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Refusal of the marketing authorisation for Rezurock (belumosudil)

The European Medicines Agency has recommended the refusal of the marketing authorisation for Rezurock, a medicine intended for the treatment of chronic graft-versus-host disease (a condition in which donor cells attack the body's organs) after a stem-cell transplant.

The Agency issued its opinion on 16 October 2025. The company that applied for authorisation, Sanofi Winthrop Industrie, may ask for re-examination of the opinion within 15 days of receiving it.

What is Rezurock and what was it intended to be used for?

Rezurock was developed as a medicine for treating chronic graft-versus-host disease in adults and adolescents from 12 years of age weighing at least 40 kg, after a blood stem-cell transplant. It was to be used when other treatments had not worked well enough or were not suitable.

Rezurock contains the active substance belumosudil and was to be available as tablets.

Rezurock was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 October 2019 for the treatment of graft-versus-host disease. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3192205

How does Rezurock work?

The active substance in Rezurock, belumosudil, stops the action of ROCK2, a protein involved in the immune reactions that take place in chronic graft-versus-host disease. By blocking the action of this protein, Rezurock was expected to help treat the disease and protect the body's organs from being attacked by donor cells.

What did the company present to support its application?

The company presented results from a main study involving 156 patients with chronic graft-versus-host disease who had tried at least two other treatments. In this study, patients took either 200 mg of Rezurock once a day or 200 mg twice a day and stopped taking the tablets if their disease got worse. Patients were also allowed to take other treatments, and Rezurock was not compared with any other treatment. This study looked at the proportion of patients who showed improvement in at least one organ without any new organ being affected.

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During the assessment, the company informed the Agency of results from an ongoing study of Rezurock used as a first treatment in patients with chronic graft-versus-host disease.

What were the main reasons for refusing the marketing authorisation?

The Agency noted that it was difficult to quantify the effect of Rezurock in patients with chronic graft-versus-host disease who had tried other treatments. The main study did not compare Rezurock with any other treatment, and patients also received other medicines.

The Agency also noted that the study in patients with chronic graft-versus-host disease who received Rezurock as a first treatment did not show that it had any beneficial effects. The study had to be stopped early because of the lack of evidence of a benefit, and the results cast further doubt about the use of Rezurock in patients who have had previous treatments.

Given the urgent need for new treatments for patients who have no suitable treatment options, the Agency considered a conditional marketing authorisation, which would have allowed the medicine to be authorised provided the company could submit further data within a short period of time. However, the company is not expected to provide further data on the effectiveness of Rezurock before April 2030.

Therefore, the Agency's opinion was that the benefits of Rezurock did not outweigh its risks, and it recommended refusing marketing authorisation.

Does this refusal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients currently participating in clinical trials or in compassionate use programmes with Rezurock.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your doctor.