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EMA/COMP/168618/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Exenatide for the treatment of idiopathic intracranial hypertension

On 21 March 2016, orphan designation (EU/3/16/1629) was granted by the European Commission to Alan Boyd Consultants Ltd, United Kingdom, for exenatide for the treatment of idiopathic intracranial hypertension.

What is idiopathic intracranial hypertension?

Idiopathic intracranial hypertension is a condition where there is increased pressure in the cerebrospinal fluid (fluid around the brain) with no known cause. Idiopathic intracranial hypertension most often occurs in obese women of childbearing age and is a debilitating condition because symptoms include vision loss, which may be permanent, and severe headaches.

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

At the time of designation, idiopathic intracranial hypertension affected approximately 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 72,000 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 medicines were authorised for the treatment of idiopathic intracranial hypertension. A carbonic anhydrase inhibitor, such as acetazolamide, was used to treat idiopathic intracranial hypertension, although it was not authorised for use in this disease. Surgery to reduce pressure on the brain was sometimes performed. Lumbar punctures (drawing out fluid from the spine) were used to improve symptoms, although effects usually lasted for a few days.

*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).

How is this medicine expected to work?

It is thought that a protein in the body called glucagon-like peptide (GLP-1) reduces the amount of cerebrovascular fluid. Exenatide acts in a similar way to GLP-1 and it is also expected to reduce the amount of cerebrovascular fluid. This is expected to reduce intracranial pressure.

What is the stage of development of this medicine?

The effects of exenatide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with exenatide in patients with idiopathic intracranial hypertension had been started.

At the time of submission, exenatide was authorised in the EU and the United States of America for type-2 diabetes.

At the time of submission, exenatide was not authorised anywhere in the EU for idiopathic intracranial hypertension 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 18 February 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Exenatide	Treatment of idiopathic intracranial hypertension
Bulgarian	Екзенатид	Лечение на идиопатична интракраниална хипертензия
Croatian	Eksenatid	Liječenje idiopatske intrakranijalne hipertenzije
Czech	Exenatid	Léčba idiopatické nitrolební hypertenze
Danish	Exenatid	Behandling af idiopatisk intrakraniell hypertension
Dutch	Exenatide	Behandeling van idiopathische intracraniele hypertensive
Estonian	Eksenatiid	Idiopaatilise koljusisese hüpertensiooni ravi
Finnish	Eksenatidi	Interkraniaalisen idiopaattisen hypertension hoito
French	Exénatide	Traitement de l'hypertension intracrânienne idiopathique
German	Exenatid	Behandlung von idiopathischer intrakranieller Hypertension
Greek	Εξενατίδη	Θεραπεία της ιδιοπαθούς ενδοκρανιακής υπέρτασης
Hungarian	Exenatid	Idiopaattisen intrakraniaalisen hypertension hoito
Italian	Exenatide	Trattamento dell'ipertensione endocranica idiopatica
Latvian	Eksenatīds	Idiopātiskās intrakraniālās hipertensijas ārstēšana
Lithuanian	Eksenatidas	Idiopatinės intrakranialinės hipertenzijos gydymas
Maltese	Exenatide	Kura ta' pressjoni għolja fil-kranju idjopatika
Polish	Eksenatyd	Leczenie idiopatycznego nadciśnienia śródczaszkowego
Portuguese	Exenatido	Tratamento da Hipertensão Intracraniana Idiopática
Romanian	Exenatidă	Tratamentul hipertensiunii intracraniene idiopatice
Slovak	Exenatid	Liečba idiopatickej vnútrolebečnej hypertenzie
Slovenian	Eksenatid	Zdravljenje idiopatske intrakranijalne hipertenzije
Spanish	Exenatida	Tratamiento de la hipertensión intracraneal idiopática
Swedish	Exenatid	Behandling av idiopatisk intrakraniell hypertension
Norwegian	Eksenatid	Behandling av idiopatisk intrakraniell hypertensjon
Icelandic	Exenatíð	Meðferð sjálfvakins innankúpuháþrýstings

¹ At the time of designation