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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Patupilone for the treatment of primary peritoneal cancer

First publication	16 November 2009
Rev.1: withdrawal from the Community Register	23 March 2011
Rev.2: sponsor's change of address	5 February 2015
Disclaimer 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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2011 on request of the Sponsor.

On 5 November 2009, orphan designation (EU/3/09/687) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for patupilone for the treatment of primary peritoneal cancer.

What is primary peritoneal cancer?

Primary peritoneal cancer is a rare cancer that starts in the peritoneum. This is the membrane that forms the lining of the abdominal (tummy) cavity. The cells that form the peritoneum ('epithelial cells') are the same as those lining the ovaries, where most of the ovarian cancers start. Therefore, this type of cancer shares some characteristics with ovarian cancer. Primary peritoneal cancer mainly affects women aged between 40 and 60 years.

Primary peritoneal cancer is a severe and life-threatening disease that leads to poor long-term survival.



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

At the time of designation, primary peritoneal cancer affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 people*, and is below the threshold 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 the treatment of primary peritoneal cancer. Because of the similarities between ovarian and primary peritoneal cancer cells, the treatments authorised for ovarian cancer were used also for primary peritoneal cancer.

How is this medicine expected to work?

Patupilone is a substance extracted from bacteria. It is a cytotoxic (a substance that kills cells that are dividing, such as cancer cells). It is expected to work by blocking the cell's ability to break down the 'skeleton' that allows cells to divide and multiply. With the skeleton still in place the cells cannot divide, and they eventually die.

What is the stage of development of this medicine?

The effects of patupilone have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with primary peritoneal cancer were ongoing.

At the time of submission, patupilone was not authorised anywhere in the EU for primary peritoneal cancer or designated as 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 2 September 2009 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.

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

For more information

Sponsor's contact details:

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E-mail: orphan.enquiries@novartis.com

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	Patupilone	Treatment of primary peritoneal cancer
Bulgarian	патупилон	Лечение на първичен перитонеален карцином
Czech	Patupilon	Léčba primárního karcinomu peritonea
Danish	Patupilon	Behandling af primær peritoneal cancer
Dutch	Patupilon	Behandeling van primaire buikvlieskanker
Estonian	Patupiloon	Esmase kõhukelmevähi ravi
Finnish	Patupiloni	Primaarisen vatsakalvosyövän hoito
French	Patupilone	Traitement des tumeurs primitives du péritoine
German	Patupilon	Behandlung des primären Peritonealkarzinoms
Greek	Πατουπιλόνη	Θεραπεία πρωτογενούς καρκίνου του περιτοναίου
Hungarian	Patupilon	Primer peritonealis daganat kezelése
Italian	Patupilone	Trattamento dei tumori peritoneali primitivi
Latvian	Patupilons	Primāra peritoneāla vēža ārstēšanai
Lithuanian	Patupilonas	Pirminio pilvaplėvės vėžio gydymas
Maltese	Patupilone	Kura tal-kanċer primarju tal-peritoneu
Polish	Patupilon	Leczenie pierwotnego raka otrzewnej
Portuguese	Patupilona	Tratamento do carcinoma primário do peritoneu
Romanian	Patupilonă	Tratamentul cancerului peritoneal primitiv
Slovak	Patupilon	Liečba primárnej rakoviny peritonea
Slovenian	patupilon	zdravljenje primarnega raka peritoneja
Spanish	Patupilona	Tratamiento del cáncer peritoneal primario
Swedish	Patupilon	Behandling av primär peritoneal cancer
Norwegian	Patupilon	Behandling av primær peritonealkreft
Icelandic	Patupiíón	Til meðferðar við krabbameini í lífhimnu

¹ At the time of designation