23 September 2014
EMA/COMP/434437/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human apolipoprotein A-I in a complex with phospholipids for the treatment of apolipoprotein A-I deficiency

On 22 August 2014, orphan designation (EU/3/14/1315) was granted by the European Commission to Cerenis Therapeutics Holding SA, France, for recombinant human apolipoprotein A-I in a complex with phospholipids for the treatment of apolipoprotein A-I deficiency.

What is apolipoprotein A-I deficiency?

Apolipoprotein A-I is a substance found in fatty particles in the blood called high-density lipoprotein (HDL). The cholesterol removed from cells normally attaches to these fatty particles and is commonly referred to as HDL or 'good' cholesterol. These particles are then transported to the liver where they can be broken down and the cholesterol removed from the body.

In patients with apolipoprotein A-I deficiency, mutations (defects) in the gene responsible for producing apoliprotein A-I result in low levels or the absence of this substance. This results in very low levels of HDL particles in the blood and in damaging build-up of cholesterol in blood vessels, liver, skin, nerves and eye.

Apolipoprotein A-I deficiency is a long-term debilitating and life-threatening condition due in particular to the cholesterol deposits in the arteries (atherosclerosis).

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

At the time of designation, the condition affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

No satisfactory methods of treatment were authorised in the EU for apolipoprotein A-I deficiency at the time of designation. Patients were usually managed with measures such as a low-fat diet and medicines to lower lipids (fats) in the blood.

How is this medicine expected to work?

The medicine contains replacement HDL-like particles containing apolipoprotein A-I in a form that absorbs cholesterol very efficiently. This temporarily restores the normal processes for removing excess cholesterol from the body by facilitating the loss of cholesterol from the cells, particularly in the walls of blood vessels.

The apolipoprotein A-I in this medicine is made by a method known as 'recombinant DNA technology': it is made by cells that have received a gene (DNA) that makes them able to produce the apolipoprotein.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with apolipoprotein A-I deficiency were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for apolipoprotein A-I deficiency 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.

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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:

Cerenis Therapeutics Holding SA
265 rue de la Découverte
Bât. A, 31670 Labege
France
Tel. +33 562 2409 46
Fax +33 562 1904 17
E-mail: info@cerenis.com

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	Recombinant human apolipoprotein A-I in a complex with phospholipids	Treatment of apolipoprotein A-I deficiency
Bulgarian	Рекомбинантен човешки аполипопротеин А-I в комплекс с фосфолипиди	Лечение на дефицит на аполипопротеин А-I
Croatian	Rekombinantni ljudski apolipoprotein A-I u kompleksu s fosfolipidima	Liječenje nedostatka apolipoproteina A-I
Czech	Rekombinantní humánní apolipoprotein A-I v komplexu s fosfolipidy	Léčba deficience apolipoproteinu A-I
Danish	Rekombinant humant apolipoprotein A-1 i et kompleks med fosfolipider	Behandling af apolipoprotein A-1 mangel
Dutch	Recombinant humaan apolipoproteïne A-I in een complex met fosfolipiden	Behandeling van apolipoproteïne A-I deficiëntie
Estonian	Rekombinantne inimese apolipoproteiin A-I kompleksis fosfolipiididega	Apolipoproteiin A-I puudulikkuse ravi
Finnish	Yhdistelmä-DNA-tekniikalla valmistettu humaani-apolipoproteiini A-I:tä ja fosfolipidejä sisältävä kompleksi	Apolipoproteiini A-I:n puutoksen hoito
French	Apolipoprotéine A-I humaine recombinante dans un complexe avec des phospholipides	Traitement de la déficience en apolipoprotéine A-I
German	Rekombinantes humanesApolipoprotein A-I in einem Komplex mit Phospholipiden	Behandlung von apoA-I- Mangel
Greek	Ανασυνδυασμένη ανθρώπινη απολιποπρωτεΐνη Α-I σε σύμπλεγμα με φωσφολιπίδια	Θεραπεία της ανεπάρκειας απολιποπρωτεΐνης Α-I
Hungarian	Rekombináns humán apolipoprotein A-I foszfolipid komplexben	Apolipoprotein A-I hiány kezelése
Italian	Apolipoproteina A1 ricombinante umana in un complesso fosfolipidico	Trattamento del deficit di apolipoproteina A1
Latvian	Rekombinants cilvēka A-I apolipoproteīns, kas saistīts ar fosfolipīdiem	A-I apolipoproteīna deficīta ārstēšana
Lithuanian	Rekombinantinis žmogaus apolipoproteinas A-I junginyje su fosfolipidais	Apolipoproteino A-I stokos gydymas
Maltese	Apolipoproteina A-I umana rikombinanti magħquda ma' fosfolipidi	Kura ta' nuqqas ta' apolipoproteina A-I
Polish	Kompleks rekombinowanej ludzkiej apolipoproteiny A-I z fosfolipidami	Leczenie niedoboru apolipoproteiny A-I
Portuguese	Apolipoproteína A-I humana recombinante num complexo com fosfolípidos	Tratamento da deficiência de apolipoproteína A-I
Romanian	Apolipoproteina A-I umană recombinantă într-un complex cu fosfolipide	Tratamentul deficitului de apolipoproteină A-I
Slovak	Rekombinantný ľudský apolipoproteín A-I v komplexe s fosfolipidmi	Liečba nedostatku apolipoproteínu A-I

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Kompleks rekombinantnega humanega apolipoproteina A-I s fosfolipidi	Zdravljenje pomanjkanja apolipoproteina A-I
Spanish	Apolipoproteína A-I humana recombinante en un complejo fosfolipídico	Tratamiento de la deficiencia de apolipoproteína A-I
Swedish	Rekombinant humant apolipoprotein A-I i ett komplex med fosfolipider	Behandling av brist på apo apolipoprotein A-I
Norwegian	Rekombinant humant apolipoprotein A-I i et kompleks med fosfolipider	Behandling av mangel på apolipoprotein A-I
Icelandic	Raðbrigða apólípóprótein A-I úr mönnum í flóka með fosfólípíðum	Meðferð við skorti á apólípópróteini A-I