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Questions and answers

Positive opinion on the marketing authorisation for Fampyra (fampridine)

Outcome of re-examination

On 19 May 2011, the Committee for Medicinal Products for Human Use (CHMP) recommended the granting of the marketing authorisation for the medicinal product Fampyra for use in improving the walking ability of adults with multiple sclerosis who have a walking disability. The company that applied for authorisation is Biogen Idec Ltd.

On 20 January 2011, the CHMP had originally adopted a negative opinion for Fampyra in patients with multiple sclerosis to improve their walking. At the request of the applicant, the CHMP started a re-examination of its opinion. Following the re-examination, the CHMP adopted a final positive opinion on 19 May 2011 recommending the granting of a marketing authorisation for Fampyra.

What is Fampyra?

Fampyra is a medicine that contains the active substance fampridine. It is to be available as prolonged-released tablets.

What is Fampyra to be used for?

Fampyra is to be used to improve walking ability in adults with multiple sclerosis who have a walking disability (4 to 7 points on the Expanded Disability Status Scale, EDSS).

Multiple sclerosis is a disease of the nerves, in which inflammation destroys the protective sheath around the nerves.

How does Fampyra work?

For the muscles to contract, electrical impulses have to be transmitted along the nerves to the muscles. This transmission of electrical impulses is impaired when the protective sheaths around the nerves are damaged.

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The active substance in Fampyra, fampridine, is a potassium channel blocker. It acts on damaged nerves, where it prevents charged potassium particles from leaving the nerve cells. This has the effect of allowing the electrical impulse to continue travelling along the nerves to stimulate the muscles.

What did the company present to support its application?

The effects of Fampyra were first tested in experimental models before being studied in humans. Because fampridine is well known as a potassium channel blocker, the company also used data from the scientific literature.

The company presented results of two main studies comparing Fampyra with placebo in 540 patients with multiple sclerosis. The patients were treated for nine or 14 weeks. The main measure of effectiveness was based on how fast they could walk along a 25-foot path (about 7.5 metres).

What were the CHMP's main concerns that led to the initial negative opinion?

At the time of the initial opinion, the CHMP was not convinced that Fampyra's small effect on the walking speed was a meaningful benefit for patients. The effect on speed could not be linked to meaningful improvements such as better coordination, balance or stamina or increased range of action.

The Committee was of the view that the medicine's uncertain benefits did not outweigh its side effects which included pain, dizziness, paraesthesia (unusual sensations like pins and needles) and problems with balance, as well as symptoms similar to those of multiple sclerosis that could impair the patient's ability to walk. The Committee also noted the lack of adequate long-term data on the medicine's benefits and safety as well as data on some groups of patients, such as the elderly and patients with epilepsy or heart problems.

Therefore, at that point in time, the CHMP concluded that the benefits of Fampyra did not outweigh its risks and recommended that it be refused marketing authorisation.

What happened during the re-examination?

During the re-examination, the Committee looked again at the data from the main studies. The CHMP also took advice from a group of experts specialising in neurology. A main focus of the re-examination was an investigation into what group of patients was more likely to benefit from Fampyra based on the available data.

What were the conclusions of the CHMP following the re-examination?

Following advice from the experts and discussions within the Committee, the Committee took the view that the medicine would be of benefit to patients who had a significant walking disability. Further recommendations were also included in the product information, explaining how doctors should evaluate treatment response and advising them to stop treatment if patients are not benefiting from it.

The Committee noted that serious side effects were rare and that, on the whole, the side effects reported with Fampyra should not preclude its use in patients who could benefit from it. The CHMP therefore concluded that the benefits of Fampyra outweigh its risks for patients with a walking disability and recommended that it be given marketing authorisation.

The Committee is recommending a conditional authorisation requiring the company to carry out a further study to find out more about the medicine's benefits and safety in the long term. In particular, the study will provide information on medicine's benefits beyond its effects on walking speed.

The summary of the positive opinion of the CHMP is published on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Pending EC decisions.