



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

3- {[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl} thiophene-2-carboxylic acid for the treatment of non-infectious uveitis

On 19 June 2015, orphan designation (EU/3/15/1507) was granted by the European Commission to Panoptes Pharma GesmbH, Austria, for 3- {[2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl]carbamoyl} thiophene-2-carboxylic acid for the treatment of non-infectious uveitis.

What is non-infectious uveitis?

Uveitis is inflammation of the uvea, the middle layer of the eye, just beneath the white part of the eye. The inflammation can affect one or both eyes, and may cause discomfort, pain, and blurring of vision. Non-infectious uveitis is usually caused by the body's immune system (the body's natural defences) attacking normal tissue and not by an infection.

Non-infectious uveitis is a long-term debilitating disease because it may lead to partial or complete loss of vision (blindness).

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

At the time of designation, non-infectious uveitis affected less than 4.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 246,000 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, several medicines were authorised in Member States of the EU for the treatment of non-infectious uveitis. The first treatment option was corticosteroids, which were used to reduce the inflammation by lowering the activity of the immune system. Other immunosuppressant

*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. This represents a population of 512,900,000 (Eurostat 2015).



agents (that reduce the activity of the immune system) such as ciclosporin were also authorised for use in non-infectious uveitis.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with non-infectious uveitis because, since the medicine is expected to be given as an injection into the eye, it is expected to have only a local effect in the eye. Use of this medicine may reduce the need to use corticosteroids and immunosuppressants that have effects on the whole body. 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?

This medicine is expected to block an enzyme called dihydroorotate dehydrogenase (DHODH), which is involved in the production of substances called 'pyrimidines' inside cells. Pyrimidines are needed for the growth of certain cells of the immune system (called auto-reactive T-cells) which in patients with uveitis attack normal tissue and cause inflammation. By reducing the growth of these cells, the medicine is expected to lead to a reduction in inflammation and damage to the uvea, thus improving the symptoms of the condition.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with non-infectious uveitis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for non-infectious uveitis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 May 2015 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	3-{{2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl}carbamoyl} thiophene-2-carboxylic acid	Treatment of non-infectious uveitis
Bulgarian	3-{{2,3,5,6-тетрафлуоро-3'-(трифлуорометокси)бифенил-4-ил}карбамоил} тиофен-2-карбоксилна киселина	Лечение на хроничен неинфекциозен увеит
Croatian	3-{{2,3,5,6-tetrafluoro-3'-(trifluorometoksi)bifenil-4-il}karbamoil} tiofen-2-karboksilatna kiselina	Liječenje neinfektivnog uveitisa
Czech	3-{{2,3,5,6-tetrafluor-3'-(trifluormethoxy)bifenyl-4-yl}karbamoyl} thiofen-2-karboxylová kyselina	Léčba neinfekční uveitidy
Danish	3-{{2,3,5,6-tetrafluor-3'-(trifluormethoxy)biphenyl-4-yl}carbamoyl} tiophen-2-karboxylsyre	Behandling af non-infektios uveitis
Dutch	3-{{2,3,5,6-tetrafluor-3'-(trifluormethoxy)bifenyl-4-yl}carbamoyl} thiofeen-2-carboxylzuur	Behandeling van niet-infectieuze uveitis
Estonian	3-{{2,3,5,6-tetrafluoro-3'-(trifluorometoksü)bifenüül-4-üül}karbamoüül} tiofeen-2-karboksüülhape	Mitte-infektsioosse uveiidi ravi
Finnish	3-{{2,3,5,6-tetrafluoro-3'-(trifluorometoksi)bifenyyl-4-yyli}karbamoyyli} tiofeeni-2-karboksyylihappo	Ei-infektioeräisen uveitin hoito
French	Acide 3-{{2,3,5,6-tétrafluoro-3'-(trifluorométhoxy)biphényle-4-yl}carbamoyl} thiophène-2-carboxylique	Traitement de l'uvéite non infectieuse
German	3-{{2,3,5,6-Tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl}carbamoyl} thiophen-2-carbonsäure	Behandlung der nicht-infektiösen Uveitis
Greek	3-{{2,3,5,6- τετραφθορο-3'-(τριφθορομεθοξυ)διφαινυλο-4-υλ} καρβαμοϋλο}θειοφαινο-2-καρβοξυλικό οξύ	Θεραπεία της μη μολυσματικής ραγοειδίτιδας.
Hungarian	3-{{2,3,5,6-tetrafluoro-3'-(trifluorometoxi)bifenil-4-il}karbamoil} tiofén-2-karboxilsav	Nem fertőzéses eredetű uveitis kezelése
Italian	Acido 3-{{2,3,5,6-tetrafluoro-3'-(trifluorometossi)bifenil-4-il}carbamoil} tiofene-2-carbossilico	Trattamento dell'uveite non infettiva
Latvian	3-{{2,3,5,6-tetrafluor-3'-(trifluormetoksi)bifenil-4-il}karbamoil} tiofēn-2-karbonskābe	Ne-infekciozā uveīta ārstēšana
Lithuanian	3-{{2,3,5,6-tetrafluoro-3'-(trifluorometoksi)bifenil-4-il}karbamoil} tiofeno-2-karboksirūgštis	Neinfekcinio uveito gydymas
Maltese	3-{{2,3,5,6-tetrafluoro-3'-(trifluoromethoxy)biphenyl-4-yl}carbamoyl} thiophene-2-carboxylic acid	Kura ta' uveite mhux infettiva

¹ At the time of designation

Language	Active ingredient	Indication
Polish	kwasy 3-{ [2,3,5,6-tetrafluoro-3'-(trifluorometoksy)bifenyl-4-yl]karbamoilo} tiofeno-2-karboksylowy	Leczenie nieinfekcyjnego zapalenia błony naczyniowej oka
Portuguese	Ácido 3-{ [2,3,5,6-tetrafluoro-3'-(trifluorometóxi)bifenil-4 il]carbamoil} tiofeno-2-carboxílico	Tratamento da uveíte não infecciosa
Romanian	Acid 3-{ [2,3,5,6-tetrafluoro-3'-(trifluorometoxi)bifenil-4 il]carbamoil} tiofen-2-carboxilic	Tratamentul uveitei non-infectioase
Slovak	Kyselina 3-{ [2,3,5,6-tetrafluór-3'-(trifluórmetyoxy)bifenyl-4-yl]karbamoyl} tiofén-2-karboxylová	Liečba neinfekčnej uveitídy
Slovenian	3-{ [2,3,5,6-tetrafluoro-3'-(trifluorometoksi)bifenil-4 il]karbamoil} tiofen-2-karboksilna kislina	Zdravljenje neinfekcijskega uveitis
Spanish	ácido 3-{ [2,3,5,6-tetrafluoro-3'-(trifluorometoxi)bifenil-4 il]carbamoil} tiofeno-2-carboxílico	Tratamiento de la uveitis no infecciosa
Swedish	3-{ [2,3,5,6-tetrafluor-3'-(trifluormetoxi)bifenyl-4-yl]karbamoyl} tiofen-2-karboxylsyra	Behandling av icke-infektiös uveit
Norwegian	3-{ [2,3,5,6-tetrafluor-3'-(trifluormetoksy) bifenyl-4-yl]karbamoyl} tiofen-2-karboksylysyre	Behandling av ikke-infeksiøs uveitt
Icelandic	3-{ [2,3,5,6-tetraflúor-3'-(triflúormetoxý) bifenýl-4-ýl]karbamóýl} tíófen-2-karboxýlsýra	Meðferð á æðahjúpsbólgu án sýkingar