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Questions and answers

Refusal of a change to the marketing authorisation for Raxone (idebenone)

On 14 September 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of a change to the marketing authorisation for the medicinal product Raxone. The change concerned the addition of a new use of Raxone in patients with Duchenne muscular dystrophy to slow their gradual loss of breathing ability.

The company that applied for the change to the authorisation is Santhera Pharmaceuticals (Deutschland) GmbH. It may request a re-examination of the opinion within 15 days of receipt of notification of this negative opinion.

What is Raxone?

Raxone is a medicine that contains the active substance idebenone; it is available as tablets (150 mg).

Raxone has been authorised in the EU since September 2015. It is already used to treat Leber's hereditary optic neuropathy (LHON), a genetic disease that leads to loss of sight.

Further information on Raxone's current use can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.

What was Raxone expected to be used for?

Raxone was also expected to be used to slow down the worsening of breathing function in patients with Duchenne muscular dystrophy who are not treated with corticosteroid medicines. Duchenne muscular dystrophy is a genetic disease that gradually causes weakness and loss of muscle function.

Raxone was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 20 March 2007 for Duchenne muscular dystrophy. Further information on the orphan designation can be found here.

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How does Raxone work?

The active substance in Raxone, idebenone, is an anti-oxidant agent that acts on mitochondria (the structures inside cells that produce the energy necessary for cells to function). In patients with Duchenne muscular dystrophy, mitochondria do not work properly and produce toxic forms of oxygen that damage muscle cells. Idebenone is thought to help improve production of energy by restoring mitochondrial function, thereby preventing cellular damage and loss of muscular function seen in patients with the condition.

What did the company present to support its application?

The company provided results from a main study involving 64 patients with Duchenne muscular dystrophy who were not being treated with corticosteroids. Raxone was compared with placebo (a dummy treatment) and the main measure of effectiveness was the change in PEF (peak expiratory flow, the maximum speed a person can breathe out air) after one year of treatment. PEF is an indicator of breathing function.

What were the CHMP's main concerns that led to the refusal of the change to the marketing authorisation?

The CHMP was of the opinion that the study results provided by the company were insufficient to determine the benefit of Raxone in patients with Duchenne muscular dystrophy. Although a difference in PEF in favour of Raxone was observed, there was no clear improvement in other indicators of breathing function or in muscle strength, motor function or quality of life. The Committee also had some concerns about the way the study was conducted and analysed.

Therefore, the CHMP was of the opinion that the benefits of Raxone in patients with Duchenne muscular dystrophy did not outweigh its risks. Hence, the CHMP recommended that the change to the marketing authorisation be refused.

What consequences does this refusal have for patients in clinical trials or compassionate use programmes?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials or compassionate use programmes with Raxone.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, contact the doctor who is giving it to you.

What is happening with Raxone for treatment of Leber's hereditary optic neuropathy?

There are no consequences on the use of Raxone in its authorised indication.