INAHTA Brief

Issue 2020/005

Title ONPATTRO – A Health Technology Assessment

Agency HAS, French National Authority for Health (Haute Autorité de santé)
2 avenue du Stade de France – F 93218 La Plaine Cedex, France
Tel: +33 (0)1 55 93 70 00 – Fax: +33 (0)1 55 93 74 35, contact.sem@has-sante.fr, www.has-sante.fr

Reference link to full report in French
hhttps://www.has-sante.fr/jcms/pprd_2982827/fr/onpattro

Aim

Assessment of ONPATTRO (patisiran) with a view to funding by the French national health insurance system and of its clinical contribution compared to other strategies in the treatment of hereditary attr amyloidosis (hATTR amyloidosis), in adult patients with stage 1 or stage 2 polyneuropathy.

Conclusions of Transparency Committee

- Hereditary attr amyloidosis with stage 1 or 2 polyneuropathy is a rare, serious, debilitating disease with fatal outcome.
- This is treatment to prevent the formation of new amyloid deposits.
- The efficacy/adverse effects ratio is high, with an improvement in the mNIS+7 score (polyneuropathy disability criterion) being demonstrated in the patisiran group, compared to a deterioration in the placebo group, but without data on a reduction in morbidity-mortality, and taking the acceptable safety profile into consideration.
- There are therapeutic alternatives.
- The drug ONPATTRO (patisiran) is a first-line treatment for hereditary attr amyloidosis, in adult patients with stage 1 or stage 2 polyneuropathy.
- ONPATTRO is likely to have a public health impact.

Given these elements, the Committee considers that the clinical benefit of ONPATTRO is substantial in the MA indication.

Considering:
- demonstration of the superiority of patisiran over the placebo in a phase 3 study, in patients with hereditary attr amyloidosis and stage 1 or stage 2 polyneuropathy,
- with an additional quantity-effect compared to the placebo demonstrated on the variation in the mNIS+7 score, primary endpoint on neuropathy-induced disability, with an improvement in the score for 56% of patients in the patisiran group compared to 4% in the placebo group, which is considered to be clinically relevant,
- the positive impact of patisiran demonstrated on quality-of-life by the NORFOLK-QoL-DN questionnaire, used as hierarchical secondary endpoint,
- the short-term safety profile considered to be acceptable,
- the very partially met medical need in stage 1 polyneuropathy, and the unmet medical need in stage 2 polyneuropathy,

ONPATTRO provides a moderate clinical added value (CAV III) in the therapeutic strategy for hereditary attr amyloidosis in adult patients with stage 1 or 2 polyneuropathy.

Recommendations

The Transparency Committee issued its approval for the funding of ONPATTRO by the French national health insurance system in the MA indication.

Methods

The assessment of ONPATTRO was founded on evidence-based medicine with a critical analysis of the clinical data.

Written by

HAS (Haute Autorité de santé), French National Authority for Health