the study information to nurses providing the PSP Balance Program services. All nurses participating will be delegated as co-investigators. The study site will complete a site delegation list with all study tasks delegated to others than the PI.
The Sponsor will sign a contract with the study site and the Principal Investigator. All co-investigators will have a contract with the study site.

Outcomes

Primary Outcome: Patient satisfaction with PSP Balance Program at baseline using the Baker Questionnaire.

Secondary Outcomes:
- Change in the satisfaction with PSP Balance Program at 6 and 12-month follow-up after inclusion in the study, using the Baker Questionnaire.
- Patient satisfaction with PSP Balance Program at baseline, 6 and 12 months after the inclusion in the study using the Likert scale.
- Dosing pattern of nintedanib calculated as absolute number of doses taken correctly divided by all the doses prescribed in that period (6 and 12 months)
- Time from first dose to permanent discontinuation of nintedanib within 12-month follow-up.
- Absolute number of nintedanib dose interruptions.
- HRQoL at baseline and 12-month follow-up using the L-PF Impacts Questionnaire.
- Anxiety and depression scores in HADS Questionnaire at baseline and 12-month follow-up.
- Disease symptoms score in L-PF Symptoms Questionnaire at baseline and 12 months follow-up.
- Absolute number of visits to emergency service and hospitalization related to

ILD between baseline and 12-month follow-up.

Data analysis plan

A description of all patients included in the study will be performed. All patients participating in the study who meet the eligibility criteria will be included in the study population.

Descriptive statistics will be presented as absolute and relative frequencies for categorical variables. Mean, standard deviation, median, 25th – 75th quartiles, minimum and maximum will be reported for quantitative variables. Number of valid values (N valid) and number of missing values (N missing) will be reported for each variable.

A linear regression will be performed to assess the association between the outcomes and the independent variables (PSP duration). To evaluate the change in PSP satisfaction mixed models will be used.

Questionnaire scores provided by patients will be described using the descriptive measures described above, and according to the manual user.

Data will be analysed for the total evaluable population and for different subgroups (PSP duration) and different timepoints (baseline and 6 and 12-months follow-up), if applicable. If sample population allows, it will also be analysed by received treatment dose.

Same methodology will be used for the interim analysis (after basal data for all patients is collected) and the final analysis.

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.

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