16 December 2016
EMA/827291/2016
EMEA/H/C/004176

Questions and answers

Withdrawal of the marketing authorisation application for Kepnetic (aceneuramic acid)

On 10 November 2016, Ultragenyx UK Ltd. officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a marketing authorisation for Kepnetic, for the treatment of GNE myopathy.

What is Kepnetic?

Kepnetic is a medicine that contains the active substance aceneuramic acid. It was to be available as prolonged-release tablets (500 mg). Prolonged-release means that aceneuramic acid is released slowly from the tablet over a few hours.

What was Kepnetic expected to be used for?

Kepnetic was to be used to treat adults with GNE myopathy, an inherited muscle-wasting disease.

Kepnetic was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 5 March 2012 for hereditary inclusion body myopathy (the condition now called GNE myopathy). Further information on the orphan designation can be found here.

How does Kepnetic work?

Patients with GNE myopathy lack a substance known as sialic acid, needed for healthy muscles. This is because the gene to make sialic acid does not work properly.

The active substance in Kepnetic, aceneuramic acid, is a type of sialic acid. It was intended to replace the patient's sialic acid and by doing so allow their muscles to develop and function normally and relieve their symptoms.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What did the company present to support its application?

The company presented results of a main study of 47 adults with GNE myopathy comparing improvement in muscle strength in patients who received different doses of Kepnetic or placebo (a dummy treatment).

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the CHMP had evaluated the documentation provided by the company and formulated lists of questions. After the CHMP had assessed the company's responses to the last round of questions, there were still some unresolved issues.

What was the recommendation of the CHMP at that time?

Based on the review of the data at the time of the withdrawal, the CHMP had some concerns and was of the provisional opinion that Kepnetic could not have been approved for the treatment of GNE myopathy. The way the main study was designed meant that evidence to support the effectiveness and clinical usefulness of the medicine was insufficient. Therefore, at the time of the withdrawal, the CHMP was of the opinion that the benefits of Kepnetic did not outweigh its risks.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that it was withdrawing its application because of the CHMP view that the data presented did not provide enough evidence for approval. The company will collect additional data from an ongoing study to establish the effects of Kepnetic.

The withdrawal letter is available here.

What consequences does this withdrawal have for patients in clinical trials?

The company informed the CHMP that there are no consequences for patients currently included in clinical trials using Kepnetic. If you are in a clinical trial and need more information about your treatment, contact the doctor who is giving it to you.