HAUTE AUTORITÉ DE SANTÉ

TRANSPARENCY COMMITTEE
SUMMARY
09 SEPTEMBER 2020
The legally binding text is the original French opinion version

ustekinumab

STELARA 45 mg solution for injection and solution for injection in pre-filled syringe
STELARA 90 mg solution for injection in pre-filled syringe

New indication

Key points

Favourable opinion for reimbursement only in the treatment of severe chronic plaque psoriasis in children aged 6 to 11 years, defined by :

- failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy
- and an extensive form and/or significant psychosocial impact.

What therapeutic improvement ?

No clinical added value in the therapeutic strategy.

Role in the care pathway ?

In the paediatric population, treatment involves topical treatments (dermocorticosteroids and vitamin D3 analogues) and, in moderate to severe forms resistant to topical treatments, non-biological systemic treatments (retinoids, ciclosporin, methotrexate) and light therapy. In the event of failure of these second-line treatments, TNFα antagonists (etanercept and adalimumab) or ustekinumab are used.

Etanercept (ENBREL) has a marketing authorisation in plaque psoriasis in children from 6 years of age and adalimumab (HUMIRA) from 4 years of age. Until now, ustekinumab (STELARA) only had a marketing authorisation in adolescents from 12 years of age.

These treatments do not definitively cure the condition but can enable a transient and more or less complete disappearance of the lesions, and the care pathway is “rotational”, due, in particular to resistance phenomena.

Treatment in children is not very different to that in adults, but there are fewer validated treatments in children. For example, calcitriol is the only vitamin D3 analogue to have a marketing authorisation in children and methotrexate only has a marketing authorisation in adults.

Furthermore, the toxicity of these treatments limits their use over time and light therapy is not recommended in children.

As in adults and adolescents, the Commission recommends the use of biological medicinal products as second-line systemic therapy in the treatment of severe chronic paediatric plaque psoriasis, defined by :
- failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy
- and an extensive form and/or significant psychosocial impact.

Role of the medicinal product in the care pathway

In children aged 6 to 11 years, STELARA (ustekinumab) is a second-line systemic therapy in the treatment of severe chronic paediatric plaque psoriasis, defined by :
- failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy
- and an extensive form and/or significant psychosocial impact.

Special recommendations

Given the risk of hypersensitivity reactions with ustekinumab administered subcutaneously (see paragraph 4.4 of the SPC), but also with other biologics, the Transparency Committee recommends that the first subcutaneous injection of this drug be given in an appropriate care structure.

COMMITTEE'S CONCLUSIONS

Considering all of this information and further to debate and voting, the Committee considers :

Clinical benefit

◗ Psoriasis is a chronic inflammatory skin condition, usually benign, that can, in its moderate to severe forms, have a significant impact on quality of life.
◗ The proprietary medicinal products STELARA 45 mg and 90 mg (ustekinumab) have a suspensive effect on symptoms.
◗ Given the limited clinical data in children, based mainly on descriptive efficacy, safety and pharmacokinetic data but supplemented by clinical and pharmacokinetic data in adults and adolescents, the efficacy/adverse effects ratio is high.
◗ In children aged 6 to 11 years, these medicinal products are second-line systemic therapies in the treatment of severe chronic plaque psoriasis, defined by :
- failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy
- and an extensive form and/or significant psychosocial impact.

◗ There are few therapeutic alternatives in children (etanercept and adalimumab).

Public health impact

Considering :
- the seriousness of the disease and the marked impairment in quality of life,
- its low prevalence in children,
- the partially met medical need in this population in the event of failure of non-biological systemic treatments and light therapy,
- the absence of any demonstrated impact on the organisation of care,
- the absence of robust data on quality of life,
- the partial response to the need in view of the limited clinical data in children, based mainly on descriptive efficacy, safety and pharmacokinetic data but supplemented by clinical and pharmacokinetic data in adults and adolescents,

STELARA is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of the proprietary medicinal products :
- **STELARA 45 mg (ustekinumab) solution for injection and solution for injection in pre-filled syringe and**
- **STELARA 90 mg (ustekinumab) solution for injection in pre-filled syringe,**

is substantial only in the treatment of severe chronic plaque psoriasis in children aged 6 to 11 years, defined by :
- **failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy**
- **and an extensive form and/or significant psychosocial impact.**

The Committee issues a favourable opinion for inclusion in both the hospital formulary list and the retail formulary list of reimbursed proprietary medicinal products approved for use only in the treatment of severe chronic plaque psoriasis in children aged 6 to 11 years, defined by :
- **failure (insufficient response, contraindication or intolerance) of at least two treatments including non-biological systemic treatments and light therapy**
- **an extensive form and/or significant psychosocial impact, and at the MA dosages.**

◗ **Recommended reimbursement rate : 65 %**

Clinical Added Value

Considering :

- **the limited clinical data in children, based mainly on the results of a non-comparative study having included 44 children aged from 6 to 11 years and pharmacokinetic data from this study, but supplemented by the data previously obtained in adults and adolescents,**
- **the absence of comparison with the available alternatives, HUMIRA (adalimumab) and ENBREL (etanercept),**
- **the short-term safety profile (experience of one year) globally similar to that observed in adults and adolescents, but with uncertainties with respect to long-term safety,**

the proprietary medicinal products :

- **STELARA 45 mg (ustekinumab) solution for injection and solution for injection in pre-filled syringe and**
- **STELARA 90 mg (ustekinumab) solution for injection in pre-filled syringe,**

provide no clinical added value (CAV V) in the treatment of severe chronic plaque psoriasis in children aged 6 to 11 years.