25 July 2016
EMA/396044/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cyclocreatine for the treatment of creatine deficiency syndromes

On 27 June 2016, orphan designation (EU/3/16/1676) was granted by the European Commission to Pharma Gateway AB, Sweden, for cyclocreatine for the treatment of creatine deficiency syndromes.

What are creatine deficiency syndromes?

Creatine deficiency syndromes are a group of disorders in which the body cannot make a substance known as creatine or transport it into the brain. The resulting lack of creatine in the brain causes severe problems including learning disability, speech and behaviour problems and epilepsy. Additional features include failure of the baby to thrive, poor growth, weak muscle tone and abnormal movements.

Creatine deficiency syndromes are debilitating in the long term because of their effects on the child's mental development.

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

At the time of designation, creatine deficiency syndromes affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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).

What treatments are available?

At the time of designation, no satisfactory treatments were authorised for the condition and the only standard treatment was life-long supportive care. Creatine supplements were used in some forms of the condition.

*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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

Cyclocreatine is structurally similar to creatine and is intended as a replacement for creatine that is lacking in the patient's brain. Cyclocreatine is expected to be transported across the blood-brain barrier in a different way to creatine and thus overcome the lack of creatine in the brain.

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 cyclocreatine in experimental models was ongoing.

At the time of submission, no clinical trials with cyclocreatine in patients with creatine deficiency syndromes had been started.

At the time of submission, cyclocreatine was not authorised anywhere in the EU for creatine deficiency syndromes. Orphan designation had been granted in the United States for creatine transporter deficiency.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 May 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Cyclocreatine	Treatment of creatine deficiency syndromes
Bulgarian	Циклокреатин	Лечение на синдроми на креатинов дефицит
Croatian	Ciklokreatin	Liječenje sindroma nedostatka kreatina
Czech	Cyklokreatin	Léčba syndromů s deficitem kreatinu
Danish	Cyclokreatin	Behandling af kreatinmangelsyndromer
Dutch	Cyclocreatine	Behandeling van creatinedeficiëntiesyndromen
Estonian	Tsüklokreatiin	Kreatiini defitsiidi sündroomide ravi
Finnish	Syklokreatiini	Kreatiinin puutosoireyhtymien hoito
French	Cyclocréatine	Traitement de syndromes de déficit en créatine
German	Zyklokreatin	Behandlung von Kreatinmangelsyndromen
Greek	Κυκλοκρεατίνη	Θεραπεία συνδρόμων ανεπάρκειας της κρεατίνης
Hungarian	Ciklokreatin	A kreatinhiányos szindrómák kezelése
Italian	Ciclocreatina	Trattamento delle sindromi da deficit di creatina
Latvian	Ciklokreatīns	Kreatīna deficīta sindromu ārstēšana
Lithuanian	Ciklokreatinas	Kreatino stokos sindromų gydymas
Maltese	Cyclocreatine	Kura ta' sindromi ta' nuqqas ta' kreatina
Polish	Cyklokreatyna	Leczenie zespołów niedoboru kreatyny
Portuguese	Ciclocreatina	Tratamento de síndromas de deficiência em creatina
Romanian	Ciclocreatină	Tratamentul sindroamelor de deficit de creatină
Slovak	Cyklokreatín	Liečba syndrómov nedostatku kreatínu
Slovenian	Ciklokreatin	Zdravljenje sindroma pomanjkanja kreatina
Spanish	Ciclocreatina	Tratamiento de los síndromes de deficiencia de creatina
Swedish	Cyklokreatin	Behandling av kreatinbristsyndrom
Norwegian	Cyclokreatin	Behandling av kreatinmangelsyndromer
Icelandic	Sýklókreatín	Meðferð við kreatínskortsheilkennum

[1] At the time of designation