SUMMARY

pegcetacoplan

ASPAVELI 1 080 mg,

solution for infusion

Indication extension

Adopted by the Transparency Committee on 9 October 2024

Summary of opinion

Unfavourable opinion for reimbursement in the indication extension "ASPAVELI is indicated in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not had previous treatment with a complement inhibitor."

Transparency Committee's conclusions

Clinical Benefit

- Paroxysmal nocturnal haemoglobinuria (PNH) is a rare (orphan disease) and serious condition caused by an acquired clonal mutation affecting all the haematopoietic cell lines. The disease is polymorphous but is usually manifested (90% of patients) by haemolysis due to the sensitivity of red blood cells to the haemolytic action of the complement. Episodes of haemolysis with haemoglobinuria occur primarily at night, as a result of a reduction in blood pH. The disease course is variable and can be life-threatening due to its complications: thromboembolic events (responsible for 40 to 67% of deaths), haemorrhages, infections, kidney and bone marrow failure.
- The proprietary medicinal product ASPAVELI (pegcetacoplan) is a preventive medication.
- The efficacy/adverse effects ratio of ASPAVELI (pegcetacoplan) has not been adequately established in the treatment of adult patients with PNH who have haemolytic anaemia and have not had previous treatment with a complement inhibitor, in the absence of robust comparative data versus the currently recommended reference treatment in this population (C5 complement inhibitor), in a context in which a direct comparative study would have been feasible, meaning that it is not possible to exclude the possibility of a loss of opportunity for patients treated with pegcetacoplan rather than a C5 complement inhibitor.
- There are currently medicinal therapeutic alternatives: SOLIRIS (eculizumab) and ULTOMIRIS (ravulizumab) (C5 complement inhibitors).
- ASPAVELI (pegcetacoplan) has no role in the care pathway for the treatment of adult patients with PNH who have not had previous treatment with a complement inhibitor.

➔ Public health benefit

Considering:

- the seriousness of the disease, which is life-threatening in the event of complications, and its low prevalence,
- the partially met medical need,
- the lack of response to the identified need given:
  - the lack of evidence of an additional impact on morbidity and mortality or on quality of life compared to the current reference treatment using a C5 inhibitor,
  - the lack of evidence of an impact on the organisation of care and the patient's care pathway compared to the current reference treatment using a C5 inhibitor,

ASPAVELI (pegcetacoplan) is unlikely to have an additional impact on public health.

Considering all of these elements, the Committee deems that the clinical benefit of ASPAVELI 1,080 mg (pegcetacoplan) solution for infusion is insufficient in the indication extension "treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not had previous treatment with a complement inhibitor" to justify public funding in view of the available alternatives.

The Committee issues a unfavourable opinion for inclusion of ASPAVELI 1,080 mg (pegcetacoplan) solution for infusion in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the indication "treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia and have not had previous treatment with a complement inhibitor".