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## Public summary of opinion on orphan designation

### Palovarotene for the treatment of multiple osteochondromas

On 27 June 2018, orphan designation (EU/3/18/2038) was granted by the European Commission to PPD Global Ltd, United Kingdom, for palovarotene for the treatment of multiple osteochondromas.

#### **What is multiple osteochondromas?**

Multiple osteochondromas is an inherited condition in which patients have abnormal non-cancerous growths at the growing points of bones in the arms, legs, ribs, hip and spine. The number of growths and where they occur vary. The growths can damage nerves, blood vessels and other tissue by pressing on them, and they cause pain and restrict movement. Deformities caused by the condition can lead to limbs of uneven lengths and to short stature. In rare cases, the growths can become cancerous.

Multiple osteochondromas is a debilitating disease that is long lasting and may be life threatening because the growths can become cancerous.

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

At the time of designation, multiple osteochondromas affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 16,000 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 for the treatment of multiple osteochondromas. Painkillers were used for treating pain caused by the disease and surgery was used for more severe forms, including procedures to treat uneven leg length or misalignment of the legs.

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\*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 517,400,000 (Eurostat 2018).



## How is this medicine expected to work?

In the body, proteins called bone morphogenetic proteins (BMPs) are involved in bone growth. The activity of BMPs is increased in multiple osteochondromas, which causes the abnormal growth. Palovarotene is expected to reduce the growth of cells that cause osteochondromas by blocking excess BMP activity. This is expected to reduce the sign and symptoms of the condition.

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

The effects of palovarotene have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with multiple osteochondromas was ongoing.

At the time of submission, palovarotene was not authorised anywhere in the EU for multiple osteochondromas. Orphan designation of the medicine had been granted in the United States for multiple osteochondromas.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 24 May 2018 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.

Withdrawn

## Translations of the active ingredient and indication in all official EU languages<sup>1</sup>, Norwegian and Icelandic

Language	Activeingredient	Indication
English	Palovarotene	Treatment of multiple osteochondromas
Bulgarian	Паловаротен	Лечение на множествена остеохондрома
Croatian	Palovaroten	Liječenje multiplih osteohondroma
Czech	Palovaroten	Léčba mnohočetné osteochondromatózy
Danish	Palovaroten	Behandling af multiple osteokondromer
Dutch	Palovarotene	Behandeling van multiple osteochondromen
Estonian	Palovaroteen	Osteokondromatoosi ravi
Finnish	Palovaroteeni	Multipelin osteokondrooman hoito
French	Palovarotène	Traitement de la maladie des exostoses multiples
German	Palovaroten	Behandlung Multipler Osteochondrome
Greek	Παλοβαροτένη	Θεραπεία πολλαπλών οστεοχονδρωμάτων
Hungarian	Palovarotene	Többszörös oszteokondróma kezelése
Italian	Palovarotene	Trattamento degli osteocondromi multipli
Latvian	Palovarotēns	Multiplu osteohondromu ārstēšana
Lithuanian	Palovarotenas	Dauginių osteochondromų gydymas
Maltese	Palovarotene	Trattament ta osteokondromi multipli
Polish	Palowaroten	Leczenie mnogich wyrosli chrzęstno-kostnych
Portuguese	Palovaroteno	Tratamento de osteocondromas múltiplos
Romanian	Palovaroten	Tratamentul osteocondroamelor multiple
Slovak	Palovarotén	Liečba mnohopočetných osteochondrómov
Slovenian	Palovaroten	Zdravljenje multiplih osteohondromov
Spanish	Palovaroteno	Tratamiento de osteocondromas múltiples
Swedish	Palovaroten	Behandling av multipla osteokondrom
Norwegian	Palovaroten	Behandling av multiple osteokondromer
Icelandic	Palóvaróten	Meðferð á bein-og brjóskæxlafjöld

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