HAUTE AUTORITÉ DE SANTÉ

ONCOLOGY
New indication
November 2014

BRIEF SUMMARY OF THE TRANSPARENCY COMMITTEE OPINION

REVLIMID (lenalidomide), immunosuppressive drug

Moderate improvement in the treatment of low-risk myelodysplastic syndromes with isolated 5q deletion when other therapeutic options are inadequate or inappropriate

Main points

- REVLIMID now has Marketing Authorisation in the treatment of patients with transfusion-dependent anaemia due to a low-risk or intermediate-1-risk myelodysplastic syndrome (MDS) associated with an isolated 5q deletion.
- In these patients, two studies have demonstrated the efficacy of lenalidomide in respect of transfusional independence, the increase in the haemoglobin level and the cytogenetic response, while its safety profile is comparable with that known for patients with myeloma.

Pre-existing indications

REVLIMID already has Marketing Authorizations in combination with dexamethasone, for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.

This summary does not cover this indication.

Therapeutic use

Treatment of low-risk MDS with the intention of correcting the cytopenias (predominantly anaemia):
- When the cytopenias are moderate or asymptomatic, no treatment is given;
- When the anaemia becomes symptomatic, it is treated with erythropoietin;
- After failure of erythropoietin and in the absence of a treatment alternative, transfusions of erythrocyte concentrates are used.

- **Role of the medicinal product in the therapeutic strategy**
  REVLIMID is an alternative to transfusion in the treatment of patients with transfusion-dependent anaemia due to low-risk MDS with isolated 5q deletion if other therapeutic options are inadequate or inappropriate.

Clinical data

- In an open, non-controlled phase II study, the percentage of patients who were transfusion-independent for at least 2 months after treatment with lenalidomide at 10 mg/day was 65.5% (95% CI: [57.3; 73.2]). The median period until transfusional independence was achieved was 4.1 weeks. The median duration of transfusional independence was about 4 months. The median increase in the level of haemoglobin was 5.6 g/dl. A cytogenetic response (major or minor) was observed in 71.6% of patients.
- In a double-blind study versus placebo, the percentage of patients who were transfusion-independent for at least 6 months was significantly higher under 10 mg/day of lenalidomide than under placebo (55.1% versus 6.0%, $p < 0.001$). In the lenalidomide 10 mg group, the median period until transfusional independence was achieved was 4.3 weeks. The median increase in the level of haemoglobin was 6.4 g/dl. A major cytogenetic response was observed in 30% of patients.
- The safety profile was comparable with that already known for patients with myeloma. The majority of adverse effects occurred in the course of the first 16 weeks of treatment. The main serious adverse effects were neutropenia, thrombocytopenia and venous thromboembolic events (deep vein thrombosis, pulmonary embolism).

- The main adverse events that merit special attention in patients with MDS are cytopenia, malignant secondary tumours, thromboembolic events, skin reactions and hepatotoxicity.

Benefit of the medicinal product

- The actual benefit* of REVLIMID is substantial.
- REVLIMID provides a moderate clinical added value** (CAV III) in terms of its efficacy in the care of patients with transfusion-dependent anaemia due to low-risk MDS with isolated 5q deletion when other therapeutic options are inadequate or inappropriate.
- The Transparency Committee recommends inclusion on the list of reimbursable products for hospital use.

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This document was created on the basis of the Transparency Committee Opinion of 19 November 2014 (CT-13681 / CT-13682) and is available at **www.has-sante.fr**

[i] * The actual benefit (AB) of a proprietary medicinal product describes its benefit primarily in terms of its clinical efficacy and the seriousness of the condition being treated. The HAS Transparency Committee assesses the AB, which can be substantial, moderate, low or insufficient for reimbursement for hospital use.

** The clinical added value (CAV) describes the improvement in treatment provided by a medicinal product compared with existing treatments. The HAS Transparency Committee assesses the degree of CAV on a scale from I (major) to IV (minor). A level V CAV means “no clinical added value”.