25 July 2016
EMA/COMP/393522/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Diclofenamide for the treatment of periodic paralysis

On 27 June 2016, orphan designation (EU/3/16/1677) was granted by the European Commission to Sun Pharmaceutical Industries Europe B.V., the Netherlands, for diclofenamide for the treatment of periodic paralysis.

What is periodic paralysis?

Periodic paralysis is a group of inherited muscle disorders that causes periodic attacks of weakness or paralysis (inability to move), which resolve spontaneously after some time. The attacks can be triggered by stress, excitement, physical activity, heat or cold. Muscle weakness can affect a small group of muscles, or can be more generalised and affect the whole body.

Periodic paralysis is caused by abnormalities in the ion channels, tiny pores in the muscle cells that control the passage of charged particles (ions) such as sodium or chloride and play a key role in the contraction and relaxation of muscles.

Periodic paralysis is a long-term debilitating disorder due to permanent muscle weakness, muscle pain and the need for mobility aids. It can be life threatening because of the risk of heart arrhythmias (unstable heartbeat) which could lead to a heart attack.

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

At the time of designation, periodic paralysis affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, no satisfactory methods were authorised in the EU for the treatment of periodic paralysis.

[*]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?

The way this medicine works in periodic paralysis is not fully understood but it is thought to increase the removal of bicarbonate, sodium and potassium through the urine. This increases the acidity of the body (a condition known as 'metabolic acidosis'), which studies show can help normalise the function of the ion channels in muscle cells, thus regulating muscle contraction.

What is the stage of development of this medicine?

The effects of diclofenamide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with diclofenamide in patients with periodic paralysis had finished.

At the time of submission, diclofenamide was authorised in the United States for the treatment of primary periodic paralysis.

At the time of submission, diclofenamide was not authorised anywhere in the EU for periodic paralysis. Orphan designation of diclofenamide had been granted in the EU for periodic paralysis and in the United States for primary periodic paralyses.

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	Diclofenamide	Treatment of periodic paralysis
Bulgarian	диклофенамид	Лечение на периодична парализа
Croatian	Diklofenamid	Liječenje periodične paralize
Czech	Diklofenamid	Léčba periodické paralýzy
Danish	Diclofenamid	Behandling af periodisk paralyse
Dutch	Diclofenamide	Behandeling van periodieke verlamming
Estonian	Diclofenaamiid	Perioodilise paralüüsi ravi
Finnish	Diklofenamidi	Jaksoittaisen halvauksen hoito
French	Diclofénamide	Traitement de la paralysie périodique
German	Diclofenamid	Behandlung von periodischer Paralyse
Greek	Διχλωροφαιναμιδη	Θεραπεία της περιοδικής παράλυσης
Hungarian	Diklofenamid	Periodikus paralízis kezelése
Italian	Diclofenamide	Trattamento della paralisi periodica
Latvian	Diklofenamīds	Periodiskas paralīzes ārstēšana
Lithuanian	Diklofenamidas	Šeiminio periodinio paralyžiaus gydymas
Maltese	Diclofenamide	Kura tal-paraliżi perjodika
Polish	Diklofenamid	Leczenie porażenia okresowego
Portuguese	Diclofenamida	Tratamento da paralisia periódica
Romanian	Diclofenamidă	Tratamentul paraliziei periodice
Slovak	Diklofenamid	Liečba periodického ochrnutia
Slovenian	Diklofenamid	Zdravljenje periodične paralize
Spanish	diclofenamida	Tratamiento de la parálisis periódica
Swedish	Diklofenamid	Behandling av periodisk paralys
Norwegian	Diklofenamid	Behandling av periodisk paralyse
Icelandic	Díklófenamíð	Meðferð við reglubundnu lömun

[1] At the time of designation