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Summary report on authorisation dated 24.10.2025

Ayvakyt® (active substance: avapritinib)

Authorisation in Switzerland: 6 July 2023

Film-coated tablets for the treatment of adults with gastrointestinal stromal tumour (GIST) that has a specific mutation (PDGFRA-D842V) and of adults with advanced systematic mastocytosis

About the medicinal product

Ayvakyt contains the active substance avapritinib and is administered in the form of film-coated tablets.

Ayvakyt is used for the treatment of adults with a particular tumour of the gastrointestinal tract, known as a gastrointestinal stromal tumour (GIST). The tumour has a specific gene mutation (PDGFRA-D842V)[1] and is either so advanced that it cannot be removed by surgery (inoperable) or has already dispersed and spread to other parts of the body (metastasised).

Ayvakyt is also used for the treatment of advanced systemic mastocytosis (AdvSM)[2], provided that the patient has already received at least one other systemic therapy[3]. AdvSM includes patients with aggressive systemic mastocytosis (ASM), systemic mastocytosis associated with a haematological neoplasm (SM-AHN), and mast cell leukaemia (MCL).

Since these are rare, life-threatening diseases, the medicine has been authorised as an orphan drug. "Orphan drug" is a designation given to medicinal products for rare diseases.

Ayvakyt 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 en-

[1] PDGFRA-D842V: PDGFRA is the name of the affected gene, which controls cell growth. D842V indicates the precise location of the mutation within this gene.

[2] Advanced systemic mastocytosis (AdvSM): Disease in which too many mast cells build up in various organs, which may cause both severe allergic symptoms as well as organ damage.

[3] Systemic therapy: in contrast to local therapy (treatment at the site of the disorder), systemic therapy involves treatment of the entire body to eliminate a disorder.

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

In deciding whether to authorise Ayvakyt in Switzerland, Swissmedic accepted the assessment and approval decision of the US Food and Drug Administration (FDA) and has only conducted a limited scientific review.

Accordingly, in the SwissPAR (Swiss Public Assessment Report) and the resulting Summary report on authorisation, Swissmedic refers to the publicly available Assessment Report issued by the reference authority: www.fda.gov

Mode of action

Avapritinib, the active substance in Ayvakyt, acts against gastrointestinal stromal tumours (GIST) with the PDGFRA-D842V mutation. Avapritinib binds to and in doing so inhibits a mutated, overactive form of an enzyme [4] (PDGFRA). This mutated enzyme transmits continuous signals for cell growth and division, thereby promoting tumour growth. Targeted inhibition with avapritinib can reduce or stop the tumour growth.

Avapritinib also acts against advanced systemic mastocytosis (AdvSM). Here, too, it inhibits a mutated, overactive form of an enzyme (KIT) that contributes to abnormal cell proliferation. Through targeted inhibition, avapritinib can reduce or stop the mast cell growth and the associated symptoms.

Administration

Ayvakyt is a prescription-only medicine and is available as film-coated tablets to be taken orally. The recommended dose depends on the disease: For GIST, it is 300 mg once daily, and for AdvSM 200 mg once daily. These doses correspond to the maximum recommended daily doses and should not be exceeded. The tablets should be swallowed whole, without chewing, with a glass of water and on an empty stomach at least 2 hours before or 1 hour after a meal.

Ayvakyt should not be used to treat AdvSM if the platelet count is below 50 x 10^9/L.

Treatments should be initiated and monitored by a doctor with experience in the diagnosis and treatment of the relevant disease.

[4] Enzymes are proteins that act as biocatalysts, controlling and accelerating biochemical reactions in the body.

Efficacy

The efficacy of Ayvakyt against GIST with the specific PDGFRA-D842V mutation was investigated in the NAVIGATOR study, a study without control therapy. An objective response rate (ORR)[5] of 92.9% was achieved in the patients in the study. The efficacy of Ayvakyt against AdvSM was also investigated in the PATHFINDER study. In patients who had received at least one previous therapy, the objective response rate (ORR) was 51.1%. These studies confirm that Ayvakyt can reduce the disease burden in these rare types of cancer.

Precautions, undesirable effects, & risks

Ayvakyt must not be used in those who are hypersensitive to the active substance or any of the excipients. The medicinal product can increase the risk of severe reactions such as gastrointestinal and cerebral bleeding.

The most common adverse reactions of any grade during treatment of inoperable or metastasised GIST with Ayvakyt were anaemia (low red blood cell count) (54%), nausea (48%), fatigue (45%), diarrhoea (33%), periorbital oedema (swelling around the eyes) (32%), vomiting (28%), facial oedema (facial swelling) (28%), high blood levels of bilirubin[6] (28%), decreased appetite (27%), peripheral oedema (swelling of the hands, feet legs, or other limbs) (26%), increased lacrimation (watery eyes) (22%), and abdominal pain (22%).

The most common adverse reactions of any grade during treatment of AdvSM with Ayvakyt were peripheral oedema (43%), anaemia (40%), periorbital oedema (40%), thrombocytopenia (low blood platelet count) (40%), diarrhoea (28%), and nausea (24%).

If symptoms occur that indicate severe side effects such as severe headache, visual impairments, or confusion, stop treatment and consult a doctor immediately.

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

Only limited treatment options are available for patients with certain types of gastrointestinal stromal tumour (GIST) and advanced systemic mastocytosis (AdvSM). The studies show that Ayvakyt offers affected patients a promising option for halting disease progression.

Taking all the risks and precautions into account, and based on the available data, the benefits of Ayvakyt outweigh the risks. Swissmedic has therefore authorised the medicinal product Ayvakyt, containing the active substance avapritinib, for use in Switzerland.

Further information on the medicinal product

[5] Objective response rate (ORR): The objective response rate is defined as the percentage of patients with a clinically relevant reduction in tumour size

[6] Bilirubin: A yellow substance produced by the breakdown of red blood cells. Too much bilirubin can cause the skin and eyes to have a yellowish colour (jaundice).

Information for healthcare professionals: Information for healthcare professionals Ayvakyt®

Information for patients (package leaflet): Information for patients Ayvakyt®

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.