SUMMARY

eculizumab

SOLIRIS 300 mg

concentrate for solution for infusion

Indication extension

Adopted by the Transparency Committee on 24 April 2024

Summary of opinion

Unfavourable opinion for reimbursement in the treatment of refractory generalised myasthenia gravis (gMG) in patients aged 6 years and above who are antiacetylcholine receptor (AChR) antibody-positive.

Transparency Committee's conclusions

Clinical Benefit

- Myasthenia is an incapacitating, rare, serious autoimmune disease causing fluctuating muscle weakness and excessive fatigability, which can impair quality of life and be life-threatening.
- This is a curative background therapy.
- Considering:
  - efficacy data from a non-comparative study in children and adolescents having reported short-term results (26 weeks) and the limitations associated with this study (absence of control group, small sample size (n=12), absence of data in the 6-12 years age group, uncertainty with respect to transposability of the data to French practice);
  - data in adults, and indication for which funding has currently not been requested by the pharmaceutical company, based on a non statistically significant comparative study versus placebo on the primary endpoint;
  - the safety profile, with limited follow-up in this indication, consistent with that already known in the other indications currently funded;

  the efficacy/adverse effects ratio has not been adequately established in the treatment of refractory generalised myasthenia gravis (gMG) in patients aged 6 years and above who are antiacetylcholine receptor (AChR) antibody-positive.
- The role of SOLIRIS (eculizumab) in the treatment of refractory generalised myasthenia gravis (gMG) in patients aged 6 years and above who are antiacetylcholine receptor (AChR) antibody-positive cannot be determined.

➔ Public health benefit

Considering:

- the seriousness of the disease and its low prevalence,
- the partially met medical need in this paediatric indication,
- the lack of response to the identified need given:
  - a lack of evidence of an additional impact on morbidity and mortality,
  - the lack of evidence of an additional impact on quality of life, in the absence of robust data,
  - the lack of evidence of an additional impact on the organisation of care and the patient's care and life pathway,

SOLIRIS (eculizumab) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of SOLIRIS (eculizumab) is insufficient to justify public funding in the treatment of refractory generalised myasthenia gravis (gMG) in patients aged 6 years and above who are antiacetylcholine receptor (AChR) antibody-positive.

The Committee issues an unfavourable opinion for inclusion of SOLIRIS (eculizumab) in the list of covered drugs for inpatients approved for use in the treatment of refractory generalised myasthenia gravis (gMG) in patients aged 6 years and above who are antiacetylcholine receptor (AChR) antibody-positive.