BioSpectrum the business of Bio & Health Sciences

Lupin Neurosciences receives CHMP nod for NaMuscla

21 October 2018 | News

The positive opinion from the CHMP was based on a pivotal Phase III clinical study (MYOMEX) which enrolled 25 participants who were diagnosed with non-dystrophic myotonic disorders and symptomatic myotonia.

Lupin Neurosciences, a specialty pharma division of Lupin Ltd, recently announced that the Committee for Medicinal Products for Human Use (CHMP), the scientific committee of the European Medicines Agency (EMA), has adopted a positive opinion recommending the marketing authorization of NaMuscla (mexiletine hydrochloride) for the symptomatic treatment of myotonia in adults with non-dystrophic myotonic (NDM) disorders. Non-dystrophic myotonic disorders are a group of rare, inherited neuromuscular disorders which cause the inability of muscle relaxation following voluntary contraction. NaMuscla reduces myotonia symptoms in adult patients, resulting in a significant improvement in patient quality-of-life and other functional outcomes.

The CHMP's positive opinion will now be reviewed by the European Commission (EC), which has the authority to approve medicines for the European Union (EU). The EC decision is expected within three months and will apply to all 28 countries of the European Union, Norway, Iceland and Liechtenstein. If approved, NaMuscla will be the first treatment licensed throughout the EU for the symptomatic treatment of myotonia in adults with NDM disorders. The therapy had already been awarded Orphan Drug designation.

"With this positive CHMP opinion we are now one step closer to offering NaMuscla to patients with non-dystrophic myotonia, for whom there are currently no licensed treatment options available across all EU countries", said Thierry Volle, President EMEA, Lupin. "We eagerly await the next step; namely the European Commission's decision, but the positive opinion represents an important milestone for Lupin Neurosciences as we build a leading specialty pharma company focused on the development, registration and commercialization of science-based therapies and solutions for neurological disorders that can restore function and significantly improve lives."

The positive opinion from the CHMP was based on a pivotal Phase III clinical study (MYOMEX) which enrolled 25 participants who were diagnosed with non-dystrophic myotonic disorders and symptomatic myotonia, in addition to bibliographical references, including three controlled clinical studies, to support the efficacy and safety of mexiletine.

Today, more than 7500 people in Europe living with NDM have limited access to a licensed treatment for myotonia which reduces the daily burden of this life-altering symptom. Limited access leads to inconsistent medication supply, administrative challenges and associated financial and geographical burdens, which, along with low awareness and clinical experience among healthcare professionals, may result in harm to patients.

“The positive CHMP opinion in favour of the registration of NaMuscla (mexiletine hydrochloride) for the symptomatic treatment of myotonia in adults with non-dystrophic myotonic disorders is a very positive step towards meeting the significant unmet medical needs of this patient group across the European Union,” said Prof. Dr. med. Benedikt Schoser, FEAN (Neurologische Klinik und Poliklinik, Klinikum der Universität München). “Untreated myotonia in NDM patients can lead to significant lifetime disability due to the stiffness, pain and fatigue associated with myotonic syndromes. Access to a consistent mexiletine formulation is the first step in removing barriers to myotonia treatment and optimizing care for these patients.”