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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Nintedanib for the treatment of idiopathic pulmonary fibrosis

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| Disclaimer<br>Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication. | |



On 26 April 2013 orphan designation (EU/3/13/1123) was granted by the European Commission to Boehringer Ingelheim International GmbH, Germany, for nintedanib for the treatment of idiopathic pulmonary fibrosis.

### What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a long-term disease of the lungs characterised by the progressive deposition of collagen and fibrous tissue in the lungs. This causes the lung tissue to become thick and to form scars. As a result, the lungs become unable to work normally, reducing the transfer of oxygen from the air into the blood. Patients with idiopathic pulmonary fibrosis have a persistent cough, frequent lung infections and shortness of breath that worsens over time.

Idiopathic pulmonary fibrosis is a life-threatening and long-term debilitating disease because the lungs gradually lose their ability to work properly.

### What is the estimated number of patients affected by the condition?

At the time of designation, idiopathic pulmonary fibrosis affected not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 154,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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## What treatments are available?

At the time of designation, Esbriet (pirfenidone) was the only medicine authorised in the EU to treat mild to moderate idiopathic pulmonary fibrosis.

The sponsor has provided sufficient information to show that nintedanib might be of significant benefit for patients with idiopathic pulmonary fibrosis based on results of early studies which showed fewer declines in lung function with nintedanib treatment compared with placebo (a dummy treatment). In addition nintedanib showed reductions in exacerbations (flare-up of symptoms) and an improvement in patients' quality of life.

These assumptions will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

## How is this medicine expected to work?

Enzymes called tyrosine kinases are known to be involved in development of idiopathic pulmonary fibrosis via a number of chemical pathways in the lungs that are related to the generation of fibrous tissue.

Nintedanib is a 'tyrosine-kinase inhibitor', which means that it blocks the actions of these enzymes, thereby interrupting the disease pathways and helping to reduce the formation of fibrous tissue in the lungs.

## What is the stage of development of this medicine?

The effects of nintedanib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with nintedanib in patients with idiopathic pulmonary fibrosis were ongoing.

At the time of submission, nintedanib was not authorised anywhere in the EU for idiopathic pulmonary fibrosis. Orphan designation of nintedanib has been granted in the United States of America and in Japan for idiopathic pulmonary fibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 March 2013 recommending the granting of this designation.

Update: nintedanib (Ofev) has been authorised in the EU since 15 January 2015. Ofev is indicated in adults for the treatment of Idiopathic Pulmonary Fibrosis (IPF).

More information on Ofev can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

## For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

## Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

| Language | Active ingredient | Indication |
|---|---|---|
| English | Nintedanib | Treatment of idiopathic pulmonary fibrosis |
| Bulgarian | нинтеданиб | Лечение на идиопатична белодробна фиброза |
| Czech | Nintedanib | Léčba idiopatické plicní fibrózy |
| Danish | Nintedanib | Behandling af idiopatisk lungefibrose |
| Dutch | Nintedanib | Behandeling van idiopathische longfibrose |
| Estonian | Nintedaniib | Idiopaatilise kopsufibroosi ravi |
| Finnish | Nintedanibi | Idiopaattisen keuhkofibroosin hoito |
| French | Nintedanib | Traitement de la fibrose pulmonaire idiopathique |
| German | Nintedanib | Behandlung von idiopathischer pulmonaler Fibrose |
| Greek | Νιντεντανίμπη | Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης |
| Hungarian | Nintedanib | Idiopathiás tüdőfibrózis kezelése |
| Italian | Nintedanib | Trattamento della fibrosi polmonare idiopatica |
| Latvian | Nintedanibs | Idiopātiskās plaušu fibrozes ārstēšana |
| Lithuanian | Nintedanibas | Idiopatinės plaučių fibrozės gydymas |
| Maltese | Nintedanib | Kura tal-fibrożi pulmonari idjopatika |
| Polish | Nintedanib | Leczenie idiopatycznego zwłóknienia płuc |
| Portuguese | Nintedanib | Tratamento da fibrose pulmonar idiopática |
| Romanian | Nintedanib | Tratamentul fibrozei pulmonare idiopatice |
| Slovak | Nintedanib | Liečba idiopatickej pľúcnej fibrózy |
| Slovenian | Nintedanib | Zdravljenje idiopatske pljučne fibroze |
| Spanish | Nintedanib | Tratamiento de la fibrosis pulmonar idiopática |
| Swedish | Nintedanib | Behandling av idiopatisk lungfibros |
| Norwegian | Nintedanib | Behandling av idiopatisk lungefibrose |
| Icelandic | Nintedaníb | Meðferð sjálfvakinnar bandvefsmyndunar í lungum |

[1] At the time of designation