



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo [3,2-d][1,3,2] dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one for the treatment of retinitis pigmentosa

On 19 March 2015, orphan designation (EU/3/15/1462) was granted by the European Commission to Universitätsklinikum Tübingen, Germany, for sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo [3,2-d][1,3,2] dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one 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 worsen over time, eventually leading to blindness.

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

At the time of designation, retinitis pigmentosa affected less than 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 190,000 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 and genetic counselling on the risks of passing the condition on to children.

^{*}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).



How is this medicine expected to work?

In some patients with retinitis pigmentosa genetic defects lead to an increase in levels of a chemical called cGMP which causes toxicity and can lead to the death of retinal cells.

This medicine is expected to work against cGMP by blocking the action of an enzyme called protein kinase G, through which cGMP exerts its action. By attaching to and blocking protein kinase G, the medicine is expected to help reduce the effects of cGMP and thereby prevent death of retinal cells and improve the patient's 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 retinitis pigmentosa had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for retinitis pigmentosa 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 12 February 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	Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one	Treatment of retinitis pigmentosa
Bulgarian	Натрий 3-[(4aR,6R,7R,7aS)-7-хидрокси-2-оксидо-2-сулфанилиден-4a,6,7,7a-тетраhydro-4H-фуоро [3,2-d][1,3,2]диоксафосфинин-6-ил]-2-бromo-6-фенил-5H-имидазо[1,2-a]пурин-9-он	Лечение на пигментен ретинит
Croatian	3-[(4aR,6R,7R,7aS)-7-hidroksi-2-oksido-2-sulfaniliden-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioksafosfinin-6-il]-2-brom-6-fenil-5H-imidazo[1,2-a]purin-9-onnatrij	Liječenje retinitisa pigmentoze
Czech	Sodný 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-brom-6-fenyl-5H-imidazo[1,2-a]purin-9-onu	Léčba pigmentosní retinitidy
Danish	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanyliden-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-on	Behandling af retinitis pigmentosa
Dutch	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxide-2-sulfanylideen-4a,6,7,7a-tetrahydro-4 H-furo[3,2-d][1,3,2]dioxaphosphinine-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purine-9-one	Behandeling van retinitis pigmentosa
Estonian	Naatrium 3-[(4aR,6R,7R,7aS)-7-hüdroksü-2-oksido-2-sulfanülideen-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioksafosfiniin-6-üül]-2-bromo-6-fenüül-5H-imidaso[1,2-a]purin-9-oon	Pigmentoosse võrkkestapõletiku ravi
Finnish	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroksi-2-oksido-2-sulfanylideeni-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioksafosfiniini-6-yl]-2-bromo-6-fenyyli-5H-imidatso[1,2-a]puriini-9-oni	Verkkokalvorappeuman hoito
French	Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidèn-4a,6,7,7a-tétrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phényl-5H-imidazo[1,2-a]purin-9-one	Traitement de la rétinite pigmentaire
German	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxo-2-sulfanyliden-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-brom-6-phenyl-5H-imidazo[1,2-a]purin-9-on	Behandlung der Retinopathia Pigmentosa

¹ At the time of designation

Language	Active ingredient	Indication
Greek	Νατριούχος 3-[(4aR,6R,7R,7aS)-7-υδροξυ-2-οξειδο-2-σουλφανιλιδινο-4a,6,7,7a-τετραϋδρο-4H-φουρο [3,2-d][1,3,2]δυσοξαφωσφινίνη-6-υλ]-2-βρωμο-6-φαινυλ-5H-ιμιδαζόλ [1,2-a]πουρίνη-9-όνη	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Nátrium 3-[(4aR,6R,7R,7aS)-7-hiroxi-2-oxido-2-szulfaniliden-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioxa-foszfínin-6-yl]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-on	Retinitis pigmentosa kezelése
Italian	Sodio 3-[(4aR,6R,7R,7aS)-7-idrossi-2-ossido-2-sulfanilidene-4a,6,7,7a-tetraidro-4H-furo[3,2-d][1,3,2]diossafosfinin-6-il]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-one	Trattamento della retinite pigmentosa
Latvian	Nātrija 3-[(4aR,6R,7R,7aS)-7-hidroksi-2-oksido-2-sulfanilidēn-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioksafosfinīn-6-il]-2-brom-6-fenil-5H-imidazo[1,2-a]purīn-9-ons	Retinitis pigmentosa ārstēšana
Lithuanian	Natrio 3-[(4aR,6R,7R,7aS)-7-hidroksi-2-oksido-2-sulfaniliden-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioksafosfinin-6-il]-2-brom-6-fenil-5H-imidazo[1,2-a]purin-9-onas	Pigmentinio retinito gydymas
Maltese	Sodium 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bromo-6-phenyl-5H-imidazo[1,2-a]purin-9-one	Kura tar-retinite pigmentuža
Polish	3-[(4aR,6R,7R,7aS)-7-hydroksy-2-oksydo-2-sulfanylideno-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioksafosfinin-6-ylo]-2-bromo-6-fenylo-5H-imidazo[1,2-a]puryn-9-on sodu	Leczenie retinopatii barwnikowej
Portuguese	Sódio 3-[(4aR,6R,7R,7aS)-7-hidroxi-2-óxido-2-sulfanilidena-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioxafosfinina-6-yl]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-one	Tratamento da retinite pigmentosa
Romanian	Sodiu 3-[(4aR,6R,7R,7aS)-7-hidroxi-2-oxido-2-sulfaniliden-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]-dioxafosfinin-6-il]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-onă	Tratamentul retinitei pigmentare
Slovak	Sodný 3-[(4aR,6R,7R,7aS)-7-hydroxy-2-oxido-2-sulfanylidene-4a,6,7,7a-tetra-hydro-4H-furo[3,2-d][1,3,2]dioxaphosphinin-6-yl]-2-bróm-6-fenyl-5H-imidazo[1,2-a]purinov-9-onu	Liečba retinitis pigmentosa
Slovenian	Natrijev 3-[(4aR,6R,7R,7aS)-7-hidroksi-2-oksido-2-sulfanylidene-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioksafosfinin-6-il]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-on	Zdravljenje pigmentozne retinopatije

Language	Active ingredient	Indication
Spanish	Sodio 3-[(4aR,6R,7R,7aS)-7-hidroxi-2-oxido-2-sulfanilidene-4a,6,7,7a-tetrahidro-4H-furo[3,2-d][1,3,2]dioxafosfinina-6-yl]-2-bromo-6-fenil-5H-imidazo[1,2-a]purin-9-ona	Tratamiento de retinosis pigmentaria
Swedish	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroxi-2-oxido-2-sulfanyliden-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioxafosfinin-6-yl]-2-bromo-6-fenyl-5H-imidazo[1,2-a]purin-9-on	Behandling av retinitis pigmentosa
Norwegian	Natrium 3-[(4aR,6R,7R,7aS)-7-hydroksy-2-oksidó-2-sulfanyliden-4a,6,7,7a-tetrahydro-4H-furo[3,2-d][1,3,2]dioksafosfinin-6-yl]-2-bromo-6-fenyl-5H-imidazo[1,2-a]purin-9-on	Behandling av retinitis pigmentosa
Icelandic	Natríum 3-[(4aR,6R,7R,7aS)-7-hýdroxý-2-oxídó-2-súlfanylidene-4a,6,7,7a-tetrahydró-4H-fúró[3,2-d][1,3,2]díoxaphosphínín-6-ýl]-2-brómó-6-fenýl-5H-ímídasó[1,2-a]púrín-9-ón	Meðferð á retinitis pigmentosa