SUMMARY OF A RECOMMENDATION BY COHERE FINLAND ON LUSPATERCEPT IN THE TREATMENT OF RED-BLOOD-CELL TRANSFUSION-DEPENDENT ANAEMIA AMONG ADULTS WITH BETA THALASSAEMIA

Recommendation approved at the meeting of the Council for Choices in Health Care in Finland (COHERE) on 24 March 2021

According to the recommendation of the Council for Choices in Health Care in Finland (COHERE Finland), luspatercept is not included in the national range of services for the treatment of red-blood-cell transfusion-dependent anaemia among adults with beta thalassaemia. The efficacy of luspatercept therapy is slight and, in relation, the costs are excessive.

Luspatercept is intended for the treatment of red-blood-cell transfusion-dependent anaemia in adult patients with beta thalassaemia. Luspatercept is given as a subcutaneous injection every three weeks. The objective of treatment is to reduce the need for red-blood-cell transfusions and to reduce the accumulation of iron, the so-called iron load, in the body. In addition, the drug is indicated for the treatment of anaemia associated with myelodysplastic syndrome.

Research data on the efficacy and safety of luspatercept have been obtained primarily from the placebo-controlled, double-blind, phase III BELIEVE study, in which patients were randomised to the luspatercept group (n = 224) and the placebo group (n = 112). The duration of treatment was at least 48 weeks. The proportion of patients with a reduction in total red-blood-cell units of at least 33% between weeks 13 and 24 and a reduction red-blood-cell transfusion of at least two units from baseline was 21% in the luspatercept group and 4% in the placebo group. In other words, four out of five patients who received luspatercept did not respond to treatment. The data on the maintenance of treatment response, the effects on the iron load or the need for iron chelation therapy are insufficient so far. Based on the information available at the time of the adoption of the recommendation, it was not possible to estimate the mean duration of luspatercept treatment.

Patients receiving luspatercept had more serious adverse events compared to the placebo group. Discontinuation of treatment due to an adverse event was also more common among the patients receiving luspatercept.

There are several uncertainties associated with cost estimation. The annual per-patient cost of the drug and administration is approximately EUR 84,000. The average annual cost of red-blood-cell transfusion treatment is EUR 5,000. It is estimated that there are about 15 red-blood-cell transfusion-dependent patients with beta thalassaemia in Finland and about five patients who would be suitable for luspatercept.

Beta thalassaemia is an inherited rare disease that is rare in Finland. Beta thalassaemia causes anaemia with symptoms such as tiredness, shortness of breath, skeletal changes and growth disturbances in children. Some people with beta thalassaemia need regular red-blood-cell transfusions.

This is a summary of a recommendation adopted by the Council for Choices in Health Care in Finland (COHERE Finland) The actual recommendation and the related background material are available in Finnish on the recommendations page of the website of COHERE Finland.

The summary of the recommendation is also available on the website in Swedish and Finnish.

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The Council for Choices in Health Care in Finland (COHERE Finland) works in conjunction with the Ministry of Social Affairs and Health, and its task is to issue recommendations on services that should be included in the range of public health services. Further information about service choices in health care is available on the COHERE Finland website