5 September 2016
EMA/COMP/444302/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Volanesorsen sodium for the treatment of familial partial lipodystrophy

On 14 July 2016, orphan designation (EU/3/16/1711) was granted by the European Commission to Ionis USA Ltd, United Kingdom, for volanesorsen sodium for the treatment of familial partial lipodystrophy.

What is familial partial lipodystrophy?

Familial partial lipodystrophy is an inherited condition in which fat is lost from some parts of the body and it accumulates in other parts. Patients with familial partial lipodystrophy have normal body fat distribution until early childhood, but during or after puberty they lose fat progressively from the arms and legs, and from the front of the belly and chest. Many patients (especially women) have accumulation of fat in the face, neck and within the belly.

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 to regulate blood sugar levels), diabetes and an abnormal build-up of fats in organs, especially the liver and heart.

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

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

At the time of designation, familial partial lipodystrophy affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of 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).

*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 513,700,000 (Eurostat 2016).

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What treatments are available?

At the time of designation, no satisfactory 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 and to exercise. Medicines that increase the effect of insulin (e.g. metformin) and lipid-lowering medicines (e.g. statins, fibrates) can improve the condition but don't significantly lower triglyceride levels.

How is this medicine expected to work?

This medicine is an 'antisense oligonucleotide', a short piece of genetic material that has been designed to attach to genetic material in the cell responsible for producing the apoC-III protein. This reduces production of apoC-III, which is often raised in lipodystrophy patients. Since apoC-III regulates triglyceride levels in the body, reducing its production is expected to reduce triglyceride levels and improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of volanesorsen sodium have been evaluated in experimental models.

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

At the time of submission, volanesorsen sodium was not authorised anywhere in the EU for familial partial lipodystrophy 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 16 June 2016 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.

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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Volanesorsen sodium	Treatment of familial partial lipodystrophy
Bulgarian	Воланесорсен натрий	Лечение на фамилна частична липодистрофия
Croatian	Volanesorsen natrij	Liječenje obiteljske parcijalne lipodistrofije
Czech	Volanesorsen sodná sůl	Léčba familiární parciální lipodystrofie
Danish	Volanesorsen natrium	Behandling af familiær partiel lipodystrofi
Dutch	Volanesorsen natrium	Behandeling van Familiale Partiële Lipodystrofie
Estonian	Volanesorseen-naatrium	Perekondliku osalise lipodüstroofia ravi
Finnish	Volanesorseeninatrium	Familiaalisen osittaisen lipodystrofian hoito
French	Volanésorsen de sodium	Traitement de la lipodystrophie familiale partielle
German	Volanesorsen Natrium	Behandlung der familiären partiellen Lipodystrophie
Greek	Βολανεσορσένη νατρίου	Θεραπεία της οικογενούς μερικής λιποδυστροφίας
Hungarian	Volanesorsen-nátrium	Familiáris parciális lipodystrophia kezelése
Italian	Volanesorsen sodico	Trattamento della lipodistrofia parziale familiare
Latvian	Volanesorsēna nātrija sāls	Ģimenes parciālās lipodistrofijas ārstēšana
Lithuanian	Volanesorseno natris	Šeiminės dalinės lipodistrofijos gydymas
Maltese	Volanesorsen sodium	Kura tal-lipodistrofija pParzjali li tintiret
Polish	Sól sodowa wolanesorsenu	Leczenie lipodystrofii rodzinnej częściowej
Portuguese	Volanesorseno de sódio	Tratamento da lipodistrofia parcial familiar
Romanian	Volanesorsen sodiu	Tratamentul lipodistrofiei familiale parţiale
Slovak	Sodná soľ volanesorsenu	Liečba parciálnej familiárnej lipodystrofie
Slovenian	Natrijev volanesorsenat	Zdravljenje familiarne parcialne lipodistrofije
Spanish	Volanesorsen de sodio	Tratamiento de la lipodistrofia familiar parcial
Swedish	Natriumvolanesorsen	Behandling av familjär partiell lipodystrofi
Norwegian	Volanesorsen natrium	Behandling av familiær partiell lipodystrofi
Icelandic	Vólanesórsen natríum	Meðferð á ættgengum, hluta fitukyrkingi

[1] At the time of designation