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# Withdrawal of the marketing authorisation application for Vynpenta (avacopan)

On 23 January 2019, ChemoCentryx officially notified the Committee for Medicinal Products for Human Use (CHMP) that it wishes to withdraw its application for a conditional marketing authorisation for Vynpenta (avacopan) for the treatment of the blood vessel disorders granulomatosis with polyangiitis and microscopic polyangiitis.

## What is Vynpenta?

Vynpenta is a medicine that contains the active substance avacopan. It was to be available as capsules to be taken by mouth.

## What was Vynpenta expected to be used for?

Vynpenta was to be used to control inflammation of blood vessels in adults with granulomatosis with polyangiitis (GPA) or microscopic polyangiitis (MPA). The medicine was expected to be used in combination with cyclophosphamide or rituximab.

Vynpenta was designated an 'orphan medicine' (a medicine to be used in rare diseases) on 19 November 2014 for these conditions. Further information on the orphan designations can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3141373 (granulomatosis with polyangiitis), ema.europa.eu/medicines/human/orphan-designations/eu3141372 (microscopic polyangiitis).

## How does Vynpenta work?

Vynpenta blocks the receptor (target) for a protein in the blood called complement 5a, which forms part of the immune system (the body's natural defences).

When C5a attaches to its receptor, it attracts and activates immune cells called neutrophils, which are thought to contribute to the inflammation of small blood vessels in granulomatosis with polyangiitis and microscopic polyangiitis. By blocking the receptor for C5a, Vynpenta was expected to reduce inflammation of blood vessels, thus improving the symptoms of the disease.

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

To support its conditional marketing authorisation application, the company presented the results from two studies involving a total of 109 patients. One of the studies compared Vynpenta with standard treatment including prednisone (a corticosteroid) while the other compared Vynpenta plus prednisone with prednisone. In both studies, the main measure of effectiveness was the proportion of patients who had at least 50% reduction in symptoms of blood vessels inflammation during 12 weeks of treatment, as measured using the Birmingham Vasculitis Activity Score (BVAS).

## 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. The company had not responded to the last round of questions at the time of the withdrawal.

## What was the recommendation of the CHMP at that time?

Based on the review of the data and the company's responses to the CHMP lists of questions, at the time of the withdrawal, the CHMP had some concerns and was of the provisional opinion that Vynpenta could not have been approved for the treatment of granulomatosis with polyangiitis and microscopic polyangiitis.

The CHMP found problems with the design of the studies and was concerned that the data available were not sufficient to show that Vynpenta is effective for treating these conditions. Although the studies suggested partial improvements in BVAS in more patients treated with Vynpenta than with standard treatment, this was not considered to be fully relevant because patients who experience only partial improvement in symptoms are at high risk of the disease coming back. The medicine did not seem to work any better than standard treatment in another measure: the proportion of patients who did not have any symptoms.

Regarding safety, the Committee noted that data on Vynpenta side effects were very limited. In addition there were concerns regarding the choice of the starting materials used to make the medicine.

Therefore, at the time of the withdrawal, the CHMP was of the opinion that the company had not provided enough data to support the application for Vynpenta.

## 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 had decided to focus its efforts on the future submission of a full marketing authorisation application given that further data from an ongoing study in over 300 patients receiving treatment for 52 weeks were soon to be available.

The withdrawal letter is available here.

## What consequences does this withdrawal have for patients in clinical trials and compassionate use programmes?

The company informed the CHMP that this withdrawal has no impact on ongoing clinical trials or compassionate use programmes with Vynpenta.

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.