EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Questions and answers

Update of 24 June 2016:

The company that applied for a marketing authorisation for Ninlaro has requested a re-examination of the CHMP's May 2016 opinion. Upon receipt of the grounds of the request, the CHMP will re-examine its opinion and issue a final recommendation.

Refusal of the marketing authorisation for Ninlaro (ixazomib)

On 26 May 2016, the Committee for Medicinal Products for Human Use (CHMP) adopted a negative opinion, recommending the refusal of the marketing authorisation for the medicinal product Ninlaro, intended for the treatment of multiple myeloma.

The company that applied for authorisation is Takeda Pharma A/S. It may request a re-examination of the opinion within 15 days of receipt of notification of this negative opinion.

What is Ninlaro?

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

What was Ninlaro expected to be used for?

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

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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 was Ninlaro expected to 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.

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 refusal?

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 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 Ninlaro.

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.