HAUTE AUTORITÉ DE SANTÉ

TRANSPARENCY COMMITTEE
SUMMARY
16 JUNE 2021

The legally binding text is the original French opinion version

tagraxofusp
ELZONRIS 1 mg/ml concentrate for solution for infusion

First assessment

Key points

Unfavourable opinion for reimbursement as monotherapy for the first-line treatment of adult patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN).

Role in the care pathway?

Currently, there is no specific treatment for blastic plasmacytoid dendritic cell neoplasm (BPDCN), and no standardised management. Management is heterogeneous and varies depending on centres and countries.

The treatments used for BPDCN are derived from the chemotherapy protocols used in non-Hodgkin lymphoma (CHOP: cyclophosphamide, doxorubicin, vincristine and prednisone), acute myeloid leukaemia (cytarabine and anthracycline) or acute lymphoblastic leukaemia (Hyper-CVAD: cyclophosphamide, vincristine, doxorubicin, dexamethasone, alternating with methotrexate and cytarabine; or methotrexate and asparaginase).

For eligible patients, and in the event of an identified donor, haematopoietic stem transplant may also be envisaged in the event of a complete response.

Role of the medicinal product in the care pathway

Considering:

- **the absence of comparative data enabling assessment of the contribution of ELZONRIS (tagraxofusp) compared to the available alternatives, even though a direct comparative study would have been possible,**
- **the absence of robust evidence of a benefit on a clinically relevant endpoint,**
- **the medical need partially met by these alternatives, the use of which is well-established in France,**

the Transparency Committee considers that, on the basis of currently available data, ELZONRIS (tagraxofusp) has no role in the care pathway.

COMMITTEE'S CONCLUSIONS

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

Clinical benefit

◗ Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is a serious, life-threatening disease.

◗ The proprietary medicinal product ELZONRIS (tagraxofusp) is a curative medicinal product.

◗ In the absence of comparative data, even though there were clinically relevant comparators available, its efficacy/adverse effects ratio versus these comparators cannot be determined.

◗ There are therapeutic alternatives.

◗ ELZONRIS (tagraxofusp) has no role in the care pathway.

Public health impact

Considering:

- the seriousness of the disease and its prevalence,
- the partially met medical need,
- the lack of response to the identified need, with the absence of an additional impact on morbidity and mortality or quality of life, given an efficacy/adverse effects ratio that has not been established versus clinically relevant comparators,
- the absence of data enabling assessment of the impact on the care and/or life pathway,

ELZONRIS (tagraxofusp) is unlikely to have an additional impact on public health.

Considering all these elements, the Committee deems that the clinical benefit of ELZONRIS (tagraxofusp) is insufficient to justify its public funding cover in the MA indication.

The Committee issues an unfavourable opinion for inclusion in the hospital formulary list of reimbursed proprietary medicinal products approved for use in the MA indication and dosages.