5 September 2016
EMA/COMP/446373/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Setmelanotide for the treatment of pro-opiomelanocortin deficiency

On 14 July 2016, orphan designation (EU/3/16/1703) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for setmelanotide for the treatment of pro-opiomelanocortin deficiency.

What is pro-opiomelanocortin deficiency?

Pro-opiomelanocortin deficiency is an inherited disease in which patients feel continuously hungry and constantly search for food. This behaviour starts in babies, and patients quickly become obese. The disease is caused by low levels of pro-opiomelanocortin, a substance that is converted into several hormones, including melanocyte-stimulating hormone (MSH) and adrenocorticotropic hormone (ACTH). In patients with this condition, low levels of MSH lead to loss of feeling of fullness after eating whereas low levels of ACTH lead to reduced levels of the stress hormone cortisol and symptoms such as muscle weakness and low blood pressure.

Pro-opiomelanocortin deficiency is a debilitating and life-threatening disease because of its serious symptoms, particularly obesity.

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

At the time of designation, pro-opiomelanocortin deficiency affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 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 the treatment of pro-opiomelanocortin deficiency. Patients were treated with the weight loss medicines orlistat and methylcellulose.

[*]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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

Setmelanotide acts as a replacement for MSH. It attaches to melanocortin receptors to promote a feeling of fullness after eating. This is expected to reduce excessive food intake and obesity.

What is the stage of development of this medicine?

The effects of setmelanotide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with setmelanotide in patients with pro-opiomelanocortin deficiency were ongoing.

At the time of submission, setmelanotide was not authorised anywhere in the EU for pro-opiomelanocortin deficiency. Orphan designation of setmelanotide had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 June 2016 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	Setmelanotide	Treatment of pro-opiomelanocortin deficiency
Bulgarian	Сетмеланотид	Лечение на дефицит на проопиомеланокортин
Croatian	Setmelanotid	Liječenje nedostatka proopiomelanokortina
Czech	Setmelanotid	Léčba nedostatku pro-opiomelanokortinu
Danish	Setmelanotid	Behandling af pro-opiomelanocortin mangel
Dutch	Setmelanotide	Behandeling van pro-opiomelanocortinedeficiëntie
Estonian	Setmelanotiid	Proopiomelanokortiini defitsiidi ravi
Finnish	Setmelanotidi	Pro-opiomelanokortiinivajauksen hoito
French	Setmélanotide	Traitement de la carence en pro-opiomélanocortine
German	Setmelanotide	Behandlung des Proopiomelanocortin-Mangels
Greek	Σετμελανοτίδη	Θεραπεία της ανεπάρκειας προοπιομελανοκορτίνης
Hungarian	Setmelanotide	A proopiomelanokortin-hiány kezelése
Italian	Setmelanotide	Trattamento del deficit di pro-opiomelanocortina
Latvian	Setmelanotīds	Proopiomelanokortīna deficīta ārstēšana
Lithuanian	Setmelanotidas	Proopiomelanokortino stokos gydymas
Maltese	Setmelanotide	Kura ta' nuqqas ta' pro-opjomelanokortin
Polish	Setmelanotyd	Leczenie niedoboru proopiomelanokortyny
Portuguese	Setmelanotido	Tratamento da deficiência em pro-ópio melanocortina
Romanian	Setmelanotidă	Tratamentul deficitului de pro-opiomelanocortină
Slovak	Setmelanotid	Liečba nedostatku pro-opiomelanokortínu
Slovenian	Setmelanotid	Zdravljenje pomanjkanja proopiomelanokortina
Spanish	Setmelanotida	Tratamiento para el déficit de proopiomelanocortina
Swedish	Setmelanotid	Behandling av proopiomelanokortinbrist
Norwegian	Setmelanotid	Behandling for proopiomelanokortin-mangel
Icelandic	Setmelanótíð	Meðferð við skorti á pró-ópíómelanócortíni

[1] At the time of designation