swissmedic

Summary report on authorisation dated 26 September 2025

VANFLYTA® (active substance: quizartinib)

Authorisation in Switzerland: 31 March 2025

Film-coated tablets for the treatment of adults with newly diagnosed acute myeloid leukaemia (AML) that is FLT3-ITD-positive in combination with standard induction and consolidation chemotherapy followed by maintenance therapy.

About the medicinal product

VANFLYTA contains the active substance quizartinib and belongs to a group of medicinal products called protein kinase inhibitors, which intervene specifically in cancer cell growth.

VANFLYTA is used in adults with newly diagnosed acute myeloid leukaemia (AML) who have a particular genetic modification called an FLT3-ITD mutation. This type of AML progresses rapidly and is difficult to treat. Without treatment, it can often end fatally within a short period of time. AML is one of the commonest forms of leukaemia in adults.

Before using VANFLYTA, patients have to be tested to determine whether they have the FLT3-ITD mutation.

Treatment with VANFLYTA takes place in several phases:

- At first, VANFLYTA is administered in combination with standard chemotherapy consisting of cytarabine and an anthracycline (induction therapy).
- This is followed by further cytarabine chemotherapy (consolidation therapy).
- After that, patients take VANFLYTA on its own as maintenance therapy.

  VANFLYTA is not used for maintenance therapy following allogeneic haematopoietic stem cell transplantation.

Since AML is a rare, life-threatening disease, VANFLYTA has been authorised as an orphan drug. "Orphan drug" is a designation given to medicinal products for rare diseases.

VANFLYTA was authorised under Article 13 of the Therapeutic Products Act (TPA). This means that the medicinal product is already authorised in another country with comparable medicinal product control.

In this case, Swissmedic takes into consideration the results of checks carried out by foreign regulatory agencies, provided certain requirements are fulfilled. These involve checks on the quality, efficacy, and safety of the medicinal product, and the extent to which the results can be accepted for Switzerland.

The consideration of the results of foreign authorisation procedures is intended to help

ensure that medicines that are already authorised abroad can be made available to patients in Switzerland as quickly as possible.

In deciding whether to authorise VANFLYTA in Switzerland, Swissmedic considered the assessments and approval decisions of the European Medicines Agency (EMA) and the US regulatory agency FDA and has only conducted a limited scientific review.

Mode of action

VANFLYTA contains the active substance quizartinib, which acts specifically against cancer cells that exhibit a particular genetic mutation known as an FLT3-ITD mutation.

As a result of this mutation, the FLT3 protein (which is what is known as a tyrosine kinase) is constantly active. FLT3 normally regulates the growth and development of blood stem cells in the bone marrow. However, if the activity of the protein is disrupted as a result of the mutation, immature white blood cells grow and multiply unchecked, causing acute myeloid leukaemia (AML).

Quizartinib specifically blocks the misregulated FLT3 protein. This inhibits the growth of the leukaemia cells.

In combination with chemotherapy, VANFLYTA is thus able to significantly reduce the number of leukaemia cells.

Administration

VANFLYTA is available only on prescription as film-coated tablets.

Treatment with VANFLYTA takes place in several phases:

- Induction phase (introductory treatment): During this first phase of treatment, two 17.7 mg tablets (a total of 35.4 mg) are administered once daily for two weeks in each treatment cycle in combination with chemotherapy.
- Consolidation phase (stabilisation phase): The dosage remains the same: 35.4 mg once daily over certain periods, for two weeks in each treatment cycle in combination with chemotherapy.
- Maintenance therapy: Once the preceding treatment phases have been completed, maintenance therapy starts. At first, patients take 26.5 mg daily. If no serious adverse reactions occur, particularly an abnormal change in the QT interval[1], the dose can be increased to 53 mg daily (two 26.5 mg tablets).

Efficacy

The efficacy of VANFLYTA was investigated in the pivotal trial QuANTUM-First. This trial compared VANFLYTA in combination with standard chemotherapy in newly diagnosed FLT3-ITD-positive AML with placebo (a dummy drug) in combination with standard chemotherapy (control arm). A total of 539 adult patients aged between 18 and 75 years took part in the trial.

[1] QT interval: The QT interval is a measurement on an electrocardiogram (ECG) that represents the time it takes for the heart to electrically "recharge" after a heartbeat. A prolonged QT interval may increase the risk of dangerous abnormal heart rhythms.

The trial showed that patients who were treated with VANFLYTA achieved a significantly longer overall survival (OS)[2] than patients in the control arm. Median[3] overall survival was 31.9 months in the VANFLYTA group compared with 15.1 months in the control arm.

Additional sub-group analyses of overall survival (OS) were performed. No benefit to OS was observed in patients who received VANFLYTA as maintenance therapy after allogeneic, haematopoietic stem cell transplantation compared to the control arm (OS HR 1.62 [95% CI: 0.62, 4.22]).

Precautions, undesirable effects, & risks

VANFLYTA must not be used in those who are hypersensitive to the active substance or any of the excipients.

VANFLYTA may prolong the heart's QT interval,[4] which may cause severe abnormal heart rhythms and even cardiac arrest. Patients should have regular ECGs before and during treatment and have their blood potassium and magnesium levels checked. A specific boxed warning has been added to this effect.

In addition, the risk of deadly infections is increased, primarily in older patients and particularly during the initial phase of treatment. Patients should be closely monitored for severe infections during induction.

The most common undesirable effects (affecting more than 10% of patients) are infections, lymphocytopenia (low number of lymphocytes, a type of white blood cell), leukopenia (low white blood cell count), anaemia (low red blood cell count), thrombocytopenia (low blood platelet count), low blood potassium level, bleeding, neutropenia (low number of neutrophils, a type of white blood cell), nausea, fever, diarrhoea, low blood magnesium level, abnormal blood test results (elevated bilirubin in blood, abnormal liver enzymes), vomiting, stomach pain, stomatitis (sores in or around the mouth), oedema (swelling of the face, arms and legs), headaches, fatigue, rash, pneumonia, infections of the upper respiratory tract (nose and throat infections), reduced appetite, sepsis (serious infection), fungal infections and herpes infections.

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

Why the medicinal product has been authorised

The current treatment options available to adults with newly diagnosed, acute myeloid leukaemia (AML) with FLT3-ITD mutation are limited. The mutation is associated with a poorer prognosis. VANFLYTA addresses this need by specifically binding the receptor for the FLT3-ITD mutation and inhibiting its activity. The trial described above has demonstrated that treatment with VANFLYTA significantly improves overall survival in patients with maintenance therapy who have not previously had allogeneic, haematopoietic stem cell transplantation.

Taking all the risks and precautions into account, and based on the available data, the benefits of VANFLYTA outweigh the risks.

[2] Overall survival (OS): Overall survival refers to the period between the start of treatment and the death of the patient.

[3] Median: The value that lies exactly in the middle of a distribution of data is called the median or central value. One half is less than the median, while the other half is greater.

[4] QT interval: The QT interval is the time the heart needs to electrically "recharge" after a beat. A prolonged interval is therefore a change in the heart's electrical activity.

Swissmedic has therefore authorised the medicinal product VANFLYTA, containing the active substance quizartinib, in Switzerland for the treatment of adult patients with newly diagnosed FLT3-ITD-positve acute myeloid leukaemia.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals VANFLYTA®

Information for patients (package leaflet): Information for patients VANFLYTA®
Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.