10 November 2014
EMA/COMP/546576/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Glucagon for the treatment of congenital hyperinsulinism

On 15 October 2014, orphan designation (EU/3/14/1342) was granted by the European Commission to S-cubed 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 and life-threatening disease since glucose is essential for brain cells to function, and severely low blood sugar levels can cause permanent brain injury or death.

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

At the time of designation, congenital hyperinsulinism affected not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 102,000 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 of treatment were authorised in the EU for congenital hyperinsulinism. The main treatment consisted of infusions of glucose to maintain normal glucose levels. Medicines such as diazoxide and octreotide were used to reduce insulin secretion, and glucagon injections were used to release glucose from the liver and thereby increase blood glucose

*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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

levels. However, existing glucagon formulations could not be used regularly over long periods because of poor stability.

How is this medicine expected to work?

This medicine is a synthetic form of glucagon, a hormone naturally secreted by the pancreas that counteracts the effects of insulin by raising blood glucose levels. It is expected to be given under the skin using a special infusion pump. This is expected to help increase blood sugar levels, thereby improving the symptoms of the disease. The way the medicine is formulated is expected to make it suitable to be given regularly for long periods.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of glucagon in experimental models was ongoing.

At the time of submission, clinical trials with glucagon in patients with congenital hyperinsulinism had not started.

At the time of submission, glucagon was authorised in the EU and the United States for the treatment of severe hypoglycaemia.

At the time of submission, glucagon was not authorised anywhere in the EU for the treatment of congenital hyperinsulinism. Orphan designation for glucagon in the treatment of congenital hyperinsulinism had been previously granted in the EU.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2014 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:

S-cubed Limited
99 Park Drive
Milton Park
Abingdon
Oxfordshire OX14 4RY
United Kingdom
Tel. +44 (0)1235 854 055
Fax +44 (0)1235 854 001
E-mail: info@s-cubed.co.uk

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	Glucagon	Treatment of congenital hyperinsulinism
Bulgarian	Глюкагон	Лечение на вроден хиперинсулинизъм
Croatian	Glukagon	Liječenje prirođene hiperinzulinemije
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