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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant microbial lipase for the treatment of malabsorption due to exocrine pancreatic enzyme insufficiency

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in June 2012 on request of the Sponsor.

On 28 October 2005, orphan designation (EU/3/05/331) was granted by the European Commission to Solvay Pharmaceuticals GmbH, Germany, for recombinant microbial lipase for the treatment of malabsorption due to exocrine pancreatic enzyme insufficiency.

Solvay Pharmaceuticals GmbH changed name to Abbott Products GmbH in November 2010.

What is malabsorption due to exocrine pancreatic enzyme insufficiency?

The pancreas is a small organ that lies behind the stomach and in front of the spine. The pancreas has two main functions in the body. It makes hormones, such as insulin, that help to control blood sugar levels (that is called endocrine activity). It also makes a juice that helps to digest (break down) food (this is called exocrine activity). The juice contains enzymes, which are proteins that speed up the transformation of certain substances (such as particles of food) into other substances.

Certain conditions affecting the pancreas, including cystic fibrosis, inflammation of the pancreas (pancreatitis), cancer of the pancreas and an inherited disease called Shwachman-Diamond syndrome, may interfere with its exocrine activity by reducing the synthesis and the release of pancreatic enzymes. This may result in a defective absorption (malabsorption) of nutrients. Typically, the patient experiences bloating and pain in the abdominal area and loose, frequent stools. Malabsorption due to exocrine pancreatic enzyme insufficiency is a severe chronically debilitating condition.

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

At the time of designation, malabsorption due to exocrine pancreatic enzyme insufficiency affected approximately 3.8 in 10,000 people in the European Union (EU). This was equivalent to a total of

around 177,000 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?

Several pancreatic enzyme extracts were authorised for treatment of the condition at the time of application. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product might be of potential significant benefit for the treatment of malabsorption due to exocrine pancreatic insufficiency because it may reduce the amounts of drug substance needed by the patients and thus improve the patient's ability to follow the required intake. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

The proposed medicinal product contains an artificially produced lipase, an enzyme that breaks down fat. By administration of this product it is expected that it will substitute the lacking pancreatic enzyme and improve the digestion.

What is the stage of development of this medicine?

The evaluation of the effects of recombinant microbial lipase in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with malabsorption due to exocrine pancreatic enzyme insufficiency were initiated.

Recombinant microbial lipase was not authorised anywhere worldwide for the treatment of malabsorption due to exocrine pancreatic enzyme insufficiency or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 2005 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 (EU 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant microbial lipase	Treatment of malabsorption due to exocrine pancreatic enzyme insufficiency
Czech	rekombinantní mikrobiální lipáza	Léčba malabsorpce při exokrinní insuficienci pankreatu
Danish	Rekombinant mikrobiel lipase	Behandling af malabsorption p.g.a eksokrin pancreas enzyminsufficiens
Dutch	Recombinant microbiëel lipase	Behandeling van malabsorptie bij exocriene pancreasenzyminsufficiëntie.
Estonian	Rekombinantne mikrobiaalne lipaas	Pankrease eksokriinse ensüümpuudulikkusest tingitud malabsorptsiooni ravi
Finnish	Rekombinantti mikrobilipaasi	Eksokriinisesta haiman vajaatoiminnasta johtuvan malabsorption hoito
French	Lipase microbienne recombinante	Traitement de la malabsorption due à une insuffisance pancréatique exocrine
German	Rekombinante mikrobielle Lipase	Behandlung exokriner Pankreasinsuffizienz bedingter Malabsorption
Greek	Ανασυνδυασμένη μικροβιακή λιπάση	Θεραπεία της δυσασπορρόφησης λόγω παγκρεατικής ενζυμικής ανεπάρκειας
Hungarian	Rekombináns bakteriális lipáz	Exokrin pancreas enzim elégtelenség következtében fellépő felszívódási zavar kezelése
Italian	Lipasi microbica ricombinante	Trattamento del malassorbimento dovuto all'insufficienza pancreatica enzimatica esocrina
Latvian	Rekombinantā mikrobiālā lipāze	Aizkuģa dziedzera eksokrīnā enzīma nepietiekamības izraisītas malabsorbcijas ārstēšana
Lithuanian	Rekombinantinė mikrobinė lipazė	Malabsorbcijos dėl egzokrininių kasos fermentų nepakankamumo gydymas
Polish	Rekombinowana lipaza mikrobiologiczna	Leczenie złego wchłaniania spowodowanego zewnątrzwydzielniczą niewydolnością trzustki
Portuguese	Lipase microbiana recombinante	Tratamento da malabsorção por insuficiência enzimática do pâncreas exócrino
Slovak	Rekombinantná mikrobiálna lipáza	Liečba malabsorbcie pre exokrinnú insuficienciu pankreatických enzýmov
Slovenian	Rekombinantna mikrobna lipaza	Zdravljenje malabsorpcije zaradi eksokrine encimske insuficiene trebušne slinavke
Spanish	Lipasa microbiana recombinante	Tratamiento de la malabsorción secundaria a insuficiencia enzimática pancreática exocrina
Swedish	Rekombinant mikrobiellt lipas	Behandling av malabsorption beroende på bristande funktion av enzymer från exokrina pankreas

¹ At the time of designation

Language	Active Ingredient	Indication
Norwegian	Rekombinant mikrobiell lipase	Behandling av malabsorpsjon forårsaket av eksokrin pankreasenzym insuffisiens
Icelandic	Raðbrigða örveru lípasi	Meðferð við vanfrásogi vegna skorts á útkirtilsensímum briss