28 November 2013
EMA/COMP/631348/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Soraprazan for the treatment of Stargardt's disease

On 13 November 2013, orphan designation (EU/3/13/1208) was granted by the European Commission to Katairo GmbH, Germany, for soraprazan 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 to 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of between 51,000 and 67,000 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 submission of the application for orphan designation, no satisfactory methods were authorised in the EU for the treatment of Stargardt's disease.

*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 512,200,000 (Eurostat 2013).

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How is this medicine expected to work?

Soraprazan is expected to be able to enter the cells of the retina, where it attaches to the abnormal deposits that damage the retina cells. Although the way it works is not fully understood, soraprazan is thought to cause the deposits to break up and partly dissolve. The broken-down deposits can then be expelled by the cell's own natural mechanisms, reducing their build-up and therefore the damage to the cell.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of soraprazan in experimental models was ongoing.

At the time of submission, no clinical trials with soraprazan in patients with Stargardt's disease had been started.

At the time of submission, soraprazan 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 9 October 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	Soraprazan	Treatment of Stargardt's disease
Bulgarian	Сорапразан	Лечение на болест на Stargardt
Czech	Soraprazan	Léčba Stargardtovy choroby
Croatian	Soraprazan	Liječenje Stargardtove bolesti
Danish	Soraprazan	Behandling af Stargardt sygdom
Dutch	Soraprazan	Behandeling van de ziekte van Stargardt
Estonian	Soraprasaan	Stargardt'tõve ravi
Finnish	Sorapratsaani	Stargardtin taudin hoito
French	Soraprazan	Traitement de la maladie de Stargardt
German	Soraprazan	Behandlung der Stargardt-Krankheit
Greek	Σοραπραζάνη	Θεραπευτική αγωγή για την νόσο του Stargardt
Hungarian	Szoraprazan	Stargardt-kór kezelése
Italian	Soraprazan	Trattamento della malattia di Stargardt
Latvian	Soraprazāns	Stargardta slimības ārstēšana
Lithuanian	Soraprazanas	Stargardt ligos gydymas
Maltese	Soraprazan	Kura tal-marda ta' Stargardt
Polish	Soraprazan	Leczenie choroby Stargardta
Portuguese	Soraprazan	Tratamento da doença de Stargardt
Romanian	Soraprazan	Tratamentul bolii Stargardt
Slovak	Soraprazan	Liečba Stargardtovej choroby
Slovenian	Soraprazan	Zdravljenje Stargardtjeve bolezni
Spanish	Soraprazán	Tratamiento de la enfermedad de Stargardt
Swedish	Soraprazan	Behandling av Stargardts sjukdom
Norwegian	Soraprazan	Behandling av Stargardts sykdom
Icelandic	Sóraprazan	Meðferð við Stargardts sjúkdómi

[1] At the time of designation