



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

13 November 2020  
EMADOC-628903358-2737

## Public summary of opinion on orphan designation

Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfane for the treatment of idiopathic pulmonary fibrosis

On 21 August 2020, orphan designation EU/3/20/2309 was granted by the European Commission to Galecto Biotech AB, Denmark, for Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfane (also known as TD139) for the treatment of idiopathic pulmonary fibrosis.

### What is idiopathic pulmonary fibrosis?

Idiopathic pulmonary fibrosis is a disease in which scar tissue (collagen and fibrous tissue) gradually builds up in the lungs, making their structure thicker and less flexible. As a result, the lungs no longer work normally, reducing the amount of oxygen that gets 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 long-term debilitating and life-threatening 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 approximately 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 166,000 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).

### What treatments are available?

At the time of designation, Esbriet (pirfenidone) and Ofev (nintedanib) were authorised in the EU to treat idiopathic pulmonary fibrosis.

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\*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with idiopathic pulmonary fibrosis. Early data suggest that the medicine may be more effective than authorised medicines in patients with IPF.

This assumption 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?**

The medicine blocks the action of a protein called galectin-3, which is involved in the production of fibrous tissue in organs including the lungs, and is thought to play a role in the development of IPF. By blocking the activity of galectin-3, the medicine is expected to reduce build-up of fibrous tissue in the lungs, thereby improving their functioning.

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

The effects of bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfane have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of idiopathic pulmonary fibrosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 16 July 2020, recommending the granting of this designation.

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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**

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfane	Treatment of idiopathic pulmonary fibrosis
Bulgarian	бис-(3-дезоксi-3-(4-(3-флуорофенил)-1H-1,2,3-триазол-1-ил)-бета-D-галактопиранозил) сулфан	Лечение на идиопатична белодробна фиброза
Croatian	Bis-(3-deoksi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galaktopiranozil) sulfan	Liječenje idiopatske plućne fibroze
Czech	Bis-(3-deoxy-3-(4-(3-fluorofenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galaktopyranosyl) sulfan	Léčba idiopatické plicní fibrózy
Danish	Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfan	Behandling af idiopatisk lungefibrose
Dutch	Bis-(3-deoxy-3-(4-(3-fluorofenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfaan	Behandeling van idiopathische longfibrose
Estonian	Bis-(3-desoksü-3-(4-(3-fluorofenüül)-1H-1,2,3-triasool-1-üül)-beeta-D-galaktopüranosüül) sulfaan	Idiopaatilise kopsufibroosi ravi
Finnish	Bis-(3-deoksi-3-(4-(3-fluorofenyli)-1H-1,2,3-triatsoli-1-yyli)-beeta-D-galaktopyranosyyli) sulfaani	Idiopaattisen keuhkofibroosin hoito
French	Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfane	Traitement de la fibrose pulmonaire idiopathique
German	Bis-(3-deoxy-3-(4-(3-fluorophenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galactopyranosyl) sulfan	Behandlung von idiopathischer pulmonaler Fibrose
Greek	Δις-(3-δεοξυ-3-(4-(3-φθοροφαινυλο)-1H-1,2,3-τριαζολ-1-υλο)-β-D-γαλακτοπιρανοζυλο) σουλφάνιο	Θεραπεία της ιδιοπαθούς πνευμονικής ίνωσης
Hungarian	Bisz-(3-deoxi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-béta-D-galaktopiranozil) szulfán	Idiopathiás tüdőfibrózis kezelése
Italian	Di-(3-desossi-3-(4-(3-fluorofenil)-1-H-1,2,3-triazol-1-il)-beta-D-galattopiranosil) solfano	Trattamento della fibrosi polmonare idiopatica
Latvian	Bis-(3-deoksi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-bēta-D-galaktopiranozil) sulfāns	Idiopātiskās plaušu fibrozes ārstēšana
Lithuanian	Bis-(3-deoksi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galaktopiranozil) sulfanas	Idiopatinės plaučių fibrozės gydymas
Maltese	Bis-(3-deossi-3-(4-(3-fluorofenil)-1H-1,2,3-triażol-1-il)-beta-D-galattopiranosil) sulfan	Kura tal-fibrożi pulmonari idjopatika

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Polish	Bis-(3-deoksy-3-(4-(3-fluorofenylo)-1H-1,2,3-triazol-1-ilo)-beta-D-galaktopiranozylo) siarczan	Leczenie idiopatycznego zwłóknienia płuc
Portuguese	Sulfano bis-(3-desoxi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galactopiranosil)	Tratamento da fibrose pulmonar idiopática
Romanian	bis-(3-deoxi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galactopiranozil)-sulfan	Tratamentul fibrozei pulmonare idiopatice
Slovak	Bis(3-deoxy-3-(4-(3-fluórfenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galaktopyranozyl) sulfid	Liečba idiopatickej pľúcnej fibrózy
Slovenian	bis-(3-deoksi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galaktopiranozil) sulfan	Zdravljenje idiopatske pljučne fibroze
Spanish	Bis-(3-desoxi-3-(4-(3-fluorofenil)-1H-1,2,3-triazol-1-il)-beta-D-galactopiranosil) sulfano	Tratamiento de la fibrosis pulmonar idiopática
Swedish	Bis-(3-deoxi-3-(4-(3-fluorofenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galaktopyranosyl) sulfan	Behandling av idiopatisk lungfibros
Norwegian	Bis-(3-deoksy-3-(4-(3-fluorofenyl)-1H-1,2,3-triazol-1-yl)-beta-D-galaktopyranosyl) sulfan	Behandling av idiopatisk lungefibrose
Icelandic	Bis-(3-deoxý-3-(4-(3-flúorófenýl)-1H-1,2,3-tríazól-1-ýl)-beta-D-galaktópýranósýl) súlfan	Meðferð sjálfvakinnar bandvefsmýndunar í lungum