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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt for the treatment of familial chylomicronaemia syndrome (type I hyperlipoproteinaemia)

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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 14 September 2012, orphan designation (EU/3/12/1036) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt for the treatment of familial chylomicronaemia syndrome (type I hyperlipoproteinaemia).

What is familial chylomicronaemia syndrome?

Familial chylomicronaemia syndrome (also known as type I hyperlipoproteinaemia) 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 (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 syndrome 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 syndrome affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 syndrome. 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?

This medicine is expected to work by blocking the action of an enzyme called 'diacylglycerol acyltransferase 1'. This enzyme is found in high levels in the intestine and fat tissue, where it is normally involved in producing triglycerides that are then released into the blood stream. By blocking this enzyme, the medicine is expected to decrease the level of triglycerides in the blood, thereby helping to control the symptoms of the disease.

What is the stage of development of this medicine?

The effects of trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine including patients with familial chylomicronaemia syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for familial chylomicronaemia syndrome. Orphan designation of this medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2012 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 509,000,000 (Eurostat 2012).

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	Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt	Treatment of familial chylomicronaemia syndrome (type I hyperliporpoteinaemia)
Bulgarian	Транс-4-[4-[5-[[6-(трифлуорометил)-3-пиридинил]амино]-2-пиридинил]фенил]циклохексан оцетна киселина, натриева сол	Лечение на наследствена хиломикронемия синдром (тип I хиперлипопротеинемия)
Czech	Sodná sůl trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl]cyclohexan octové kyseliny	Léčba familiární chylomikronemie syndrom (typ I hyperlipoproteinemie)
Danish	Trans-4-[4-[5-[[6-(trifluormethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl]cyclohexan ethansyre, natriumsalt	Behandling af familiær kylomikronæmi syndrom (type I hyperlipoproteinæmi)
Dutch	Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl]cyclohexaanazijnzuurnatriumzout	Behandeling van familiale chylomicronemie syndroom (type I hyperlipoproteïnemie)
Estonian	Trans-4-[4-[5-[[6-(trifluorometüül)-3-püridinüül]amino]-2-püridinüül]fenüül]tsükloheksaan etaanhappe naatriumisool	Perekondliku hüperkülomikroneemia ravi sündroom (I tüüpi hüperlipideemia)
Finnish	Trans-4-[4-[5-[[6-(trifluorometyyli)-3-pyridinyyli]amino]-2-pyridinyyli]fenyyli]sykloheksaani etaanihapon, natriumsuola	Familiaalisen kylomikronemiaoireyhtymän hoito (tyyppi I hyperlipoproteinemiai)
French	Acide trans-4-[4-[5-[[6-(trifluorométhyl)-3-pyridinyl]amino]-2-pyridinyl]phényl]cyclohexane acétique, sel sodique	Traitement du syndrome de chilomircronemia familiale syndrome (type I hyperlipipoproteinemie)
German	Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexan essigsäure natrium salz	Behandlung des familiären Chylomikronämie Syndroms (Typ I-Hyperlipoproteinämie)
Greek	Trans-4-[4-[5-[[6-(τριφθοριομεθυλο)-3-πιρυδινυλ]αμινο]-2-πιριδυνυλ]φαινυλο]κυκλοεξανοξικό οξύ, ;άλας νατρίου	Θεραπεία του συνδρόμου οικογενούς χυλομικροναιμίας (υπερλιποπρωτεϊναιμία τύπου Ι)
Hungarian	Transz-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]ciklohexán-ecetsav nátrium sója	Familiaris chylomicronaemia szindróma (I. típusú hyperlipidaemia) kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	Sale sodico dell'acido trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]cicloesanacetico	Trattamento della chilomicronemia familiare (iperlipoproteinemia di tipo I)
Latvian	Trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]cikloheksānetiķskābes nātrija sāls	Ģimenes hilomikronēmijas ārstēšana sindroms (I tipa hiperlipidēmija)
Lithuanian	Trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]cikloheksano acto rūgšties natrio druska	Šeiminės chilomikronemijos gydymas sindromas (I tipo hiperlipidemija)
Maltese	Trans-4-[4-[5-[[6-(trifluoromethyl)-3-pyridinyl]amino]-2-pyridinyl]phenyl] cyclohexane acetic acid sodium salt	Kura tas-sindrome tal-kilomikronemija li tintiret (iperlipoproteinemija tat-tip I)
Polish	Sól sodowa kwasu trans-4-[4-[5-[[6-(trifluorometylo)-3-pirydynylo]amino]-2-pirydynylo]fenylo]cykloheksano octowego	Leczenie zespołu chylomikronemii rodzinnej (hiperlipoproteinemia typu I)
Portuguese	Sal sódico de trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]ciclohexano de ácido acético.	Tratamento do síndrome de quilomicronemia familiar (hiperlipoproteinémia tipo I)
Romanian	Sare sodică a acidului trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]ciclohexan acetic,	Tratamentul sindromului chilomicronemiei familiale (hiperlipoproteinemia de tip I)
Slovak	Sodná soľ kyseliny trans-4-[4-[5-[[6-(trifluórometyl)-3-pyridinyl]amino]-2-pyridinyl]fenyl]cyklohexán-octovej	Liečba familiárnej chylomikronémie syndróm (typ I hyperlipidémia)
Slovenian	Natrijev trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]cikloheksan acetat	Zdravljenje sindroma družinske hilomikronemije (hiperlipoproteinemija tip I)
Spanish	Sal sódica de ácido acético trans-4-[4-[5-[[6-(trifluorometil)-3-piridinil]amino]-2-piridinil]fenil]ciclohexano	Tratamiento del síndorme de quilomicronemia familiar (hiperlipidemia de tipo I)
Swedish	Trans-4-[4-[5-[[6-(trifluorometyl)-3-pyridinyl]amino]-2-pyridinyl]fenyl]cyklohexan ättiksyra, natrium salt	Behandling av familjär kylomikronemi syndrom (typ I hyperlipoproteinemi)
Norwegian	Trans-4-[4-[5-[[6-(trifluorometyl)-3-pyridinyl]amino]-2-pyridinyl]fenyl]sykloheksan eddiksyre natriumsalt	Behandling av familiær kylomikronemi syndrom (type I hyperlipoproteinemi)
Icelandic	Trans-4-[4-[5-[[6-(tríflúormetýl)-3-pýridínýl]amínó]-2-pýridínýl]fenýl]sýklóheksan edikssýru, natríumsalt	Meðferð við ættgengum fitukirnadreyra heilkenni (gerð I hyperfitupróteindreyra heilkenni)