

12 January 2015  
EMA/COMP/660449/2014  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Mexiletine hydrochloride for the treatment of myotonic disorders

On 19 November 2014, orphan designation (EU/3/14/1353) was granted by the European Commission to Temmler Pharma GmbH & Co. KG, Germany, for mexiletine hydrochloride for the treatment of myotonic disorders.

#### What are myotonic disorders?

Myotonic disorders are a group of inherited muscle disorders where muscles are slow to relax after contraction. The slow relaxation of the muscles causes symptoms such as stiffness and pain.

Myotonic disorders are 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. When a muscle is affected by a myotonic disorder, these ion channels do not function properly. This dysfunction affects the ability of a muscle to relax after it has contracted. Until relaxation can properly occur, the muscles remain stiff and unusable.

Myotonic disorders are long-term debilitating conditions due to the pain and muscle stiffness, which are associated with frequent falls and disability.

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

At the time of designation, myotonic disorders affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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 the orphan designation, mexiletine hydrochloride was authorised in France to treat myotonic disorders.

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\*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 511,100,000 (Eurostat 2014).

The sponsor has provided sufficient information to show that mexiletine hydrochloride might be of significant benefit for patients with myotonic disorders since the orphan designation may encourage it to be made more widely available. 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?**

Mexiletine hydrochloride is a known antiarrhythmic medicine (used to restore normal heart rhythm). It works by blocking ion channels for sodium ions in muscle cells. These sodium channels play a role in the contraction and relaxation of muscles and by blocking them, the medicine helps to reduce the rate of contractions as well as the stiffness that occurs when the contractions are prolonged.

### **What is the stage of development of this medicine?**

The effects of mexiletine hydrochloride have been evaluated in experimental models and in patients.

At the time of submission of the application for orphan designation, mexiletine hydrochloride was authorised in France for myotonic disorders. Orphan designations of mexiletine hydrochloride medicines have been granted in the EU and the United States for myotonic disorders.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 October 2014 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mexiletine hydrochloride	Treatment of myotonic disorders
Bulgarian	Мексилетин хидрохлорид	Лечение на миотонични нарушения
Croatian	Meksiletinklorid	Liječenje miotoničnih poremećaja
Czech	Mexiletin hydrochlorid	Léčba myotonických chorob
Danish	Mexiletin hydrochlorid	Behandling af myotone lidelser
Dutch	Mexiletine hydrochloride	Behandeling van myotone aandoeningen
Estonian	Meksiletiin hüdrokloriid	Mütooniiliste häirete ravi
Finnish	Meksileitiinihydrokloridi	Myotonisten häiriöiden hoito
French	Chlorhydrate de mexilétine	Traitement des troubles myotoniques
German	Mexiletine Hydrochloride	Behandlung von myotonen Erkrankungen
Greek	Υδροχλωρική μεξιλετίνη	θεραπεία των μυοτονικών διαταραχών
Hungarian	Mexiletin-hidroklorid	Myotoniák kezelése
Italian	Mexiletina cloridrato	Trattamento dei disordini miotonici
Latvian	Meksiletīna hidrohlorīds	Miotonijas ārstēšana
Lithuanian	Meksiletino hidrochloridas	Miotoninių ligų gydymas
Maltese	Mexiletine hydrochloride	Kura ta' mard mijotoniku
Polish	Chlorowodorek meksyletyny	Leczenie zaburzeń miotonicznych
Portuguese	Cloridrato de mexiletina	Tratamento das perturbações miotónicas
Romanian	Clorhidrat de mexiletină	Tratamentul afecțiunilor miotonice
Slovak	Mexiletín hydrochlorid	Liečba myotonických porúch
Slovenian	Meksiletinijev hidroklorid	Zdravljenje miotoničnih motenj
Spanish	Chlorhidrato de mexiletina	El tratamiento de los trastornos miotónicos
Swedish	Mexiletin hydroklorid	Behandling av myotona sjukdomar
Norwegian	Meksiletin hydroklorid	Behandling av myotoniske lidelser
Icelandic	Mexiletín hýdróklóríð	Meðferð mýótónik kvilla

<sup>1</sup> At the time of designation