4 April 2012
EMA/COMP/61086/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Glucagon for the treatment of congenital hyperinsulinism

On 5 March 2012, orphan designation (EU/3/12/960) was granted by the European Commission to Biodel UK Limited, United Kingdom, for glucagon for the treatment of congenital hyperinsulinism.

What is congenital hyperinsulinism?

Congenital hyperinsulinism is an inherited disorder caused by high levels of insulin, a hormone that helps control blood glucose (sugar) levels. Insulin lowers blood glucose levels by driving glucose into the cells of the body. In hyperinsulinism, more insulin is produced than is needed which results in hypoglycaemia (low blood glucose levels). The severity of congenital hyperinsulinism varies among patients, with some patients already developing episodes of hypoglycaemia shortly after birth. Repeated episodes of hypoglycaemia increase the risk of serious complications such as seizures (fits), mental disability, breathing difficulties and coma.

Congenital hyperinsulinism is a long-term debilitating condition because of the effects of long-term hypoglycaemia on the brain, such as mental disability and seizures.

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

At the time of designation, congenital hyperinsulinism affected approximately 2 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 101,000 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 satisfactory methods of treatment in the EU for congenital hyperinsulinism. Products such as diazoxide and octreotide were used to reduce insulin secretion, and glucagon injections were used in emergency situations to release glucose from the liver and thereby increase blood glucose levels. However, these medicines were not authorised for use in the condition.

[*]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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

How is this medicine expected to work?

Glucagon is a hormone naturally secreted by the pancreas that counteracts the effects of insulin by raising blood glucose levels. This medicine is expected to be given to patients as a continuous infusion under the skin to help prevent hypoglycaemic episodes.

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, no clinical trials with this medicine in patients with congenital hyperinsulinism had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for congenital hyperinsulinism 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 January 2012 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:

Biodel UK Limited
41, Chalton Street
London NW1 1JD
United Kingdom
Telephone: +44 1 203 796 5014
Telefax: +44 1 203 796 5003
E-mail: MTurner@OCTAGONRESEARCH.com

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 substance	Indication
English	Glucagon	Treatment of congenital hyperinsulinism
Bulgarian	Глюкагон	Лечение на вроден хиперинсулинизъм
Czech	Glukagon	Léčba kongenitálního hyperinzulinismu
Danish	Glukagon	Behandling af kongenit hyperinsulinisme
Dutch	Glucagon	Behandeling van congenitaal hyperinsulinisme
Estonian	Glükagoon	Kaasasündinud hüperinsulinismi ravi
Finnish	Glukagoni	Synnynnäisen hyperinsulinismin hoito
French	Glucagon	Traitement de l'hyperinsulinisme congénital
German	Glukagon	Behandlung des kongenitalen Hyperinsulinismus
Greek	Γλυκαγόνη	Θεραπεία του συγγενούς υπερινσουλινισμού
Hungarian	Glukagon	Congenitalis hyperinsulinismus kezelése
Italian	Glucagone	Trattamento dell' iperinsulinemia congenita
Latvian	Glikagons	Iedzimtas hiperinsulinēmijas ārstēšana
Lithuanian	Gliukagonas	Įgimto hiperinsulinizmo gydymas
Maltese	Glucagon	Kura ta' iperinsulinimja konġenitali
Polish	Glukagon	Leczenie wrodzonego hiperinsulinizmu
Portuguese	Glucagon	Tratamento do hiperinsulinismo congénito
Romanian	Glucagon	Tratamentul hiperinsulinismului congenital
Slovak	Glukagón	Liečba kongenitálneho hyperinzulinizmu
Slovenian	glukagon	zdravljenje prirojenega hiperinzulinizma
Spanish	Glucagón	Tratamiento del hiperinsulinismo congénito
Swedish	Glukagon	Behandling av medfödd hyperinsulinism
Norwegian	Glukagon	Behandling av medfødt hyperinsulinisme
Icelandic	Glúkagon	Meðferð á meðfæddu insúlínóhófi

[1] At the time of designation