10 December 2018
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Public summary of opinion on orphan designation

(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylic acid for the treatment of dermatomyositis

On 26 October 2018, orphan designation (EU/3/18/2070) was granted by the European Commission to Accelsiors CRO and Consultancy Services Ltd, Hungary, for (6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylic acid (also known as lenabasum) for the treatment of dermatomyositis.

What is dermatomyositis?

Dermatomyositis is an inflammatory disease of the muscles and the skin which causes muscle weakness and severe skin rash. Although skeletal muscle and skin problems are the most frequent signs of the disease, inflammation may also affect the muscles of the oesophagus (the food pipe that leads from the mouth to the stomach), the lungs and the heart, leading to difficulties in eating and breathing.

Dermatomyositis is an auto-immune disease. This means that it is caused by the body's immune (defence) system attacking its own tissues. The reason why the immune system acts in this way is not known.

Dermatomyositis is a life-threatening and long-term debilitating condition due to the severe skin problems, muscle weakness and heart problems, and also because patients with the condition are at higher risk of developing cancer.

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

At the time of designation, dermatomyositis affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 67,000 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. This represents a population of 517,400,000 (Eurostat 2018).

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What treatments are available?

At the time of designation, azathioprine and corticosteroid medicines were authorised for dermatomyositis in some EU countries.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with dermatomyositis because early studies showed that it may improve the skin rash in patients who did not respond to other medicines.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

This medicine attaches to receptors (targets) called cannabinoid type 2 receptors found on immune system cells. By attaching to these receptors, it is expected to control the activity of the body's immune system in patients with dermatomyositis, reducing inflammation and so improving symptoms of the 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, clinical trials with the medicine in patients with dermatomyositis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for dermatomyositis 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 13 September 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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylic acid	Treatment of dermatomyositis
Bulgarian	(6aR,10aR)-3-(1,1-диметилхептил)-делта8-тетрахидро-канабинол-9-карбоксилова киселина	Лечение на дерматомиозит
Croatian	(6aR,10aR)-3-(1,1-dimetilheptil)-delta8-tetrahidro-canabinol-9-karboksilna kiselina	Liječenje dermatomiozitisa
Czech	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylic acid	Léčba dermatomyositidy
Danish	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylsyre	Behandling af dermatomyositis
Dutch	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxyliczuur	Behandeling van dermatomyositis
Estonian	(6aR,10aR)-3-(1,1-dimetüülheptüül)-delta8-tetrahüdro-kannabinool-9-karboksüülhape	Dermatomüosiidi i ravi
Finnish	(6aR,10aR)-3-(1,1-dimetyyliheptyyli)-delta8-tetrahydro-kannabinoli-9-karboksyylihappo	Dermatomyosiitin hoito
French	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylacid	Traitement des dermatomyosites
German	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carbonsäure	Behandlung von Dermatomyositis
Greek	(6aR,10aR)-3-(1,1-διμεθυλεπτυλ)-δ8-τετραϋδροκανναβινολ-9-καρβοξυλικό οξύ	Θεραπεία της δερματομυοσίτιδος
Hungarian	(6aR,10aR)-3-(1,1-dimetilheptil)-delta8-tetrahidro-kannabinol-9-karboxilsav	Dermatomyositis kezelése
Italian	(6aR,10aR)-3-(1,1-dimethylheptyl)-delta8-tetrahydro-cannabinol-9-carboxylic acid	Trattamento delle dermatomiositi
Latvian	(6aR,10aR)-3-(1,1-dimetilheptil)-delta8-tetrahidro-kannabinol-9-karbonskābe	Dermatomiozīta ārstēšana
Lithuanian	(6aR,10aR)-3-(1,1-dimetilheptil)-delta8-tetrahidro-kanabinolio-9-karboksilinė rūgštis	Dermatomiozito gydymas
Maltese	(6aR,10aR)-3-(1,1-dimetilħeptil)-delta8-tetraidro-kannabinol-9-aċidu karbossiliku	Kura tal-dermatomijosite
Polish	Kwas (6aR,10aR)-3-(1,1-dimetyloheptylo)-delta8-tetrahydro-kanabinolo-9-karboksylowy	Leczenie zapalenia skórno-mięśniowego
Portuguese	Ácido (6aR,10aR)-3-(1,1-dimetil-heptil)-delta8-tetra-hidro-cannabinol-9-carboxilico	Tratamento da dermatomiosite
Romanian	Acido (6aR,10aR)-3-(1,1-dimetil-eptil)-delta8-tetra-hidro-cannabinol-9-carbossilico	Tratamentul dermatomiozitei
Slovak	(6aR,10aR)-3-(1,1-dimetylheptyl)-delta8-tetrahydro-kanabinol-9-karboxylová kyselina	Liečba dermatomyozitídy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	(6aR,10aR)-3-(1,1-dimetilheptil)-delta8-tetrahidro-kanabinol-9-karboksilna kislina	Zdravljenje dermatomiozitisa
Spanish	Acido (6aR,10aR)-3-(1,1-dimetilheptil) de 9-carboxilico delta8-tetrahidro-canabinol	Tratamiento de la dermatomiositis
Swedish	(6aR,10aR)-3-(1,1-dimetylheptyl)-delta8-tetrahydro-kannabinol-9-karboxylsyra	Behandling av dermatomyosit
Norwegian	(6aR,10aR)-3-(1,1-dimetylheptyl)-delta8-tetrahydro-kannabinol-9-karboksylsyre	Behandling av dermatomyositt
Icelandic	(6aR,10aR)-3-(1,1-dímetýlheptýl)-delta8-tetrahýdró-kannabínól-9-karboxýlsýra	Meðferð við húð- og fjölvöðvabólgu