

SUMMARY

treosulfan

TRECONDI 1 g and 5 g

powder for solution for infusion

Indication extension

Adopted by the Transparency Committee on 17 July 2024

Summary of opinion

Favourable opinion for reimbursement in the following indication: “treosulfan in combination with fludarabine is indicated as part of conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT) in paediatric patients older than one month with non-malignant diseases”.

Transparency Committee's conclusions**Clinical Benefit**

- ➔ Non-malignant diseases requiring allogeneic haematopoietic stem cell transplantation can be life-threatening.
- ➔ TRECONDI (treosulfan) is a preparatory (conditioning) treatment prior to curative haematopoietic stem cell transplantation.
- ➔ The efficacy/adverse effects ratio is low in the paediatric population with non-malignant diseases due to:
 - the weakness of the evidence of its efficacy based on a descriptive study in which the primary endpoint of no transplant/conditioning-related mortality at D+100 is not the most appropriate endpoint to determine a clinical benefit;
 - a higher number of transplant rejections in the treosulfan group;
 - the absence of formal demonstration of lower toxicity compared to busulfan.
- ➔ In the absence of sufficiently robust comparative data, the role of TRECONDI (treosulfan) in combination with fludarabine cannot be defined compared to the other conditioning regimens used in paediatric patients. The treosulfan-fludarabine combination constitutes a new treatment option as a conditioning regimen prior to allogeneic haematopoietic stem cell transplantation (alloHSCT) in paediatric patients older than one month with non-malignant diseases.

➔ Public health benefit

Considering:

- the seriousness of the disease and its incidence,
- the partially met medical need,

- the lack of response to the identified need considering:
 - the lack of evidence of an additional impact on morbidity and mortality and on quality of life
 - the absence of a potential impact on the organisation of care,

TRECONDI (treosulfan) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of TRECONDI (treosulfan) in combination with fludarabine is low in paediatric patients (older than one month) with non-malignant diseases as part of conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT).

The Committee issues a favourable opinion for inclusion of TRECONDI (treosulfan) in the list of covered drugs for inpatients approved for use in paediatric patients (older than one month) with non-malignant diseases as part of conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT), and at the MA dosages.

As a reminder¹, the Committee granted a clinical benefit level insufficient to justify public funding cover in combination with fludarabine as part of conditioning treatment prior to allogeneic haematopoietic stem cell transplantation (alloHSCT) in adult patients with non-malignant diseases (Transparency Committee opinion of 20/07/2020).

Clinical Added Value

Considering:

- the not very robust quality of the evidence of the efficacy of TRECONDI (treosulfan) in combination with fludarabine based on data from a descriptive phase 2 study;
- the absence of data enabling a conclusion to be reached with respect to an improvement in overall survival, quality of life or a better safety of the treosulfan-fludarabine combination compared to the busulfan-fludarabine combination;
- the absence of comparative data versus the conditioning regimens used in paediatric patients;
- the partially met substantial medical need in this indication;

the Committee considers that TRECONDI (treosulfan) provides no clinical added value (CAV V) in the current care pathway, which includes clinically relevant comparators.