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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Beloranib for the treatment of Prader-Willi syndrome

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 4 July 2014, orphan designation (EU/3/14/1287) was granted by the European Commission to Dr Ulrich Granzer, Germany, for beloranib for the treatment of Prader-Willi syndrome.

What is Prader-Willi syndrome?

Prader-Willi syndrome is a genetic 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 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 approximately 0.33 people in 10,000 in the European Union (EU). This was equivalent to approximately 17,000 people*, and is below the ceiling for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*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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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 beloranib 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 by a different mechanism to existing anti-obesity treatments. 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?

Beloranib blocks the action of an enzyme in the body called methionine aminopeptidase 2 (MetAP2). In Prader-Willi syndrome patients, beloranib is expected to have an anti-obesity effect by reducing patients' hunger, and thus their food intake, and by affecting the way fats are broken down in the body. The mechanism of action is not well understood. Additionally, blocking MetAP2 may reduce the production of fats and cholesterol and reduce inflammation, which are expected to improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of beloranib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with Prader-Willi syndrome had been completed and further studies were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for Prader-Willi syndrome. Orphan designation 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 14 May 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

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

[1] At the time of designation