



EUROPEAN MEDICINES AGENCY
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Media and Public Relations

Press release

First 'histology-independent' treatment for solid tumours with a specific gene mutation

EMA's human medicines committee (CHMP) has recommended granting a marketing authorisation in the European Union for Vitrakvi (larotrectinib) for the treatment of adult and paediatric patients with solid tumours that display a Neurotrophic Tyrosine Receptor Kinase (NTRK) gene fusion. Treatment with Vitrakvi is recommended for patients whose disease has spread or cannot be surgically removed, and who have no other satisfactory treatment options.

Vitrakvi is the first so-called 'histology-independent' cancer treatment recommended for approval in the EU. This means that it can be used to treat non-haematological (i.e. that do not begin in the blood or bone marrow) tumours with this specific mutation, regardless of where in the body the tumour originated. Before patients can be started on the medicine, the presence of the mutation in the tumour should be confirmed by a validated test.

The active substance in Vitrakvi – larotrectinib - targets a very specific genomic alteration of a patient's tumour. This occurs when NTRK genes that encode specific proteins are abnormally fused to a gene. This mutation, called NTRK gene fusion, leads to the development of proteins that can cause cancer cells to grow. Vitrakvi blocks the action of these proteins and in doing so inhibits the growth of the cancer.

NTRK gene fusions can be observed very frequently in a certain number of rare cancer types that affect both adults and children. In addition, this gene fusion occurs rarely in some of the most common cancer types.

The efficacy and safety of Vitrakvi were studied in three single-arm trials (i.e. studies with no control group) that included a total of 102 adults and children with cancer that were evaluated. These patients had either already received standard therapy, or would have had to undergo disfiguring surgery, or were unlikely to respond to available therapies.

The share of patients who responded to treatment with Vitrakvi was 67%. Of those, the response lasted six months or longer in 88% and 12 months or longer in 75%. Tumour responses were seen both in rare tumour types such as infantile fibrosarcoma and salivary gland tumours, as well as in common diseases such as lung and colon cancer.

The most common side effects were tiredness, increased levels of liver enzymes, dizziness, constipation, nausea, anaemia (low red blood cell count), and vomiting.



The CHMP recommended a conditional approval for this medicine. This is one of the EU's regulatory mechanisms to facilitate early access to medicines that fulfil an unmet medical need. This type of approval allows the Agency to recommend a medicine for marketing authorisation with less complete data than normally expected, in cases where the benefit of a medicine's immediate availability to patients outweighs the risk inherent in the fact that not all the data are yet available.

The opinion adopted by the CHMP is an intermediary step on Vitrakvi's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once the marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Vitrakvi is Bayer AG.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

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