swissmedic

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Welireg® (active substance: belzutifan)

Temporary authorisation in Switzerland: 21 March 2024

Medicinal product (film-coated tablets) for monotherapy of adults with von Hippel-Lindau (VHL) disease who require therapy for a type of tumour associated with VHL disease and who do not require immediate surgery.

About the medicinal product

Von Hippel-Lindau (VHL) disease is a hereditary disorder. Patients with VHL disease often develop cancer. The most common types of tumour occur in the kidneys, brain and spinal cord, or pancreas. Kidney tumours, in particular, are malignant and may form metastases, which can be life-threatening for patients.

The medicinal product Welireg, containing the active substance belzutifan, is used to treat adults with VHL disease who require therapy for VHL-associated tumours of the kidneys, known as renal cell carcinomas, the brain and spinal cord, known as haemangioblastomas, the central nervous system, or the pancreas, known as pancreatic neuroendocrine tumours, and do not require immediate surgery.

Since VHL disease is a rare, life-threatening disease, Welireg has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Welireg 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 the foreign regulatory agency, provided certain requirements are fulfilled. 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 Welireg in Switzerland, Swissmedic has accepted parts of the assessment and approval decision of the British authority (MHRA) and the U.S. Food and Drug Administration (FDA).

Mode of action

The active substance belzutifan is an inhibitor of so-called hypoxia-inducible factor 2 alpha (HIF-2α)[1]. Belzutifan binds to HIF-2α and in this way blocks regulatory mechanisms in specific genes capable of triggering the formation of tumours. This mechanism of action enables tumour growth to be slowed or stopped in patients with VHL disease.

Administration

Welireg, containing the active substance belzutifan, is a prescription-only medicine.

It is available as film-coated tablets in the dosage strength of 40 mg. The recommended dose is 120 mg (3 film-coated tablets of 40 mg) daily. The film-coated tablets are not to be chewed and can be taken with or without food.

Treatment with Welireg is initiated and monitored by a healthcare professional with experience in the administration of cancer treatments.

The doctor will advise the patient to continue the therapy until the disease progresses or until serious side effects occur.

Women of child-bearing age should use a highly effective method of contraception during treatment with Welireg and for at least 1 week after the last dose as the active substance belzutifan can harm the unborn child.

Efficacy

The efficacy of belzutifan was studied in an open clinical Phase 2 trial with a total of 61 male and female patients with VHL disease.

The trial subjects had at least one kidney tumour not requiring immediate surgery. The patients enrolled in the study also had other VHL-associated tumours. None of the tumours had formed metastases.

The subjects were given 120 mg belzutifan once daily for at least 3 years.

The study showed a decrease in the size of the VHL-associated tumours in 64% of those treated. The study also showed that the VHL-associated tumours had not progressed after 36 months in 86.3% of the trial subjects.

Precautions, undesirable effects, & risks

Welireg must not be used in those who are hypersensitive to the active substance or any of the excipients. The most common undesirable effects at the time the authorisation decision was taken were anaemia (83.2%), fatigue and exhaustion (42.7%), nausea (24.1%), breathlessness or shortness of breath (21.4%), dizziness (17.9%) and impaired oxygen supply to the body (16.3%).

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

[1]Hypoxia-inducible factor 2 alpha: A regulatory protein capable of triggering specific mechanisms in genes by binding to regions in their DNA.

Why the medicinal product has been authorised

There is currently no medicinal treatment option available for patients with VHL disease whose tumours do not require immediate surgery.

The clinical trial showed a reduction in the size of VHL-associated tumours in approx. 64% of those treated and that tumour progression was able to be slowed in the majority of patients.

Treatment of these patients with Welireg is therefore promising. However, additional data are required to confirm the results.

The medicinal product Welireg was authorised temporarily in Switzerland (in accordance with Art. 9a TPA) since not all clinical trials were available or had been concluded at the time of authorisation.

The temporary authorisation is contingent on the timely submission of the data requested by Swissmedic. Once these authorisation conditions have been met, the temporary authorisation can be converted into an authorisation without special conditions in the event of a positive benefit-risk assessment of the results.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals Welireg®

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

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 Public Summary SwissPAR.

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.