26 March 2013
EMA/COMP/92888/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ramiprilat for the treatment of Stargardt's disease

On 12 March 2013, orphan designation (EU/3/13/1117) was granted by the European Commission to Iris Pharma, France, for ramiprilat for the treatment of Stargardt's disease.

What is Stargardt's disease?

Stargardt's disease is a genetic (hereditary) disorder of the eye that leads to progressive loss of sight. Stargardt's disease is caused by abnormalities in a gene called *ABCA4*. The *ABCA4* gene is responsible for the production of a protein called ABCR that regulates the transport of substances in and out of some cells in the retina (the light-sensitive surface at the back of the eye). In patients with Stargardt's disease, ABCR does not work properly. This causes deposits to build up inside the retina cells, which become damaged and eventually die.

Stargardt's disease is a long-term debilitating disease because the patient's sight becomes progressively worse and eventually leads to blindness.

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

At the time of designation, Stargardt's disease affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,100 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 submission of the application for orphan designation, no satisfactory methods were authorised in the EU for the treatment of Stargardt's disease. Patients with the disease were usually given physical aids such as sunglasses to reduce the rate of damage to the retina, or spectacles, magnifiers or telescopes to help them see during the early stages of the disease. Laser treatment can help to stabilise the loss of vision in some patients.

*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. This represents a population of 509,000,000 (Eurostat 2013).

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How is this medicine expected to work?

Ramiprilat is an angiotensin-converting enzyme (ACE) inhibitor. ACE inhibitors have several actions in the body, one of which is reducing the breakdown of a substance called bradykinin. This in turn results in increased release of nitric oxide, a substance which is known to protect nerve cells from damage, including retina cells. ACE inhibitors may also help reduce damage to cells in the retina by reducing the formation of toxic forms of oxygen called free radicals. Ramiprilat is expected to be given as eye drops.

What is the stage of development of this medicine?

The effects of ramiprilat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with ramiprilat in patients with Stargardt's disease had been started.

At the time of submission, ramiprilat was not authorised anywhere in the EU for Stargardt's disease 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 6 February 2013 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:

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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	Ramiprilat	Treatment of Stargardt's disease
Bulgarian	Рамиприлат	Лечение на болест на Stargardt
Czech	Ramiprilát	Léčba Stargardtovy choroby
Danish	Ramiprilat	Behandling af Stargardt sygdom
Dutch	Ramiprilaat	Behandeling van de ziekte van Stargardt
Estonian	Ramiprilaat	Stargardt'tõve ravi
Finnish	Ramiprilaatti	Stargardtin taudin hoito
French	Ramiprilate	Traitement de la maladie de Stargardt
German	Ramiprilat	Behandlung der Stargardt-Krankheit
Greek	Ραμιπριλάτη	Θεραπευτική αγωγή για την νόσο του Stargardt
Hungarian	Ramiprilát	Stargardt-kór kezelése
Italian	Ramiprilato	Trattamento della malattia di Stargardt
Latvian	Ramiprilāts	Stargardta slimības ārstēšana
Lithuanian	Ramiprilatas	Stargardt ligos gydymas
Maltese	Ramiprilat	Kura tal-marda ta' Stargardt
Polish	Ramiprylat	Leczenie choroby Stargardta
Portuguese	Ramiprilato	Tratamento da doença de Stargardt
Romanian	Ramiprilat	Tratamentul bolii Stargardt
Slovak	Ramiprilát	Liečba Stargardtovej choroby
Slovenian	Ramiprilat	Zdravljenje Stargardtjeve bolezni
Spanish	Ramiprilato	Tratamiento de la enfermedad de Stargardt
Swedish	Ramiprilat	Behandling av Stargardts sjukdom
Norwegian	Ramiprilat	Behandling av Stargardts sykdom
Icelandic	Ramiprílat	Meðferð við Stargardts sjúkdómi

[1] At the time of designation