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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Metreleptin for the treatment of familial partial lipodystrophy

First publication	28 August 2012
Rev.1: transfers of sponsorship	25 April 2014
Rev.2: sponsor's change of address	1 October 2014
Rev.3: transfer of sponsorship	20 April 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 July 2012, orphan designation (EU/3/12/1022) was granted by the European Commission to Aptiv Solutions (UK) Limited, United Kingdom, for metreleptin for the treatment of familial partial lipodystrophy.

The sponsorship was transferred to Bristol-Myers Squibb / AstraZeneca EEIG, United Kingdom, in February 2014 then to AstraZeneca AB, Sweden, in April 2014 and subsequently to Aegerion Pharmaceuticals Limited, United Kingdom, in March 2015.

What is familial partial lipodystrophy?

Familial partial lipodystrophy is a condition characterised by loss of subcutaneous (under the skin), adipose (fatty) tissue in some parts of the body, and accumulation of fat in other parts. Patients with familial partial lipodystrophy have normal body fat distribution during infancy and early childhood, but during or after puberty, patients develop progressive loss of fat in the limbs, anterior abdomen and chest. Many patients (especially women) have accumulation of fat in the face, neck, and intra-abdominal regions.

The disease leads to severe complications, including high levels of fats called triglycerides circulating in the blood, insulin resistance (when the body is unable to recognise insulin, a hormone that helps regulate blood sugar levels), diabetes and an abnormal build-up of fats in organs, especially the liver.

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Familial partial lipodystrophy is a long-term debilitating condition because of its severe complications including diabetes, hypertriglyceridemia (high blood triglyceride levels), acute pancreatitis (inflammation of the pancreas), and severe disease of major organs such as the heart and liver.

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

At the time of designation, familial partial lipodystrophy affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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 methods were authorised in the EU for the treatment of familial partial lipodystrophy. Patients with the condition were advised to follow a low-fat diet.

How is this medicine expected to work?

Metreleptin is similar to a human hormone called leptin, which plays a key role in regulating body fat. In familial partial lipodystrophy, metreleptin is expected to increase fat breakdown in the blood, muscles and liver, and improve insulin function, thereby correcting some abnormalities in patients with this condition such as insulin resistance. However, the medicine is not expected to restore adipose tissue.

Metreleptin is made by a method known as 'recombinant DNA technology': it is made by bacteria that have received a gene (DNA) which makes them able to produce metreleptin.

What is the stage of development of this medicine?

The effects of metreleptin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with metreleptin including patients with familial partial lipodystrophy were ongoing.

At the time of submission, metreleptin was not authorised anywhere in the EU for familial partial lipodystrophy. Orphan designation of metreleptin had been granted in the United States of America for metabolic disorders secondary to lipodystrophy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 June 2012 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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	Metreleptin	Treatment of familial partial lipodystrophy
Bulgarian	Метрелептин	Лечение на фамилна частична липодистрофия
Czech	Metreleptin	Léčba familiární parciální lipodystrofie
Croatian	Metreleptin	Liječenje obiteljske parcijalne lipodistrofije
Danish	Metreleptin	Behandling af familiær partiel lipodystrofi
Dutch	Metreleptine	Behandeling van Familiale Partiële Lipodystrofie
Estonian	Metreleptiin	Perekondliku osalise lipodüstroofia ravi
Finnish	Metreleptiini	Familiaalisen osittaisen lipodystrofian hoito
French	Métréleptine	Traitement de la lipodystrophie familiale partielle
German	Metreleptin	Behandlung der familiären partiellen Lipodystrophie
Greek	Μετρελεπτίνη	Θεραπεία της οικογενούς μερικής λιποδυστροφίας
Hungarian	Metreleptin	Familiáris parciális lipodystrophia kezelése
Italian	Metreleptina	Trattamento della lipodistrofia parziale familiare
Latvian	Metreleptīns	Ģimenes parciālās lipodistrofijas ārstēšana
Lithuanian	Metreleptinas	Paveldimos dalinės lipodistrofijos gydymas
Maltese	Metreleptin	Kura tal-Lipodistrofija Parzjali li Tintiret
Polish	Metreleptyna	Leczenie lipodystrofii rodzinnej częściowej
Portuguese	Metreleptina	Tratamento da Lipodistrofia parcial familiar
Romanian	Metreleptină	Tratamentul lipodistrofiei parţiale familiale
Slovak	Metreleptin	Liečba parciálnej familiárnej lipodystrofie
Slovenian	Metreleptin	Zdravljenje familiarne parcialne lipodistrofije
Spanish	Metreleptina	Tratamiento de la lipodistrofia familiar parcial
Swedish	Metreleptin	Behandling av familjär partiell lipodystrofi
Norwegian	Metreleptin	Behandling av familiær partiell lipodystrofi
Icelandic	Metreleptín	Meðferð á ættgengum, hluta fitukyrkingi

[1] At the time of transfer of sponsorship