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Withdrawal of application for the marketing authorisation of Fanskya (mozafancogene autotemcel)

Rocket Pharmaceuticals B.V. withdrew its application for a marketing authorisation of Fanskya for the treatment of Fanconi anaemia type A, an inherited condition that affects the bone marrow, the spongy tissue inside the large bones where blood cells are made.

The company withdrew the application on 11 August 2025.

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

Fanskya was developed as a medicine to be used in children aged from 1 to 18 years for the treatment of Fanconi anaemia type A.

In Fanconi anaemia type A, the bone marrow gradually loses its ability to produce enough healthy blood cells, leading to blood disorders such as anaemia (low levels of red blood cells) and an increased risk of infections and bleeding. People with the condition also have a higher risk of developing certain types of cancer, such as leukaemia (cancer of the white blood cells) and may experience problems affecting organs such as the kidneys and heart. There are several different subtypes of Fanconi anaemia; type A is the most common and is caused by mutations (changes) in the *FANCA* gene.

Fanskya contains the active substance mozafancogene autotemcel and was to be given as a single infusion (drip) into a vein.

Fanskya was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 December 2010 for Fanconi anaemia type A. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-10-822.

How does Fanskya work?

Fanconi anaemia type A is caused by mutations in the *FANCA* gene, which provides instructions for making a protein that helps to repair damaged DNA, particularly in cells that divide often such as those in the bone marrow.

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The active substance in Fanskya, mozafancogene autotemcel, was to be prepared using the patient's own blood stem cells (cells that can develop into different types of blood cells). These would then be modified in a laboratory using a virus. The virus is altered so that it cannot spread in the body, but it can deliver a healthy copy of the *FANCA* gene into the blood stem cells, allowing them to repair damaged DNA. The modified blood stem cells were to be given back to the patient via an infusion and were expected to travel to the bone marrow and make healthy blood cells.

What did the company present to support its application?

The company presented data from 3 studies involving 14 children aged from 1 to 7 years with Fanconi anaemia type A. The studies did not compare Fanskya with another medicine or placebo (a dummy treatment). The main measure of effectiveness included three different outcomes: the restoration of healthy blood cell production in the bone marrow, correction of genetic abnormalities in circulating blood cells and the attainment of normal levels of different blood cells after Fanskya is given.

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

The application was withdrawn after that the European Medicines Agency had evaluated the information from the company and prepared questions for the company. The company had not responded to the last round of questions at the time of the withdrawal.

What did the Agency recommend at that time?

Based on the review of the data, at the time of the withdrawal, the Agency had some concerns and its provisional opinion was that Fanskya could not have been authorised for the treatment of Fanconi anaemia type A.

The Agency had concerns about the potential safety of the medicine, as results showing how many copies of the *FANCA* gene enter stem cells are only available after treatment. This is important because introducing a high number of gene copies into stem cells may impact how they function. As the possible risk of the gene changing the normal functioning of cells could not be fully evaluated before the medicine had been given to the patient, the Agency requested a comprehensive risk assessment.

Although the company carried out several tests to assess the quality of Fanskya before it is given to patients, the Agency had concerns about whether the available data were sufficient to confirm that the tests reliably predicted the safety and effectiveness of the medicine. One of the key measures used to check the quality of the stem cells is the percentage of cells that carry a marker called CD34. However, the Agency had concerns regarding the thresholds of stem cells in Fanskya that had this marker.

Despite the lack of long-term safety data, Fanskya appeared to have an acceptable safety profile. However, the Agency concluded that company had not convincingly shown that the benefits of Fanskya outweigh its risks. This was mainly because the clinical benefit of the medicine had not been clearly established. Uncertainties in the main study, such as the lack of comparator and the young age of the participants, prevented the Agency from reaching robust conclusions at the time of withdrawal on whether Fanskya could have prevented bone marrow failure at a later age. The Agency also had concerns about the feasibility of the proposed measures to collect comprehensive post-marketing data to meet the criteria for a conditional marketing authorisation.

Therefore, at the time of the withdrawal, the Agency's opinion was that the company had not fully addressed its concerns and the benefit of Fanskya could not be established.

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 although it expected to satisfactorily address the concerns raised by the Agency, the application was withdrawn solely due to business reasons.

Does this withdrawal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials using Fanskya.

If you or your child is in a clinical trial and need more information about you or your child's treatment, speak with your clinical trial doctor.