7 November 2016
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Exendin (9-39) for the treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome

On 14 October 2016, orphan designation (EU/3/16/1750) was granted by the European Commission to Eiger Biopharmaceuticals Europe Ltd, United Kingdom, for exendin (9-39) for the treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome.

What is noninsulinoma pancreatogenous hypoglycaemia syndrome?

Noninsulinoma pancreatogenous hypoglycaemia syndrome (NIPHS) is a disorder characterised by excess growth of cells in the pancreas (a small organ behind the stomach) that make insulin. As a result, patients produce too much insulin and are prone to episodes of abnormally low blood sugar (hypoglycaemia). NIPHS can occur as an inborn condition in children due to genetic changes or can develop following weight-loss surgery in adults.

NIPHS is a life-threatening and long-term debilitating condition because repeated episodes of low blood sugar can cause fits, loss of consciousness, heart problems and brain damage.

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

At the time of designation, NIPHS affected approximately 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 190,000 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 orphan designation there was no satisfactory treatment authorised in the EU for NIPHS. Patients were sometimes treated with medicines such as diazoxide that help to reduce insulin secretion or given surgery to reduce the size of the pancreas and so the numbers of insulin-producing cells.

*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).

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How is this medicine expected to work?

Release of insulin from the insulin-producing cells in the pancreas is triggered by a natural hormone, glucagon-like peptide-1 (GLP-1), when it attaches to receptors on these cells. Exendin (9-39) is able to attach to and block the GLP-1 receptors. This prevents GLP-1 from triggering insulin release, resulting in more normal blood sugar levels in patients with NIPHS.

What is the stage of development of this medicine?

The effects of exendin (9-39) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with NIPHS were ongoing.

At the time of submission, exendin (9-39) was not authorised anywhere in the EU for NIPHS. Orphan designation of the medicine had been granted in the United States for treatment of congenital (inborn) hyperinsulinaemic hypoglycaemia and other causes of low blood sugar due to excess insulin secretion in adults and children.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2016 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Exendin (9-39)	Treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome
Bulgarian	Ексендин (9-39)	Лечение на панкреатогенен хипогликемичен синдром, несвързан с инсулином
Croatian	Eksendin (9-39)	Liječenje ne-inzulinomskog pankreatogenog sindroma hipoglikemije
Czech	Exendin (9-39)	Léčba pankreatogenního hypoglykemického syndromu nesouvisejícího s inzulinomem
Danish	Exendin (9-39)	Behandling af ikke insulinom pancreatogenous hypoglykæmi syndrom
Dutch	Exendine (9-39)	Behandeling van niet-insulinoom pancreatogeen hypoglykemiesyndroom
Estonian	Exendiin (9-39)	Mitteinsulinoomse pankreatogeense hüpoglükeemia sündroomi ravi
Finnish	Eksendiini (9-39)	Insulinoomasta johtumattoman haimasyntyisen hypoglykemiaoireyhtymän hoito
French	Exendin (9-39)	Traitement du syndrome d'hypoglycémie pancréatogène sans insulinome
German	Exendin (9-39)	Behandlung von Non-Insulinoma pankreatogenem Hypoglykämie-Syndrom
Greek	Εξενδίνη (9-39)	Θεραπεία του συνδρόμου παγκρεατογενούς υπογλυκαιμίας χωρίς ινσουλίνωμα
Hungarian	Exendin (9-39)	Nem inzulinoma okozta pancreatogen hypoglykaemia szindróma kezelése
Italian	Exendin (9-39)	Trattamento della sindrome ipoglicemica di origine pancreatica non secondaria a insulinoma
Latvian	Eksendīns (9-39)	Neinsulinējama pankreatogēna hipoglikēmijas sindroma ārstēšana
Lithuanian	Eksendinas (9-39)	Ne kasos insulinomų sukeliamo hipoglikemijos sindromo gydymas
Maltese	Exendin (9-39)	Kura tas-sindrome ta' ipogliċemija ta' origini pankreatika mhux marbuta mal-insulinoma
Polish	Eksendyna (9-39)	Leczenie zespołu hipoglikemii pochodzenia trzustkowego, niezwiązanej z występowaniem guza insulinowego
Portuguese	Exendina (9-39)	Tratamento da síndrome de hipoglicemia pancreatogénica não insulinoma
Romanian	Exendină (9-39)	Tratamentul sindromului de hipoglicemie pancreatogenă non-insulinomică
Slovak	Exendin (9-39)	Liečba pankreatogenného hypoglykemického syndrómu nesúvisiaceho s inzulinómom
Slovenian	Eksendin (9-39)	Zdravljenje sindroma neinsulinomske pankreatogene hipoglikemije
Spanish	Exendina (9-39)	Tratamiento del síndrome de hipoglucemia pancreatógena no

[1] At the time of designation

Language	Active ingredient	Indication
		insulinoma
Swedish	Exendin (9-39)	Behandling av icke-insulinom pankreatogen hypoglykemi
Norwegian	Eksendin (9-39)	Behandling av ikke-insulinom pankreatogent hypoglykemisyndrom
Icelandic	Exendin (9-39)	Meðhöndlun á blóðsykurslækkunar heilkenni af völdum brisæxlis sem ekki er insúlínóma