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Media and Public Relations

Press release

First treatment for rare inherited muscle contraction disorders

The European Medicines Agency has recommended granting a marketing authorisation for Namuscla (mexiletine hydrochloride) for the treatment of adult patients with non-dystrophic myotonia, a group of inherited muscle disorders where muscles are slow to relax after contraction. These disorders are chronic life-long debilitating conditions characterised by pain, fatigue, and muscle stiffness, resulting in frequent falls and disability.

This is the first time that a treatment for certain forms of myotonic disorders could be authorised EU-wide. The active substance mexiletine has been approved for treatment of these disorders in France only since 2010. 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.

Mexiletine is a known antiarrhythmic medicine (used to restore normal heart rhythm), which was first authorised in Europe in the 1970s. 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.

The opinion from the Committee for Medicinal Products for Human Use (CHMP) is based on data from one phase 3 clinical trial in patients with non-dystrophic myotonia as well as data from the literature. These data show that treatment with mexiletine allows relieving stiffness in the muscles. The medicine's safety profile is well-established; the most common unfavourable effects with this medicine were gastrointestinal disorders, such as heartburn, nausea, vomiting, diarrhoea and abdominal pain. Another less frequently occurring side effect of mexiletine is that it can also trigger arrhythmia or aggravate an existing arrhythmia; the CHMP therefore agreed on specific measures to minimise this risk such as certain contraindications and cardiac monitoring.

Namuscla was designated as an orphan medicinal product in November 2014. As always at time of approval, EMA's Committee for Orphan Medicinal Products (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Namuscla's orphan status.

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The opinion adopted by the CHMP is an intermediary step on Namuscla's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Namuscla is Lupin Europe GmbH.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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