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Questions and answers

Positive opinion on the marketing authorisation for Ninlaro (ixazomib)

Outcome of re-examination

On 15 September 2016, the Committee for Medicinal Products for Human Use (CHMP) recommended the granting of a conditional marketing authorisation for the medicinal product Ninlaro for the treatment of multiple myeloma. The company that applied for authorisation is Takeda Pharma A/S.

On 26 May 2016, the CHMP had originally adopted a negative opinion for Ninlaro in multiple myeloma. 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 15 September 2016 recommending the authorisation of Ninlaro, conditional to the company providing further data to confirm the benefits.

What is Ninlaro?

Ninlaro is a cancer medicine that contains the active substance ixazomib. It is to be available as capsules.

What is Ninlaro to be used for?

Ninlaro is to be used for treating multiple myeloma (a cancer of the bone marrow) in adults who have received at least one prior treatment.

Ninlaro was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 27 September 2011, for the treatment of multiple myeloma. Further information can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

How does Ninlaro work?

The active substance in Ninlaro, ixazomib, is a proteasome inhibitor. This means that it blocks the proteasome, which is a system within cells that breaks down proteins when they are no longer needed. When the proteins in the cancer cells are not broken down, including the proteins that control cell growth, the cancer cells are damaged and they eventually die.

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What did the company present to support its application?

The effects of Ninlaro were first tested in experimental models before being studied in humans.

The company presented results from one main study involving 722 adults with multiple myeloma whose disease had not responded to or had come back after previous treatment. The study compared Ninlaro with placebo (a dummy treatment), both taken together with the medicines lenalidomide and dexamethasone. The main measure of effectiveness was progression-free survival (how long the patients lived without their disease getting worse).

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

The CHMP considered that the data from the main study were insufficient to demonstrate a benefit of Ninlaro in the treatment of multiple myeloma. The company had proposed restricting the use of the medicine to patients whose disease is more difficult to treat and had come back after one previous treatment, and to those whose disease had come back after at least two previous treatments. However, the data in these subgroups were not compelling enough and the rationale for assuming greater effectiveness in these patients was not clear.

Therefore, the CHMP was of the opinion that, based on the currently available data, the benefits of Ninlaro did not outweigh its risks and recommended that it be refused marketing authorisation.

What happened during the re-examination?

During the re-examination, the CHMP consulted a group of experts in cancer and considered, among other things, Ninlaro's safety profile, the fact that it is taken by mouth, and the possibility of the company providing further data on the benefits of the medicine.

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

The CHMP agreed with the expert group's conclusion that the available data from the main study indicate that Ninlaro improves patients' progression-free survival. As there is some uncertainty regarding the size of the improvement, further confirmatory data will need to be provided by the company as a condition for marketing authorisation. The Committee also acknowledged Ninlaro's favourable safety profile and the convenience of patients taking the capsules at home.

Therefore, the CHMP concluded that the benefits of Ninlaro outweigh its risks and recommended that Ninlaro be granted a conditional marketing authorisation.