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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Carbetocin for the treatment of Prader-Willi syndrome

On 21 March 2012, orphan designation (EU/3/12/975) was granted by the European Commission to Ferring Pharmaceuticals A/S, Denmark, for carbetocin for the treatment of Prader-Willi syndrome.

What is Prader-Willi syndrome?

Prader-Willi syndrome is a genetic condition caused by damage to 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 a constant desire to eat food 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 less than 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 101,000 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 carbetocin might be of significant benefit for patients with Prader-Willi syndrome because it works in a different way to the existing treatment and early studies in experimental models show that it may improve the behavioural symptoms of this

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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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?

Carbetocin is similar to oxytocin, a hormone mostly associated with childbirth because of its effect in uterine (womb) contractions but which is also thought to influence several aspects of behaviour. A lack of oxytocin is thought to contribute to the behavioural problems seen in Prader-Willi syndrome. Carbetocin is expected to activate to the oxytocin receptors, and thereby mimic oxytocin's function in patients with Prader-Willi syndrome. This is expected to reduce their behavioural problems.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of carbetocin in experimental models was ongoing.

At the time of submission, no clinical trials with carbetocin in patients with Prader-Willi syndrome had been started.

At the time of submission, carbetocin 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 11 January 2012 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	Carbetocin	Treatment of Prader-Willi syndrome
Bulgarian	карбетоцин	Лечение на синдром на Прадер-Вили
Czech	karbetocin	léčba Prader-Williho syndromu
Danish	Carbetocin	Behandling af Prader-Willis syndrom
Dutch	Carbetocine	Behandeling van Prader-Willi syndroom
Estonian	Karbetotsiin	Prader-Willi sündroomi ravi
Finnish	Karbetosiini	Prader-Willin oireyhtymän hoito
French	Carbétocine	Traitement du syndrome de Prader-Willi
German	Carbetocin	Behandlung des Prader-Willi-Syndroms
Greek	Καρμπετοσίνη	Θεραπεία του συνδρόμου Prader-Willi
Hungarian	Karbetocin	Prader-Willi (PWS) szindróma kezelése
Italian	Carbetocina	Trattamento della sindrome di Prader-Willi
Latvian	karbetocīns	Prader-Wili sindroma ārstēšana
Lithuanian	Karbetocinas	*Prader-Willi* sindromo gydymas
Maltese	Carbetocin	Kura għal Prader-Willi Syndrome
Polish	Karbetocyna	Leczenie zespołu Pradera-Williego
Portuguese	Carbetocina	Tratamento da síndrome de Prader-Willi
Romanian	Carbetocină	Tratamentul sindromului Prader-Willi
Slovak	Karbetocín	Liečba Praderovho-Williho syndrómu
Slovenian	Karbetocin	Zdravljenje Prader-Willijevega sindroma
Spanish	Carbetocina	Tratamiento del Síndrome de Prader-Willi
Swedish	Karbetocin	Behandling av Prader Willis syndrom
Norwegian	karbetocin	Behandling av Prader-Willis syndrom
Icelandic	Carbetócín	Meðhöndlun á Prader-Willi heilkenni

[1] At the time of designation