6 May 2015
EMA/COMP/125722/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Enoxacin for the treatment of amyotrophic lateral sclerosis

On 19 March 2015, orphan designation (EU/3/15/1459) was granted by the European Commission to Impasara Ltd, United Kingdom, for enoxacin for the treatment of amyotrophic lateral sclerosis.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS vary depending on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, and difficulty speaking, swallowing and breathing. ALS usually starts in mid-life and men are more likely to develop the disease than women.

ALS is a long-term debilitating and life-threatening disease because of the gradual loss of function and its paralysing effect on muscles used for breathing which usually leads to death due to respiratory failure.

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

At the time of designation, ALS affected not more than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 51,000 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, riluzole was authorised in the EU to treat ALS. Patients also received supportive treatment to temporarily relieve the symptoms of the disease, such as physiotherapy and speech therapy.

*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 512,900,000 (Eurostat 2015).

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The sponsor has provided sufficient information to show that enoxacin might be of significant benefit for patients with the condition because early studies in experimental models showed that the medicine may delay the onset of symptoms and improve muscle function. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Enoxacin is an antibiotic used to treat a variety of bacterial infections. Apart from its antibacterial activity, studies have shown that enoxacin can increase the activity of an enzyme called 'dicer', which cuts the RNA (the genetic material that guides the production of proteins) in small fragments called siRNA and miRNA, which are involved in the production of proteins. In patients with ALS, miRNA is produced at a reduced rate, and this is thought to contribute to the deterioration of the nerve cells and the development of the disease. By increasing the activity of dicer, enoxacin is expected to increase the levels of miRNA, therefore slowing down the progression of the disease.

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, no clinical trials with the medicine in patients with ASL had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for ALS or designated as an orphan medicinal product elsewhere for this condition. Enoxacin was authorised in some EU member states for the treatment of bacterial infections.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 February 2015 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

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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	Enoxacin	Treatment of amyotrophic lateral sclerosis
Bulgarian	еноксацин	Лечение на амиотрофична латерална склероза
Croatian	Enoksacin	Liječenje amiotrofične lateralne skleroze
Czech	Enoxacin	Léčba amyotrofické laterální sklerózy
Danish	Enoxacin	Behandling af amyotrofisk lateralsklerose
Dutch	Enoxacine	Behandeling van amyotrofe lateraalsclerose
Estonian	Enoxaksiin	Amüotroofilise lateraalskleroosi ravi
Finnish	Enoksasiini	Amyotrofisen lateraaliskleroosin hoito
French	Énoxacine	Traitement de la sclérose latérale amyotrophique
German	Enoxacin	Behandlung der amyotrophen Lateralsklerose
Greek	Ενοξασίνη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Enoxacin	Amyotrophiás lateral sclerosis kezelése
Italian	Enoxacina	Trattamento della sclerosi laterale amiotrofica
Latvian	Enoksacīns	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Enoksacinas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Enoxacin	Kura tas-sklerosi laterali amjotrofika
Polish	Enoksacyna	Leczenie stwardnienia bocznego zanikowego
Portuguese	Enoxacina	Tratamento da esclerose lateral amiotrófica
Romanian	Enoxacină	Tratamentul sclerozei laterale amiotrofice
Slovak	Enoxacín	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Enoksacin	Zdravljenje amiotrofične lateralne skleroze
Spanish	Enoxacino	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Enoxacin	Behandling av amyotrofisk lateralskleros
Norwegian	Enoksacin	Behandling av amyotrofisk lateralsklerose
Icelandic	Enoxacín	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of designation