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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

[gly2]-recombinant human glucagon-like peptide for the treatment of short bowel syndrome

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 11 December 2001, orphan designation (EU/3/01/077) was granted by the European Commission to Pharm Research Associates (UK) Ltd, United Kingdom, for [gly2]-recombinant human glucagon-like peptide for the treatment of short bowel syndrome.

The sponsorship was transferred to PAREXEL International Limited, United Kingdom in November 2002, to Nycomed Danmark ApS, Denmark in April 2008 and subsequently to NPS Pharma Holdings Limited, Ireland, in October 2013.

What is short bowel syndrome?

Some diseases of the gastrointestinal tract (the stomach and the intestines) have to be treated through the surgical removal of a part of the small intestines. In many patients this procedure leads to subsequent short bowel syndrome. The small intestine (the bowels) is the organ that food passes through from the stomach and it is where it is degraded (mainly in the first part, the jejunum) and absorbed through the walls of the intestines (mainly in the lower part, the ileum). Depending on which part of the bowels is removed, different symptoms may occur, such as diarrhoea, dehydration, malabsorption leading to progressive malnutrition associated with fatty acid and vitamin deficiencies, anaemia (blood deficiency), and weight loss. In children, this is typically accompanied by delayed growth. Short bowel syndrome is chronically debilitating.

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

At the time of designation, short bowel syndrome affected not more than 0.35 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 13,000 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?

There were no authorised medicines for the treatment of short bowel syndrome in the Community at the time of submission of application for orphan drug designation. Many patients with the syndrome are treated with total or supplemental parenteral nutrition (receiving food intravenously).

How is this medicine expected to work?

[gly2]-recombinant human glucagon-like peptide is a protein that is structurally similar to a natural protein called glucagon-like peptide 2 which is produced by cells in the lower part of the intestines. This substance acts as a signalling molecule and when it binds to its receptors it can stimulate various reactions and processes in the tissue. One of the effects is thought to be an increase in proliferation and decrease in cell death as well as improving the absorptive qualities of the small intestine. Thus, [gly2]-recombinant human glucagon-like peptide is expected to improve the function of the remaining part of the intestine.

What is the stage of development of this medicine?

The effects of [gly2]-recombinant human glucagon-like peptide were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with short bowel syndrome were ongoing.

[gly2]-recombinant human glucagon-like peptide was not authorised anywhere worldwide for short bowel syndrome, at the time of submission.

Orphan designation of [gly2]-recombinant human glucagon-like peptide was granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 26 October 2001 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 375,500,000 (Eurostat 2000).

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:

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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	[gly²] Recombinant human glucagon-like peptide	Treatment of Short Bowel Syndrome
Bulgarian	[gly2]-рекомбинантен човешки глюкагон-подобен пептид	Лечение на синдром на късото черво
Croatian	[gly2]-rekombinantni ljudski peptid sličan glukagonu	Liječenje sindroma kratkog crijeva
Czech	[gly2]-rekombinantní humánní glucagonu-podobný peptid	Léčba syndromu krátkého střeva
Danish	[gly²] Rekombinant humant glucagon-lignende peptid	Behandling af korttarmssyndrom
Dutch	[gly²] Recombinant humaan glucagonachtig peptide	Behandeling van kortedarm syndroom
Estonian	[gly2]-rekombinantne inimese glükagoonisarnane peptiid	Lühikese soole sündroomi ravi
Finnish	[gly²] Rekombinantti ihmisen glukagonin kaltainen peptidi	Lyhytsuolioireyhtymän hoito
French	*[gly²] Glucagon-like peptide humain recombinant*	Traitement du syndrome de l'intestin court
German	[gly²] Rekombinantes humanes glukagonähnliches Peptid	Behandlung des Kurzdarmsyndroms bei Erwachsenen und Kindern
Greek	[gly²] Ανασυνδυασμένο ανθρώπινο τύπου γλυκαγόνης πεπτίδιο	Θεραπεία του συνδρόμου βραχείας ελικας σε ενήλικες και παιδιά
Hungarian	[gly2]-rekombináns humán glukagonszerű peptid	Rövid bél szindróma kezelése
Italian	[gly²] Peptide glucagone-simile ricombinante umano	Trattamento della sindrome dell'intestino breve
Latvian	[gly²]-rekombinantais cilvēka glikagonam līdzīgais peptīds	Īsās zarnas sindroma ārstēšana
Lithuanian	[gly2]-rekombinantinis žmogaus į gliukagoną panašus peptidas	Trumposios žarnos sindromui gydyti
Maltese	[gly2]-peptide bħal *glucagon* uman rikombinanti	Kura tas-sindromu tal-musrana qasira
Polish	Rekombinowany ludzki peptyd glukagono-podobny [gly2]	Leczenie zespołu krótkiego jelita
Portuguese	[gly²] Péptido tipo glucagon humano recombinante	Tratamento do síndroma do intestino curto
Romanian	Peptidă umană recombinantă -[gly2] de tip glucagon	Tratamentul sindromului de intestin scurt
Slovak	[gly²]-rekombinantný ľudský glukagónu podobný peptid	Liečba syndrómu krátkeho čreva

[1] At the time of designation

Language	Active Ingredient	Indication
Slovenian	[gli2]-rekombinantni humani glukagonu podoben peptid	Zdravljenje sindroma kratkega črevesja
Spanish	[gly²] Péptido glucagonoide humano recombinante	Tratamiento del síndrome de intestino corto
Swedish	[gly²] Rekombinant humant glukagonliknande peptid	Behandling av korttarmssyndrom