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Press Office

Press release

First medicine for rare blood cancer

EMA recommends Imbruvica for the treatment of Waldenström's macroglobulinaemia

The European Medicines Agency (EMA) has recommended extending the use of Imbruvica (ibrutinib) to include the treatment of patients with Waldenström's macroglobulinaemia, a rare blood cell cancer. Imbruvica is the first medicine that is recommended for this disease.

The medicine is indicated for adults who have received at least one prior therapy or as a first line treatment for patients unsuitable for chemo-immunotherapy.

Imbruvica was first authorised in the European Union in October 2014 for the treatment of two other types of blood cancer: chronic lymphocytic leukaemia and mantle cell lymphoma.

Waldenström's macroglobulinaemia, a type of non-Hodgkin lymphoma, is characterised by an excess of abnormal white blood cells, called B lymphocytes and plasma cells, in the bone marrow and sometimes in other organs. These abnormal cells produce large amounts of an immunoglobulin called IgM, which can make the blood thicker than normal. This cancer usually begins in people over 60 years of age. Five years after diagnosis, between 36% and 87% of patients are still alive, depending on their individual risk factors.

Imbruvica represents a novel strategy in the treatment of malignancies involving B lymphocytes. The active substance contained in Imbruvica, ibrutinib, works by blocking an enzyme called Bruton's tyrosine kinase (Btk), which has a key role in the survival of B lymphocytes and their migration to the organs where these cells normally divide. By blocking Btk, ibrutinib decreases survival and migration of B lymphocytes, thereby delaying the progression of the cancer.

The recommendation from EMA's Committee for Medicinal Products for Human Use (CHMP) is based on the results of a phase 2 study in 63 patients with previously treated Waldenström's macroglobulinaemia. Around 90% of the patients treated with Imbruvica responded positively to the treatment and approximately 80% of patients were alive without disease progression after 18 months.

The adverse events reported during the clinical trial were similar to those observed in the already approved indications of Imbruvica. They include events affecting the blood and bone marrow such as neutropenia and thrombocytopenia.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

Because Waldenström's macroglobulinaemia (also known as lymphoplasmacytic lymphoma) is rare, Imbruvica received an orphan designation for this indication from the Committee for Orphan Medicinal Products (COMP) in 2014. Orphan designation and the associated incentives such as fee reductions for scientific advice are among the Agency's most important instruments to encourage the development of medicines for patients with rare diseases.

The opinion adopted by the CHMP at its May 2015 meeting is an intermediary step on Imbruvica's access to patients with Waldenström's macroglobulinaemia. The CHMP opinion will now be sent to the European Commission for the adoption of a decision to change the marketing authorisation. Once the extension of indication has been granted, each Member State will take a decision on price and reimbursement based on the potential role/use of this medicine in the context of its national health system.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Imbruvica is Janssen-Cilag International NV.
3. Following this positive CHMP opinion, the Committee for Orphan Medicinal Products (COMP) will assess whether the designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu