SUMMARY OF COHERE FINLAND'S RECOMMENDATION CONCERNING THE USE OF ZYNTEGLO IN THE TREATMENT OF BETA-THALASSAEMIA

The Council for Choices in Health Care in Finland (COHERE Finland) adopted the recommendation at its meeting on 5 May 2021.

COHERE Finland recommends that the treatment of transfusion-dependent beta-thalassaemia using Zynteglo be excluded from the services financed from public funds. Although Zynteglo has been shown to be initially effective, the long-term benefits and risks are not yet known. The cost of the treatment is high compared to the anticipated benefits and given the uncertainty surrounding the evidence.

Zynteglo (betibeglogene autotemcel) is indicated for the treatment of patients 12 years and older with transfusion-dependent beta-thalassaemia (TDT) who do not have a β^0/β^0 genotype, for whom haematopoietic stem cell (HSC) transplantation is appropriate but a matching related HSC donor is not available. Zynteglo contains as its active substance stem cells taken from the patients that have been genetically modified to contain a working gene for beta-globin. When these modified cells are given back to the patient, they are transported in the bloodstream to the bone marrow where they start to make red blood cells that are able to produce beta-globin. Zynteglo is a one-time gene replacement therapy that targets the main cause of TDT.

Current knowledge of Zynteglo is based on four small clinical trials and data from 24 patients. No control group was studied. Zynteglo was found to be effective in allowing 83% of the participants to reach blood transfusion independency over a period of 12 months. However, the participants have not been followed up for very long at this point, and the long-term effect of the treatment cannot be evaluated. It is not currently known whether patients treated with Zynteglo will need blood transfusions later down the line. The effect

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on the patients' iron levels is still being evaluated. Due to the short follow-up period, Zynteglo's contribution to the risk of complications and mortality from iron overload is not known. However, iron chelation therapy is expected to continue for years after Zynteglo infusion to manage iron overload. The impact on the patients' quality of life also remains unknown.

There is considerable uncertainty surrounding the cost-effectiveness of the treatment. The therapy is estimated to increase the cost per patient by around one million euros compared to the current treatment standard. There are currently estimated to be five patients eligible for the therapy in Finland.

Beta-thalassaemia is a rare genetic blood disorder with only a handful of sufferers in Finland. It causes anaemia, and some patients require regular blood 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 website of COHERE Finland under Recommendations.

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

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 healthcare is available on the COHERE Finland website.

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