25 July 2016
EMA/COMP/383369/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Setmelanotide for the treatment of Prader-Willi syndrome

On 27 June 2016, orphan designation (EU/3/16/1688) was granted by the European Commission to TMC Pharma Services Ltd, United Kingdom, for setmelanotide for the treatment of Prader-Willi syndrome.

What is Prader-Willi syndrome?

Prader-Willi syndrome is an inherited condition caused by defects in specific regions of chromosome 15. This causes a wide range of symptoms, some of which can appear at birth, such as feeding problems, small size and reduced muscle strength. During childhood further symptoms develop, including increased appetite leading to constant eating and severe obesity, short stature, incomplete sexual development, learning difficulties and behavioural problems, such as aggression and stubbornness.

Prader-Willi syndrome is a life-long debilitating and life-threatening disease because of its serious symptoms, particularly learning difficulties, behavioural problems and obesity.

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

At the time of designation, Prader-Willi syndrome affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 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, growth hormone was authorised in the EU for treating Prader-Willi syndrome. In addition, symptoms were treated or managed in various ways, including supervised access to food to prevent obesity.

*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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

An agency of the European Union

© European Medicines Agency, 2016. Reproduction is authorised provided the source is acknowledged.

The sponsor has provided sufficient information to show that setmelanotide might be of significant benefit for patients with Prader-Willi syndrome because early studies show that it may reduce the excessive food intake associated with this condition, thereby inducing weight loss, a benefit not currently seen with growth hormone therapy. 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?

Setmelanotide has an action similar to a group of hormones called melanocortins which are produced by the nervous system and regulate eating behaviour and how the body processes food. Setmelanotide attaches to melanocortin receptors in the nervous system and is expected to reduce the patients' hunger, and thus their food intake. Additionally, setmelanotide is expected to improve the symptoms of the disease by helping to regulate how fats and glucose (sugar) are used by the body.

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 Prader-Willi syndrome were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 May 2016 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.

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 Prader-Willi syndrome
Bulgarian	Сетмеланотид	Лечение на синдром на Прадер-Вили
Croatian	Setmelanotid	Liječenje Prader-Willijevog sindroma
Czech	Setmelanotid	Léčba Prader-Williho syndromu
Danish	Setmelanotid	Behandling af Prader-Willis syndrom
Dutch	Setmelanotide	Behandeling van Prader-Willi syndroom
Estonian	Setmelanotiid	Prader-Willi sündroomi ravi
Finnish	Setmelanotidi	Prader-Willin oireyhtymän hoito
French	Setmélanotide	Traitement du syndrome de Prader-Willi
German	Setmelanotide	Behandlung des Prader-Willi-Syndroms
Greek	Σετμελανοτίδη	Θεραπεία του συνδρόμου Prader-Willi
Hungarian	Setmelanotide	Prader-Willi szindróma kezelése
Italian	Setmelanotide	Trattamento della sindrome di Prader-Willi
Latvian	Setmelanotīds	Prader-Wili sindroma ārstēšana
Lithuanian	Setmelanotidas	*Prader-Willi* sindromo gydymas
Maltese	Setmelanotide	Kura għal Prader-Willi Syndrome
Polish	Setmelanotyd	Leczenie zespołu Pradera-Williego
Portuguese	Setmelanotida	Tratamento da síndrome de Prader-Willi
Romanian	Setmelanotid	Tratamentul sindromului Prader-Willi
Slovak	Setmelanotid	Liečba Praderovho-Williho syndrómu
Slovenian	Setmelanotid	Zdravljenje Prader-Willijevega sindroma
Spanish	Setmelanotida	Tratamiento del Síndrome de Prader-Willi
Swedish	Setmelanotid	Behandling av Prader Willis syndrom
Norwegian	Setmelanotid	Behandling av Prader-Willis syndrom
Icelandic	Setmelanótíð	Meðhöndlun á Prader-Willi heilkenni

[1] At the time of designation