



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide for the treatment of scedosporiosis

On 29 August 2016, orphan designation (EU/3/16/1713) was granted by the European Commission to F2G Ltd, United Kingdom, for 2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide (also known as F901318) for the treatment of scedosporiosis.

What is scedosporiosis?

Scedosporiosis is an infection caused by a group of fungi called *Scedosporium species*. Infection mainly occurs through inhalation of spores and can then spread within the body. Scedosporiosis is usually seen in patients with a weakened immune (defence) system and it is often resistant to many antifungal medicines.

Scedosporiosis is a life-threatening and long-term debilitating disease because it can spread throughout the body and lead to infections in the bone, heart and brain.

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

At the time of designation, scedosporiosis affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 500 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 submission of the application for orphan drug designation, the only product authorised for scedosporiosis was the antifungal medicine voriconazole. Surgery was also used to treat the condition.

^{*}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 513,700,000 (Eurostat 2016).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with scedosporiosis because laboratory studies showed that it could alleviate fungal infections that are resistant to other antifungal medicines. 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?

This medicine is expected to block production of pyrimidine in the fungus that causes scedosporiosis. Because the fungus needs pyrimidine for its genetic material, blocking its production prevents the fungus from multiplying and spreading.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with the condition were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for scedosporiosis 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 July 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Treatment of scedosporiosis
Bulgarian	2-(1,5-диметил-3-фенил-1Н-пирол-2-ил)-N-{4-[4-(5-флуоро-пиримидин-2-yl)пиперазин-1-ил]-фенил}-2-оксо-ацетамид	Лечение на скедоспориоза
Croatian	2-(1,5-dimetil-3-fenil-1H-pirol-2-yl)-N-{4-[4-(5-fluoro-pirimidin-2-yl)piperazin-1-il]-fenil}-2-okso-acetamid	Liječenje scedosporioze
Czech	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Léčba scedosporiosy
Danish	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Behandling af scedosporiosis
Dutch	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Behandeling van scedosporiosis
Estonian	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Scedosporioosi ravi
Finnish	2-(1,5-dimetyyli-3-fenyyli-1H-pyrroli-2-yl)-N-{4-[4-(5-fluoro-pyrimidiini-2-yl)piperatsiini-1-yl]-fenyyli}-2-okso-asetamidi	Skedosporioosin hoito
French	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Traitement des infections à Scedosporium
German	2-(1,5-Dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamid	Behandlung der Scedopsoriasis
Greek	2-(1,5-διμεθυλ-3-φενυλ-1Η-πυρρολ-2-yl)-N-{4-[4-(5-φλουορο-πυριμιδινη-2-yl)πιπεραζινη-1-yl]-φενυλ}-2-οξο-ακεταμίδη	θεραπεία λοιμώξεων από Scedosporium
Hungarian	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamid	Scedosporiosis kezelése
Italian	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Trattamento della scedosporiosi
Latvian	2-(1,5-dimetil-3-fenil-1H-pirrol-2-il)-N-{4-[4-(5-fluoro-pirimidīn-2-il)piperazīn-1-il]-fenil}-2-okso-acetamīds	Scedosporiozes ārstēšana

¹ At the time of designation

Language	Active ingredient	Indication
Lithuanian	2-(1,5-dimetil-3-fenil-1H-pirol-2-il)-N-{4-[4-(5-fluoro-pirimidin-2-il)piperazin-1-il]-fenil}-2-okso-acetamidas	Skedosporiozės gydymas
Maltese	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Kura tas-sedosporjosi
Polish	2-(1,5-dimetylo-3-fenyl-1H-pirol-2-yl)-N-{4-[4-(5-fluoro-pyrimidyno-2-yl)piperazyn-1-yl]-fenyl}-2-okso-acetamid	Leczenie scedosporiozy
Portuguese	2-(1,5-dimetil-3-phenil-1H-pirrol-2-il)-N-{4-[4-(5-fluoropirimidin-2-il)piperazin-1-il]-phenyl}-2-oxoacetamida	Tratamento da scedosporiose
Romanian	2-(1,5-dimetil-3-fenil-1H-pirol-2-il)-N-{4-[4-(5-fluoro-pirimidin-2-il)piperazin-1-il]-fenil}-2-oxo-acetamidă	Tratamentul scedosporiozei
Slovak	2-(1,5-dimetyl-3-fenyl-1H-pyrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazín-1-yl]-fenyl}-2-oxo-acetamid	Liečba scedosporiosis
Slovenian	2-(1,5-dimetil-3-fenil-1H-pirol-2-il)-N-{4-[4-(5-fluoro-pyrimidin-2-il)piperazin-1-il]-fenil}-2-okso-acetamid	Zdravljenje scedosporiaze
Spanish	2-(1,5-dimethyl-3-fenil-1H-pirrol-2-il)-N-{4-[4-(5-fluoro-pirimidin-2-il)piperazin-1-il]-fenil}-2-oxo-acetamide	Tratamiento de scedosporiosis
Swedish	2-(1,5-dimethyl-3-phenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-phenyl}-2-oxo-acetamide	Behandling av scedosporiosis
Norwegian	2-(1,5-dimetyl-3-fenyl-1H-pyrrol-2-yl)-N-{4-[4-(5-fluoro-pyrimidin-2-yl)piperazin-1-yl]-fenyl}-2-oxo-acetamid	Behandling av scedosporiosis
Icelandic	2-(1,5-dímetyl-3-phenýl-1H-pýrról-2-yl)-N-{4-[4-(5-flúóró-pýrimídín-2-ýl)píperazín-1-ýl]-phenýl}-2-oxó-acetamíð	Meðferð scedosporiosis