



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mexiletine hydrochloride for the treatment of non-dystrophic myotonia

On 7 June 2013, orphan designation (EU/3/13/1126) was granted by the European Commission to Prof Michael Hanna, United Kingdom, for mexiletine hydrochloride for the treatment of non-dystrophic myotonia.

What is non-dystrophic myotonia?

Non-dystrophic myotonia is a term used to describe 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, but does not cause the progressive weakening of the muscles seen in 'myotonic dystrophy', another muscle disorder.

Non-dystrophic myotonia 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.

Non-dystrophic myotonia is chronically debilitating 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, non-dystrophic myotonia affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 the orphan designation, the anti-arrhythmia medicine mexiletine was authorised in some EU Member States to treat non-dystrophic myotonia.

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).



The sponsor has provided sufficient information to show that mexiletine hydrochloride might be of significant benefit for patients with non-dystrophic myotonia because mexiletine has been in short supply in the EU and the orphan designation may help address the problem. 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 works by blocking ion channels for sodium ions in the muscle cells. These sodium channels play a role in the contraction and relaxation of muscles and by blocking them, the medicine helps 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 the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with non-dystrophic myotonia were ongoing.

At the time of submission, the medicinal product was not authorised anywhere in the EU for non-dystrophic myotonia. Orphan designation of the medicinal product had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 17 April 2013 recommending the granting of this designation.

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:

Prof Michael Hanna
MRC Centre for Neuromuscular Diseases
8-11 Queen Square
London
WC1N 3BG
United Kingdom
Telephone: +44 0203 448 8014
Telefax: +44 0203 448 3633
E-mail: m.hanna@ucl.ac.uk

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mexiletine hydrochloride	Treatment of non-dystrophic myotonia
Bulgarian	Мексилетин хидрохлорид	Лечение на недистрофична миотония
Czech	Mexiletin hydrochlorid	Léčba Non-dystrofických myotonia
Danish	Mexiletin Hydrochloride	Behandling af ikke-dystrofisk Myotoni
Dutch	Mexiletine hydrochloride	Behandeling van niet-dystrofische myotonia
Estonian	Meksiletiinhüdrokloriid	Mitte-düstroofilise müotoonia ravi
Finnish	Meksiletiinihydrokloridi	Ei-dystrofisen myotonian hoito
French	Chlorhydrate de mexilétine	Traitement de la myotonie non-dystrophique
German	Mexiletinhydrochlorid	Behandlung der nicht-dystrophen Myotonie
Greek	Υδροχλωρική μεξιλετίνη	Θεραπεία της μη-δυστροφικής μυοτονίας
Hungarian	Mexiletin hidroklorid	Nem- dystrophiás myotonia kezelése
Italian	Mexiletina cloridrato	Trattamento della Miotonia Non Distrofica
Latvian	Meksiletīna hidrohlorīds	Ne-distrofiskās miotonijas ārstēšana
Lithuanian	Meksiletino hidrochloridas	Nedistrofinės miotonijos gydymas
Maltese	Mexiletine hydrochloride	Kura ta' mijotonija mhux distrofika
Polish	Meksyletyny chlorowodorek	Leczenie miotonii niedystroficznej
Portuguese	Cloridrato de mexiletina	Tratamento da Miotonia não-distrófica
Romanian	Clorhidrat de mexiletină	Tratamentul miotoniei non-distrofice
Slovak	Mexiletín hydrochlorid	Liečba nedystrofickéj myotónieNon-dystrofických myotonia
Slovenian	Meksiletin hidroklorid	Zdravljenje nedistrofične miotonije
Spanish	Clorhidrato de mexiletina	Tratamiento de la miotonía no distrófica
Swedish	Mexiletin hydroklorid	Behandling av icke-Dystrofisk Myotonia
Norwegian	Meksiletin hydroklorid	Behandling av non-dystrof myotoni
Icelandic	Mexiletín hýdróklóríð	Meðferð non-dystrófic mýótóníu

¹ At the time of designation