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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lomitapide for the treatment of familial chylomicronaemia

First publication	18 January 2011
Rev.1: transfer of sponsorship	16 March 2012
Rev.2: sponsor's change of address	12 June 2013
Rev.3: transfer of sponsorship	12 March 2015
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 17 December 2010, orphan designation (EU/3/10/823) was granted by the European Commission to Dimensione Ricerca S.r.l., Italy, for lomitapide for the treatment of familial chylomicronaemia.

The sponsorship was transferred to Aegerion Pharmaceuticals, France, in February 2012 and subsequently to Aegerion Pharmaceuticals Limited, United Kingdom, in February 2015.

What is familial chylomicronaemia?

Familial chylomicronaemia is an inherited disease where patients have abnormally high levels of some types of fat called triglycerides in their blood. The excess fat accumulates in organs such as the spleen and liver, which become abnormally enlarged. Fat accumulation can also cause repeated bouts of pancreatitis (inflammation of the pancreas) and xanthomas (the formation of yellow fatty deposits just under the skin, generally around joints).

The cause of the disease is often the body's failure to produce enough quantities of an enzyme called lipoprotein lipase, which is involved in breaking down fats from the diet.

Familial chylomicronaemia is a debilitating disease that may be life threatening because the bouts of pancreatitis can be severe and sometimes fatal.

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

At the time of designation, familial chylomicronaemia affected less than 0.1 in 10,000 people in the European Union (EU). This is equivalent to fewer than 5,000 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, there were no satisfactory methods authorised in the EU for the treatment of familial chylomicronaemia. Patients were treated using dietary restrictions (avoiding foods that contain a high level of fat). Patients were also advised to avoid the use of substances known to increase the level of triglycerides in the blood, such as alcohol, diuretics or oestrogens.

How is this medicine expected to work?

Lomitapide is expected to work by blocking the action of 'microsomal triglyceride transfer protein'. This protein is located within the liver and the gut cells, where it is involved in assembling fatty substances into larger particles that are then released into the blood stream. By blocking this protein, the medicine is expected to decrease the level of fats in the blood, thereby helping to control the symptoms of the disease.

What is the stage of development of this medicine?

The effects of lomitapide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with lomitapide in patients with familial chylomicronaemia were planned.

At the time of submission, lomitapide was not authorised anywhere in the EU for familial chylomicronaemia 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 7 October 2010 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.

[*]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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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	Lomitapide	Treatment of familial chylomicronaemia
Bulgarian	Ломитапид	Лечение на наследствена хиломикронемия
Croatian	Lomitapid	Liječenje obiteljske hilomikronemije
Czech	Lomitapid	Léčba familiární chylomikronemie
Danish	Lomitapid	Behandling af familiær kylomikronæmi
Dutch	Lomitapide	Behandeling van familiale chylomicronemie
Estonian	Lomitapiid	Perekondliku hüperkülomikroneemia ravi
Finnish	Lomitapidi	Familiaalisen kylomikronemian hoito
French	Lomitapide	Traitement de l'hypercholestérolémie familiale homozygote
German	Lomitapid	Behandlung der familiären Chylomikronämie
Greek	Λομιταπίδη	Θεραπεία οικογενούς χυλομικροναιμίας
Hungarian	Lomitapid	Familiaris chylomicronaemia kezelésére
Italian	Lomitapide	Trattamento della chilomicronemia familiare
Latvian	Lomitapīds	Ģimenes hilomikronēmijas ārstēšana
Lithuanian	Lomitapidas	Šeiminės chilomikronemijos gydymas
Maltese	Lomitapide	Kura tal-kilomikronemija li tintiret
Polish	Lomitapid	Leczenie chylomikronemii rodzinnej
Portuguese	Lomitapida	Tratamento de quilomicronemia familiar
Romanian	Lomitapidă	Tratamentul chilomicronemiei familiale
Slovak	Lomitapid	Liečba familiárnej chylomikronémie
Slovenian	Lomitapid	Zdravljenje družinske hilomikronemije
Spanish	Lomitapida	Tratamiento de la quilomicronemia familiar
Swedish	Lomitapid	Behandling av familjär kylomikronemi
Norwegian	Lomitapid	Behandling av familiær kylomikronemi
Icelandic	Lomitapíð	Meðferð við ættgengum fitukirnadreyra

[1] At the time of transfer of sponsorship