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# Withdrawal of application for a change to the marketing authorisation for Aranesp (darbepoetin alfa)

On 21 February 2018, Amgen Europe B.V. officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wished to withdraw its application to add treatment of anaemia in adult patients with myelodysplastic syndromes to the existing marketing authorisation.

## What is Aranesp?

Aranesp is a medicine already used to treat anaemia (low red-blood-cell count) that is causing symptoms in the following groups of patients:

- adults and children with 'chronic renal failure' (long-term, progressive decrease in the ability of the kidneys to work properly);
- adults who are receiving chemotherapy for 'non-myeloid' cancer (cancer not originating in the bone marrow).

Aranesp has been authorised since June 2001. It contains the active substance darbepoetin alfa.

Further information on Aranesp's current uses can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.

## What was Aranesp expected to be used for?

Aranesp was also expected to be used to treat anaemia in patients with myelodysplastic syndromes, a group of bone marrow disorders. The medicine was to be used in patients who did not need frequent blood transfusions and who had a low or intermediate risk of their condition progressing to acute myeloid leukaemia (AML, a type of cancer affecting white blood cells).

## How does Aranesp work?

In the treatment of anaemia in patients with myelodysplastic syndromes, Aranesp was expected to work in the same way as it does in its existing indications. Darbepoetin alfa, the active substance in

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

Aranesp, acts exactly like a natural hormone called erythropoietin that is made by the kidneys to stimulate red blood cell production, but it is very slightly different in its structure. This means that darbepoetin alfa has a longer duration of action, and can be given less often than natural erythropoietin. By acting in the same way as erythropoietin, Aranesp stimulates the body to make more red blood cells and so treats anaemia.

## What did the company present to support its application?

The applicant presented data from two main studies in 356 anaemic patients with myelodysplastic syndromes. The first study compared Aranesp with placebo (a dummy treatment) over 24 weeks. The main measure of effectiveness was a reduction in the number of transfusions of red blood cells. In the second study, all patients received Aranesp for 13 weeks and the study measured the level of haemoglobin, the main constituent of red blood cells, in the blood.

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

The application was withdrawn after the CHMP had evaluated the initial documentation provided by the company and formulated a list of questions. The company had not yet responded to the questions at the time of the withdrawal.

## 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 Aranesp could not have been approved for the treatment of anaemia in adult patients with myelodysplastic syndromes.

The CHMP was of the opinion that changes to the study design of one study and the exclusion of a high number of patients from the analysis of the results raise questions about the validity of the data. In addition, one study conducted in the United States was not in line with EU recommendations for treatment of patients with myelodysplastic syndromes.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the results of the study were not considered to be reliable and the Committee concluded that the change to the use of the medicine could not have been approved based on the data presented by the company.

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

In its letter notifying the Agency of the withdrawal of application, the company stated that the withdrawal is based on the CHMP's opinion that the data provided do not allow the Committee to authorise use in myelodysplastic syndromes.

The withdrawal letter is available here.

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

The company informed the Agency that there are no Amgen-sponsored ongoing clinical trials with Aranesp.

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

## What is happening with Aranesp in its authorised uses?

There are no consequences for the use of Aranesp in its authorised uses.