12 August 2015
EMA/COMP/422821/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Beloranib for the treatment of craniopharyngioma

On 28 July 2015, orphan designation (EU/3/15/1530) was granted by the European Commission to Dr Ulrich Granzer, Germany, for beloranib for the treatment of craniopharyngioma.

What is craniopharyngioma?

Craniopharyngioma is a benign tumour (a type of non-cancerous growth) that develops at the base of the brain near the pituitary gland (a small gland that produces several hormones). Children between 5 and 10 years of age are most commonly affected. Symptoms depend on the location and size of the tumour. They include headache, nausea and vomiting, which are due to increased pressure inside the skull, stunted growth, due to disruption of normal hormone production by the pituitary gland, and reduced vision caused by damage to the optic nerve. A frequent symptom of craniopharyngioma, or of surgery to treat it, is a constant desire to eat which leads to severe obesity.

Craniopharyngioma is a life-long debilitating disease mainly because it can lead to visual impairment, hormone deficiencies and obesity.

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

At the time of designation, craniopharyngioma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 methods were authorised in the EU for the treatment of craniopharyngioma. Patients were usually treated with surgery to remove the tumour, sometimes followed by radiotherapy (radiation treatment) to remove any tumour cells that might be left behind.

*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 512,900,000 (Eurostat 2015).

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How is this medicine expected to work?

How beloranib works in patients with craniopharyngioma is not fully understood. However, it is known that it blocks the action of an enzyme in the body called methionine aminopeptidase 2 (MetAP2). In craniopharyngioma 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. This expected to improve the obesity-related 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, clinical trials with beloranib in patients with craniopharyngioma were ongoing.

At the time of submission, beloranib was not authorised anywhere in the EU for craniopharyngioma. Orphan designation of beloranib had been granted in the EU and in the USA for the treatment of Prader-Willi syndrome, another condition associated with compulsive eating.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 2015 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 craniopharyngioma
Bulgarian	Белораниб	Лечение на краниофарингеом
Croatian	Beloranib	Liječenje kraniofaringeoma
Czech	Beloranib	Léčba kraniofaryngeomu
Danish	Beloranib	Behandling af kraniefaryngiom
Dutch	Beloranib	Behandeling van craniofaryngioom
Estonian	Beloranib	Kraniofarüngioomi ravi
Finnish	Beloranibi	Kraniofaryngiooman hoito
French	Béloranib	Traitement du craniopharyngiome
German	Beloranib	Behandlung von Kraniopharyngeom
Greek	Μπελορανίμπη	Θεραπεία κρανιοφαρυγγιώματος
Hungarian	Beloranib	Craniopharyngioma kezelése
Italian	Beloranib	Trattamento del craniofaringioma
Latvian	Beloranib	Kraniofaringiomas ārstēšana
Lithuanian	Beloranibas	Kraniofaringiomos gydymas
Maltese	Beloranib	Kura tal-kranjofarinġoma
Polish	Beloranib	Leczenie czaszkogardlaka
Portuguese	Beloranib	Tratamento do craniofaringioma
Romanian	Beloranib	Tratamentul craniofaringiomului
Slovak	Beloranib	Liečba kraniofaryngeómu
Slovenian	Beloranib	Zdravljenje kraniofaringioma
Spanish	Beloranib	Tratamiento del craneofaringioma
Swedish	Beloranib	Behandling av kraniofaryngiom
Norwegian	Beloranib	Behandling av kraniofaryngeom
Icelandic	Belóraníb	Meðferð á æxli frá heiladingulsvef (e. craniopharyngioma)

[1] At the time of designation