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EMA/COMP/314054/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide for the treatment of retinitis pigmentosa

On 30 May 2016, orphan designation (EU/3/16/1658) was granted by the European Commission to Shire Pharmaceuticals Ireland Limited, Ireland, for 4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide for the treatment of retinitis pigmentosa.

What is retinitis pigmentosa?

Retinitis pigmentosa is a group of hereditary diseases of the eye that lead to progressive loss of sight. In patients with retinitis pigmentosa, cells in the retina (the light-sensitive surface at the back of the eye) become damaged and eventually die.

Retinitis pigmentosa is a long-term debilitating disease because it causes the patient's sight to get worse, eventually leading to blindness.

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

At the time of designation, retinitis pigmentosa affected approximately 2.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 144,000 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, no satisfactory methods were authorised in the EU for treating retinitis pigmentosa. Patients with the condition were given sunglasses to slow down the damage to the retina, genetic counselling (discussion of the risks of passing the condition on to children) and general support.

*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).

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How is this medicine expected to work?

One form of retinitis pigmentosa is caused by mutations (changes) in the gene responsible for the production of a protein called rhodopsin, which is essential for the normal functioning of retinal cells. In patients with this form of the disease this protein is misshaped and does not function properly.

This medicine stabilises the mutated rhodopsin protein, enabling it to fold properly and performs its function. This is expected to slow down the progression of the disease.

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 retinitis pigmentosa had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for retinitis pigmentosa. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 April 2016 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

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide	Treatment of retinitis pigmentosa
Bulgarian	4-[(2E)-1-оксо-3-(2,6,6-триметил-1-циклохексен-1-ил)-2-пропен-1-ил]-1-пиперазинкарбоксамид	Лечение на пигментен ретинит
Croatian	4-[(2E)-1-okso-3-(2,6,6-trimetil-1-cikloheksen-1-il)-2-propen-1-il]-1-piperazinkarboksamid	Liječenje retinitisa pigmentoze
Czech	4-[(2E)-1-oxo-3-(2,6,6-trimetyl-1-cyklohexen-1-yl)-2-propen-1-yl]-1-piperazin karboxamid	Léčba pigmentosní retinitidy
Danish	4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazincarboxamid	Behandling af retinitis pigmentosa
Dutch	4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexeen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide	Behandeling van retinitis pigmentosa
Estonian	4-[(2E)-1-okso-3-(2,6,6-trimetüül-1-tsüklohekseen-1-üül)-2-propeen-1-üül]-1-piperasiinkarboksamiid	Pigmentoosse võrkkestapõletiku ravi
Finnish	4-[(2E)-1-okso-3-(2,6,6-trimetyyli-1-syklohekseeni-1-yyli)-2-propeeni-1-yyli]-1-piperatsiinikarboksamidi	Verkkokalvorappeuman hoito
French	4-[(2E)-1-oxo-3-(2,6,6-triméthyl-1-cyclohexène-1-yl)-2-propène-1-yl]-1-pipérazinecarboxamide	Traitement de la rétinite pigmentaire
German	4-[(2E)-1-Oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazincarboxamid	Behandlung der Retinopathia Pigmentosa
Greek	4-[(2E)-1-οξο-3-(2,6,6-τριμεθυλ-1-κυκλοξεν-1-υλ)-2-προπεν-1-υλ]-1-πιπεραζινοκαρβοξαμίδη	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	4-[(2E)-1-oxo-3-(2,6,6-trimetil-1-ciklohexén-1-il)-2-propén-1-il]-1-piperazin-karboxamid	Retinitis pigmentosa kezelése
Italian	4-[(2E)-1-osso-3-(2,6,6-trimetil-1-cicloesen-1-il)-2-propen-1-il]-1-piperazinacarbossamide	Trattamento della retinite pigmentosa
Latvian	4-[(2E)-1-okso-3-(2,6,6-trimetil-1-cikloheksēn-1-il)-2-propēn-1-il]-1-piperazīna karboksamīds	*Retinitis pigmentosa* ārstēšana
Lithuanian	4-[(2E)-1-okso-3-(2,6,6-trimetil-1-cikloheksen-1-il)-2-propen-1-il]-1-piperazino karboksamidas	Pigmentinio retinito gydymas
Maltese	4-[(2E)-1-oxo-3-(2,6,6-trimethyl-1-cyclohexen-1-yl)-2-propen-1-yl]-1-piperazinecarboxamide	Kura tar-retinite pigmentuża
Polish	4-[(2E)-1-okso-3-(2,6,6-trimetylo-1-cykloheksen-1-ylo)-2-propen-1-ylo]-1-piperazynokarboksyamid	Leczenie retinopatii barwnikowej
Portuguese	4-[(2E)-1-oxo-3-(2,6,6-trimetil-1-ciclo-hexeno-1-il)-2-propen-1-il]-1-piperazinacarboxamida	Tratamento da retinite pigmentosa
Romanian	4-[(2E)-1-oxo-3-(2,6,6-trimetil-1-ciclohexen-1-il)-2-propen-1-il]-1-piperazincarboxamidă	Tratamentul retinitei pigmentare
Slovak	4-[(2E)-1-oxo-3-(2,6,6-trimetyl-1-cyklohexén-1-yl)-2-propén-1-yl]-1-piperazínkarboxamid	Liečba retinitis pigmentosa

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	4-[(2E)-1-okso-3-(2,6,6-trimetil-1-cikloheksen-1-il)-2-propen-1-il]-1-piperazinkarboksamid	Zdravljenje pigmentozne retinopatije
Spanish	4-[(2E)-1-oxo-3-(2,6,6-trimetil-1-ciclohexen-1-il)-2-propen-1-il]-1-piperazincarboxamida	Tratamiento de la retinosis pigmentaria
Swedish	4-[(2E)-1-oxo-3-(2,6,6-trimetyl-1-cyklohexen-1-yl)-2-propen-1-yl]-1-piperazinkarboxamid	Behandling av retinitis pigmentosa
Norwegian	4-[(2E)-1-okso-3-(2,6,6-trimetyl-1-sykloheksen-1-yl)-2-propen-1-yl]-1-piperazinkarboksamid	Behandling av retinitis pigmentosa
Icelandic	4-[(2E)-1-oxó-3-(2,6,6-trímetýl-1-sýklóhexen-1-ýl)-2-própen-1-ýl]-1-píperasínkarboxamíð	Meðferð á retinitis pigmentosa