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EMA/COMP/364093/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Oxytocin for the treatment of Prader-Willi syndrome

On 29 July 2014, orphan designation (EU/3/14/1302) was granted by the European Commission to Maïté Tauber, France, for oxytocin for the treatment of Prader-Willi syndrome.

What is Prader-Willi syndrome?

Prader-Willi syndrome is a genetic condition caused by defects in specific areas 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 absence of the sense of satiety often leading to 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 approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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, patients' symptoms were treated or managed in various ways, including supervised access to food to prevent obesity.

The sponsor has provided sufficient information to show that oxytocin might be of significant benefit for patients with Prader-Willi syndrome because early studies show improvements in the behaviour and

*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 511,100,000 (Eurostat 2014).

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feeding patterns of patients with the condition. 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?

Oxytocin is a hormone mostly associated with childbirth because of its effect in uterine (womb) contractions. Oxytocin is also thought to influence several aspects of behaviour (such as bonding with other people) and to regulate food intake. A lower number of oxytocin-producing cells have been reported in the brain of patients with Prader-Willi syndrome. The medicine is to be used to increase the patients' levels of oxytocin and thereby improve behaviour and feeding in patients with Prader-Willi.

What is the stage of development of this medicine?

The effects of oxytocin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with oxytocin in patients with Prader-Willi syndrome were ongoing.

At the time of submission, oxytocin was not authorised anywhere in the EU for Prader-Willi syndrome 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 12 June 2014 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:

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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	Oxytocin	Treatment of Prader-Willi syndrome
Bulgarian	Окситоцин	Лечение на синдром на Прадер-Вили
Croatian	Oksitocin	Liječenje Prader-Willijevog sindroma
Czech	Oxytocin	Léčba Prader-Williho syndromu
Danish	Oxytocin	Behandling af Prader-Willis syndrom
Dutch	Oxytocine	Behandeling van Prader-Willi syndroom
Estonian	Oksütotsiin	Prader-Willi sündroomi ravi
Finnish	Oksitosiini	Prader-Willin oireyhtymän hoito
French	Ocytocine	Traitement du syndrome de Prader-Willi
German	Oxytocin	Behandlung des Prader-Willi-Syndroms
Greek	Ωκυτοκίνη	Θεραπεία του συνδρόμου Prader-Willi
Hungarian	Oxitocin	Prader-Willi szindróma kezelése
Italian	Ossitocina	Trattamento della sindrome di Prader-Willi
Latvian	Oksitocīns	Prader-Wili sindroma ārstēšana
Lithuanian	Oksitocinas	*Prader-Willi* sindromo gydymas
Maltese	Oxytocin	Kura għal Prader-Willi Syndrome
Polish	Oksytocyna	Leczenie zespołu Pradera-Williego
Portuguese	Ocitocina	Tratamento da síndrome de Prader-Willi
Romanian	Oxitocină	Tratamentul sindromului Prader-Willi
Slovak	Oxytocín	Liečba Praderovho-Williho syndrómu
Slovenian	Oksitocin	Zdravljenje Prader-Willijevega sindroma
Spanish	Oxitocina	Tratamiento del Síndrome de Prader-Willi
Swedish	Oxytocin	Behandling av Prader Willis syndrom
Norwegian	Oksytocin	Behandling av Prader-Willis syndrom
Icelandic	Oxýtósín	Meðhöndlun á Prader-Willi heilkenni

[1] At the time of designation