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Public summary of opinion on orphan designation

Setmelanotide for the treatment of leptin receptor deficiency

On 19 November 2018, orphan designation (EU/3/18/2101) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for setmelanotide for the treatment of leptin receptor deficiency.

What is leptin receptor deficiency?

Leptin receptor deficiency is an inherited disease that causes severe obesity. Patients are born at normal weight but they are constantly hungry and quickly put on weight.

The disease is caused by mutations (changes) in the gene responsible for the production of the 'leptin receptor', the target for the hormone leptin. Normally when leptin attaches to the leptin receptor on nerve cells in the brain it stimulates signals to other nerves that make the body feel full and control feelings of hunger. In patients with the condition, the receptor does not work properly, so these signals cannot be sent, leaving the patient always hungry.

Leptin receptor deficiency is a long-term debilitating and life-threatening disease because it leads to severe obesity and related complications.

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

At the time of designation, leptin receptor deficiency affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 treatments were authorised in the EU for leptin receptor deficiency. Patients were managed with medicines for general weight control, or by surgery.

*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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How is this medicine expected to work?

In patients with leptin receptor deficiency a signal cannot be sent to the nerves that control appetite. Setmelanotide is a small molecule that is expected to work by stimulating these nerves directly, bypassing the need for a signal triggered by leptin. This is expected to restore appetite control in patients with leptin receptor deficiency and so reduce their food intake and weight gain.

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 leptin receptor deficiency were ongoing.

At the time of submission, setmelanotide was not authorised anywhere in the EU for leptin receptor deficiency. Orphan designation of the medicine had been granted in the United States for LEPR deficiency obesity.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 October 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	Setmelanotide	Treatment of leptin receptor deficiency
Bulgarian	Сетмеланотид	Лечение на недостатъчност на лептиновия рецептор
Croatian	Setmelanotid	Liječenje deficijencije leptinskog receptora
Czech	Setmelanotid	Léčba deficitu leptinového receptoru
Danish	Setmelanotid	Behandling af leptin-receptor mangel
Dutch	Setmelanotide	Behandeling van leptine receptor deficiëntie
Estonian	Setmelanotiid	Leptiini retseptori vaeguse ravi
Finnish	Setmelanotidi	Leptiinireseptori puutoksen hoito
French	Setmélanotide	Traitement du déficit en récepteur de la leptine
German	Setmelanotide	Behandlung des Leptin-Rezeptor-Mangels
Greek	Σετμελανοτίδη	Θεραπεία της ανεπάρκειας του υποδοχέα της λεπτίνης
Hungarian	Setmelanotide	Leptinreceptor elégtelenség kezelése
Italian	Setmelanotide	Trattamento della deficienza del recettore della leptina
Latvian	Setmelanotīds	Leptīna receptoru deficīta ārstēšana
Lithuanian	Setmelanotidas	Leptino receptoriaus stokos gydymas
Maltese	Setmelanotide	Trattament tan-nuqqas tar-riċettatur ta' leptin
Polish	Setmelanotyd	Leczenie niedoboru receptora leptyny
Portuguese	Setmelanotido	Tratamento da deficiência no receptor de leptina
Romanian	Setmelanotidă	Tratamentul deficitului de receptori de leptină
Slovak	Setmelanotid	Liečba deficitu leptínového receptora
Slovenian	Setmelanotid	Zdravljenje pomanjkanja leptinskih receptorjev
Spanish	Setmelanotida	Tratamiento de la deficiencia del receptor de leptina
Swedish	Setmelanotid	Behandling av leptinreceptorbrist
Norwegian	Setmelanotid	Behandling av leptinreseptor mangel
Icelandic	Setmelanótíð	Meðferð á skorti á leptínviðtaka

[1] At the time of designation