

SUMMARY

omaveloxolone

SKYCLARYS 50 mg

hard capsules

First listing

Adopted by the Transparency Committee on 11 September 2024

Summary of opinion

Favourable opinion for reimbursement in “the treatment of Friedreich’s ataxia in adults and adolescents aged 16 years and older”

Transparency Committee’s conclusions

Clinical Benefit

- Friedreich’s ataxia is a serious and disabling rare neurogenetic disease. The manifestations of the disease have a significant impact on all aspects of daily life, and on the family, social and work lives of patients.
- This is a symptomatic treatment that aims to slow the progression of the disease.
- The efficacy/adverse effects ratio appears to be limited in the short term and remains to be specified in the longer term in view of the low level of evidence and/or the lack of data on progression of morbidity associated with the disease, quality of life and patient survival.
- This is a first-line treatment in the absence of a clinically relevant comparator.

→ Public health benefit

Considering:

- the major medical need to have access to effective, well tolerated medicines in these patients,
- the lack of response to the identified medical need due to:
 - uncertainties concerning the impact of the medicinal product in terms of reduction of morbidity in the short term, in view of the results versus placebo on the mFARS neurological assessment score after 48 weeks of treatment,
 - the absence of data supporting an impact on quality of life, long-term morbidity and/or patient survival,

- the absence of data supporting an additional impact on the organisation of care or the care and/or life pathway of patients and their families; a negative impact (risks of drug-induced liver injury and/or congestive heart failure) cannot be excluded,

SKYCLARYS 50 mg (omaveloxolone) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of SKYCLARYS 50 mg (omaveloxolone) hard capsules is moderate in the MA indication.

The Committee issues a favourable opinion for inclusion of SKYCLARYS 50 mg (omaveloxolone) hard capsules in both the list of covered drugs for outpatients and the list of covered drugs for inpatients approved for use in the indication and at the MA dosages.

→ **Recommended reimbursement rate for inclusion in the list of covered drugs for outpatients: 35%**

Clinical Added Value

Considering:

- reservations with respect to the clinical relevance of the effect observed versus placebo for the mFARS neurological score in patients with “pes cavus” in the MOXle study part 2 (-2.4 points, CI_{95%} [-4.31; -0.50]) and the absence of evidence of a difference versus placebo for the ranked secondary endpoints,
- uncertainties with respect to the transposability of the results to all patients with Friedreich’s ataxia, in particular to patients with advanced cardiomyopathy, uncontrolled diabetes or an advanced form of the disease,
- reservations with respect to the level of evidence and interpretation of the results derived from indirect comparison data,
- the frequency of adverse effects reported and evidence of risks of liver injury and congestive heart failure,
- and despite the major medical need to have access to effective, well tolerated medicines in these patients,

the Committee deems that SKYCLARYS 50 mg (omaveloxolone) hard capsules provides no clinical added value (CAV V) in the care pathway for Friedreich’s ataxia.