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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148 for the prevention of necrotizing enterocolitis

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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 31 October 2006, orphan designation (EU/3/06/407) was granted by the European Commission to Dr Michael Moore, United Kingdom, for heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148 for the prevention of necrotizing enterocolitis.

What is necrotizing enterocolitis?

Necrotizing enterocolitis is a disease that affects preterm infants. The gut and the immune system (the body's natural defence system) of these very low weight infants are immature and not as well developed as full term infants', increasing the risk of infection during the first few weeks after birth. "Necrotizing enterocolitis" means that portions of the gut (both small and large intestine) may undergo tissue death due to the infection. The symptoms may include problems with feeding, abdominal distension, and bloody stools. If the disease progresses, it can lead to peritonitis and sepsis (infection of the abdominal cavity and infection of the blood). These complications are life-threatening and due to the risk of long-term disabilities, necrotizing enterocolitis may also be chronically debilitating.

What is the estimated number of patients at risk of developing the condition>?

At the time of designation, the number of patients at risk of necrotizing enterocolitis was estimated to be approximately 1.2 people in 10,000 in the European Union (EU). This was equivalent to a total of

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around 56,000 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 methods of prevention are available?

The condition is treated with surgery if possible (removal of the damaged intestinal tissue). There were, however, no products authorised for the prevention of necrotizing enterocolitis in the Community at the time of submission of application for orphan drug designation.

How is this medicine expected to work?

Heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148 is a part of protein naturally occurring in the amniotic fluid (the fluid that the baby is surrounded by in the womb). This protein is known to stimulate growth and development of epithelial cells, which include the cells that line the inside of the intestines. According to the sponsor, heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148 might thus help prevent necrotizing enterocolitis in low birth weight preterm infants.

What is the stage of development of this medicine?

The evaluation of the effects of heparin-binding epidermal growth factor-like growth factor (HB EGF), amino acids 74-148 in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients at risk of being affected by necrotizing enterocolitis were initiated.

Heparin-binding epidermal growth factor-like growth factor (HB EGF), amino acids 74-148 was not authorised anywhere worldwide for the prevention of necrotizing enterocolitis, at the time of submission.

Orphan designation of heparin-binding epidermal growth factor-like growth factor (HB EGF), amino acids 74-148 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 6 September 2006 recommending the granting of this designation.

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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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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

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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	Heparin-binding epidermal growth factor-like growth factor (HB-EGF), amino acids 74-148	Prevention of necrotizing enterocolitis
Czech	Heparin vázající růstový faktor podobný epidermálnímu růstovému faktoru (HB-ECF), aminokyseliny 74-148	Prevence nekrotizující enterokolitidy
Danish	Heparinbindende epidermisk vækstfaktor-lignende vækstfaktor (HB-EGF), aminosyrer 74-148	Forebyggelse af nekrotiserende enterocolitis
Dutch	Heparine-bindende epidermale groeifactorachtige groeifactor (HB-EGF), aminozuren 74-148	Preventie van necrotiserende enterocolitis
Estonian	Hepariini siduva epidermaalse kasvufaktori taoline kasvufaktor(HB-EGF) , aminohapped 74-148	Nekrotiseeriva enterokoliidi ennetamine.
Finnish	Hepariinia sitovan ihon kasvutekijän kaltainen kasvutekijä (HB-EGF), aminohapot 74-148	Nekrotisoivan suolitulehduksen ehkäisy
French	Facteur de croissance analogue du facteur de croissance épidermique lié à l'héparine , acides aminés 74-148	Prévention de l'entérocolite nécrosante
German	Heparinbindender EGF-ähnlicher Wachstumsfaktor (HB-EGF), Aminosäuren 74-148	Verhinderung von nekrotisierender Enterokolitis
Greek	Επιδερμικός παράγοντας - τύπου παράγοντα ανάπτυξης με δέσμευση ηπαρίνης, (HB-EGF), αμινοξέα 74-148	Πρόληψη νεκρωτικής εντεροκολίτιδας
Hungarian	Heparinkötő epidermális növekedési faktor szerű növekedési faktor (HB-EGF), 74-148 aminosavak	Nekrotizáló enterocolitis megelőzése
Italian	Fattore di crescita legante eparina simil-fattore di crescita epidermico, aminoacidi 74-148	Prevenzione dell'enterocolite
Latvian	Heparīnu saistošs epidermālo šūnu augšanas faktoram līdzīgais augšanas faktors (HB-EGF), aminoskābes 74-148	Nekrotizējošā enterokolīta profilakse
Lithuanian	74–148 aminorūgštys, į augimo faktorių panašaus hepariną surišančio epidermio augimo faktoriaus (HB-EGF),	Nekrozuojančio enterokolito prevencija
Polish	naskórkowy czynnik wzrostu, przypominający czynnik wzrostu (HB-EGF) wiążący heparynę, aminokwasy 74-148	Zapobieganie martwiczemu zapaleniu jelit

[1] At the time of designation

Language	Active Ingredient	Indication
Portuguese	Factor de crescimento semelhante ao factor de crescimento epidérmico ligado a heparina (HB-EGF), aminoácidos 74-148	Prevenção de enterocolite necrosante
Slovak	Heparín viažuci epidermálny rastový faktor (HB-EGF) podobný rastovému faktoru, aminokyseliny 74-148	Prevencia nekrotizujúcej enterokolitídy
Slovenian	Heparin vezoči rastnemu faktorju podoben epidermalni rastni faktor, aminokisline 74-148	Preprečevanje nekrotizirajočega enterokolitisa
Spanish	Factor de crecimiento epidérmico de unión a heparina semejante al factor de crecimiento (HB-EGF), aminoácidos 74-148	Prevención de la enterocolitis necrotizante
Swedish	Heparinbindande epidermal tillväxtfaktorliknande tillväxtfaktor (HB-EGF), aminosyror 74-148	Förebyggande av nekrotisk enterokolit
Norwegian	Heparinbindende epidermisk vekstfaktorlignende vekstfaktor (HB-EGF), aminosyrene 74 – 148	Forebygging av nekrotiserende enterokolitt
Icelandic	Heparínbindandi húðþekju vaxtarþáttar-líkur vaxtarþáttur (HB-EGF), amínósýrur 74-148	Vörn gegn garna - og ristilbólgudrepi