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EMA/COMP/443087/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ulinastatin for the treatment of acute pancreatitis

On 22 August 2014, orphan designation (EU/3/14/1318) was granted by the European Commission to BSV BioScience GmbH, Germany, for ulinastatin for the treatment of acute pancreatitis.

What is acute pancreatitis?

Acute pancreatitis is a disease characterised by sudden, painful inflammation of the pancreas, an organ of the digestive system. The pancreas produces various enzymes in an inactive form, which are secreted into the gut where they become active and help break down components of the diet, especially proteins. In acute pancreatitis, some of these enzymes become active in the pancreas itself and start to break down the tissues of the pancreas and surrounding organs, causing inflammation and pain and killing the cells. Complications may include infection, internal bleeding and organ failure. Pancreatitis can have various causes but is often triggered by gallstone disease or excess alcohol consumption.

Acute pancreatitis is potentially life threatening due to complications such as internal bleeding and organ failure.

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

At the time of designation, acute pancreatitis affected approximately 4.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 230,000 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 were authorised in the EU for the treatment of acute pancreatitis. Patients were generally managed with supportive care, including fluid replacement, oxygen, feeding via a tube or into a vein, antibiotics and painkillers. Severely ill patients with organ failure needed to be managed in intensive-care units.

*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).

How is this medicine expected to work?

The active substance in this medicine, ulinastatin, is a protease inhibitor, a substance which blocks the actions of enzymes that break down protein. By blocking the effect of these enzymes in the pancreas it is expected to reduce the tissue damage and inflammation of acute pancreatitis, relieving the symptoms of the condition and reducing its complications.

What is the stage of development of this medicine?

The effects of ulinastatin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with ulinastatin in patients with acute pancreatitis had been completed.

Ulinastatin was authorised in China, India, Japan and South Korea for acute pancreatitis. Ulinastatin was not authorised anywhere in the EU for acute pancreatitis 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 10 July 2014 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:

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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	Ulinastatin	Treatment of acute pancreatitis
Bulgarian	Улинастатин	лечение на остър панкреатит
Croatian	Ulinastatin	Liječenje akutnog pankreatitisa
Czech	Ulinastatin	Léčba akutní pankreatitidy
Danish	Ulinastatin	Behandling af akut pancreatitis
Dutch	Ulinastatine	Behandeling van acute pancreatitis
Estonian	Ulinastatiin	Akuutse pankreatiidi ravi
Finnish	Ulinastatiini	Akuutin haimatulehduksen hoito
French	Ulinastatine	Traitement de la pancréatite aiguë
German	Ulinastatin	Behandlung der akuten Pankreatitis
Greek	Ουλιναστατίνη	θεραπεία της οξείας παγκρεατίτιδας
Hungarian	Ulinastatin	Acut pancreatitis kezelése
Italian	Ulinastatina	Trattamento della pancreatite acuta
Latvian	Ulinastatīns	Akūta pankreatīta ārstēšana
Lithuanian	Ulinastatinas	Ūmaus pankreatito gydymas
Maltese	Ulinastatin	Kura tal-pankreatite akuta
Polish	Ulinastatyna	Leczenie ostrego zapalenia trzustki
Portuguese	Ulinastatina	Tratamento da pancreatite aguda
Romanian	Ulinastatin	Treatment of acute pancreatitis
Slovak	Ulinastatín	Liečba akútnej pankreatitídy
Slovenian	Ulinastatin	Zdravljenje akutnega pankreatitisa
Spanish	Ulinastatina	Tratamiento de la pancreatitis aguda
Swedish	Ulinastatin	Behandling av akut pankreatit
Norwegian	Ulinastatin	Behandling av akutt pankreatitt
Icelandic	Úlínastatín	Meðferð bráðrar briskirtilsbólgu

¹ At the time of designation