

15 January 2019  
EMA/769575/2018

## Public summary of opinion on orphan designation

### Glucagon for the treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome

On 19 November 2018, orphan designation (EU/3/18/2091) was granted by the European Commission to Pharma Gateway AB, Sweden, for glucagon 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 with this syndrome 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 191,000 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, there were no treatments authorised in the EU for NIPHS. Patients often underwent surgery to remove some of the pancreas. Products such as diazoxide and octreotide were used to reduce insulin secretion. However, these medicines were not authorised for use in the condition.

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\*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 517,400,000 (Eurostat 2018).

## How is this medicine expected to work?

The medicine is a synthetic form of the hormone glucagon, which is naturally produced by the body. It acts like natural glucagon and releases glucose from the liver, which increases blood glucose levels thereby reducing the symptoms of the disease.

## What is the stage of development of this medicine?

The effects of glucagon 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, glucagon injections were authorised for use in emergency situations to treat episodes of hypoglycaemia in diabetes when the patient is unconscious.

At the time of submission, the medicine was not authorised anywhere in the EU for NIPHS 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 October 2018 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Glucagon	Treatment of noninsulinoma pancreatogenous hypoglycaemia syndrome
Bulgarian	Глюкагон	Лечение на панкреатогенен хипогликемичен синдром, несвързан с инсулином
Croatian	Glukagon	Liječenje ne-inzulinomskog pankreatogenog sindroma hipoglikemije
Czech	Glukagon	Léčba pankreatogenního hypoglykemického syndromu nesouvisejícího s inzulinomem
Danish	Glukagon	Behandling af ikke insulinomt pancreatisk hypoglykæmi syndrom
Dutch	Glucagon	Behandeling van niet-insulinoom pancreatogeen hypoglykemiesyndroom
Estonian	Glükagoon	Mitteinsulinoomse pankreatogeense hüpoglükeemia sündroomi ravi
Finnish	Glukagoni	Insulinoomasta johtumattoman haimasyntyisen hypoglykemiaoireyhtymän hoito
French	Glucagon	Traitement du syndrome d'hypoglycémie pancréatogène sans insulinome
German	Glukagon	Behandlung von Non-Insulinoma pankreatogenem Hypoglykämie- Syndrom
Greek	Γλυκαγόνη	Θεραπεία του συνδρόμου παγκρεατογενούς υπογλυκαιμίας χωρίς ινσουλίνωμα
Hungarian	Glukagon	Nem inzulinoma okozta pancreatogen hypoglykaemia szindróma kezelése
Italian	Glucagone	Trattamento della sindrome ipoglicemica di origine pancreatica non secondaria a insulinoma
Latvian	Glikagons	Neinsulinējama pankreatogēna hipoglikēmijas sindroma ārstēšana
Lithuanian	Gliukagonas	Ne insulinomos sąlygotu, pankreatogeninės hipoglikemijos sindromo, gydymas
Maltese	Glucagon	Trattament tas-sindrome ta' ipoglicemija ta' origini pankreatika mhux marbuta mal-insulinoma
Polish	Glukagon	Leczenie zespołu hipoglikemii pochodzenia trzustkowego, niezwiązanej z występowaniem guza insulinowego
Portuguese	Glucagon	Tratamento da síndrome de hipoglicemia pancreatogénica não insulinoma
Romanian	Glucagon	Tratamentul sindromului de hipoglicemie pancreatogenă non-insulinomică
Slovak	Glukagón	Liečba pankreatogenného hypoglykemického syndrómu nesúvisiaceho s inzulinómom

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovenian	glukagon	Zdravljenje sindroma neinsulinomske pankreatogene hipoglikemije
Spanish	Glucagón	Tratamiento del síndrome de hipoglucemia pancreatogena no insulinoma
Swedish	Glukagon	Behandling av icke-insulinom pankreatogen hypoglykemi
Norwegian	Glukagon	Behandling av ikke-insulinom pankreatogent hypoglykemisyndrom
Icelandic	Glúkagon	Meðhöndlun á blóðsykurslækkunar heilkenni af völdum brisæxlis sem ekki er insúlínóma