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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Unoprostone isopropyl for the treatment of retinitis pigmentosa

First publication	9 July 2013
Rev.1: sponsor's change of address	10 March 2015
Disclaimer	
<p>Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.</p>	

On 19 June 2013, orphan designation (EU/3/13/1146) was granted by the European Commission to Sucampo Pharma Europe Ltd, United Kingdom, for unoprostone isopropyl 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?

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).

\*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. At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

## **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.

## **How is this medicine expected to work?**

Unoprostone isopropyl is a medicine authorised outside Europe for other eye disorders. The exact way in which unoprostone isopropyl works in retinitis pigmentosa is not known although the medicine is known to affect potassium and chloride channels, which are pores through which the essential substances potassium and chloride move out of cells, and which are widely found in the eye. Experiments suggest that the medicine has a number of actions that could benefit patients with the condition: suppression of the programmed cell death of the retinal cells, improvement of blood flow in the retina and improving the clearing up of retinal debris.

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

The effects of unoprostone isopropyl have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with unoprostone isopropyl in patients with retinitis pigmentosa were ongoing.

At the time of submission, unoprostone isopropyl was not authorised anywhere in the EU for retinitis pigmentosa. Orphan designation had been granted in the United States of America for retinitis pigmentosa.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 May 2013 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:

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

Language	Active ingredient	Indication
English	Unoprostone isopropyl	Treatment of retinitis pigmentosa
Bulgarian	Унопростон изопропил	Лечение на пигментен ретинит
Czech	Isopropylunoproston	Léčba pigmentosní retinitidy
Danish	Unoproston isopropyl	Behandling af retinitis pigmentosa
Dutch	Unoprostone isopropyl	Behandeling van retinitis pigmentosa
Estonian	Unoprostoон-isopropüül	Pigmentoosse vörkkestapöletiku ravi
Finnish	Isopropyliunoprostoni	Verkkokalvorappeuman hoito
French	Isopropyle d'unoprostone	Traitemennt de la rétinite pigmentaire
German	Unoproston-Isopropyl	Behandlung der Retinopathia Pigmentosa
Greek	Ισοπροπυλεστέρας ουνοπροστόνης	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Unoproszton-izopropilát	Retinitis pigmentosa kezelése
Italian	Isopropile di unoprostone	Trattamento della retinite pigmentosa
Latvian	Unoprostona izopropils	Retinitis pigmentosa ārstēšana
Lithuanian	Unoprostono izopropilas	Pigmentinio retinito gydymas
Maltese	Unoprostone isopropyl	Kura tar-retinite pigmentuža
Polish	Unoproston izopropylu	Leczenie retinopatii barwnikowej
Portuguese	Esterisopropílico de unoprostona	Tratamento da retinite pigmentosa
Romanian	Izopropil de unoprostonă	Tratamentul retinitiei pigmentare
Slovak	Izopropyl unoproston	Liečba retinitis pigmentosa
Slovenian	Unoproston izopropil	Zdravljenje pigmentozne retinopatije
Spanish	Unoprostona isopropilo	Tratamiento de retinosis pigmentaria
Swedish	Unoprostonisopropyl	Behandling av retinitis pigmentosa
Norwegian	Unoprostonisopropyl	Behandling av retinitis pigmentosa
Icelandic	Únóprostón ísóprópýl	Meðferð á retinitis pigmentosa

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