Original French opinion adopted by the Transparency Committee

The legally binding text is the original French opinion version

SUMMARY

melphalan flufenamide

PEPAXTI 20 mg

powder for concentrate for solution for infusion

Inclusion on list

Adopted by the Transparency Committee on 12 February 2025

Summary of opinion

Unfavourable opinion for reimbursement in the indication "in combination with dexamethasone, for the treatment of adult patients with multiple myeloma who have received at least three prior lines of therapies, whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one anti-CD38 monoclonal antibody, and who have demonstrated disease progression on or after the last therapy. For patients with a prior autologous stem cell transplantation, the time to progression should be at least 3 years from transplantation (see section 4.4 of the SPC)."

Transparency Committee's Conclusions

Clinical Benefit

- Multiple myeloma is a serious blood disease that is almost always fatal, with a short median survival time (3 to 5 years).
- This is a curative medicinal product.
- The efficacy/adverse effects ratio has not been adequately established given the absence of robust data in the patients covered by the MA and a safety profile marked, in particular by haematological toxicity.
- On the basis of currently available data, PEPAXTI (melphalan flufenamide) has no role in the current care pathway.

Public health benefit

Considering:

- the seriousness of the disease, which is life-threatening in the short term, and its prevalence,
- the partially met medical need,
- the lack of response to the identified need considering:
  - evidence of an impact on morbidity, but in a population not transposable to that of the MA,
  - the lack of evidence of an impact on quality of life,
  - the lack of evidence of an impact on the organisation of care,

PEPAXTI (melphalan flufenamide) is unlikely to have an additional impact on public health.

Considering all of these elements, the Committee deems that the clinical benefit of PEPAXTI (melphalan flufenamide) 20 mg powder for concentrate for solution for infusion is insufficient to justify public funding in the MA indication.

The Committee issues an unfavourable opinion for inclusion of PEPAXTI (melphalan flufenamide) 20 mg powder for concentrate for solution for infusion in the list of covered drugs for inpatients approved for use in the MA indication and at the MA dosages.