SUMMARY

27 October 2021 1(2)

STM051:00/2020

VN/28713/2020

Summary of a recommendation by COHERE Finland

Onasemnogene abeparvovec (Zolgensma) for treating spinal muscular atrophy

The Council for Choices in Health Care in Finland (COHERE Finland) adopted the recommendation at its meeting on 27 October 2021.

COHERE Finland recommends that onasemnogene abeparvovec (Zolgensma®) be included in the national range of services for treating type 1 (two copies of the SMN2 gene) spinal muscular atrophy (SMA) on the condition that the seller and buyer of the medicinal product agree on a price significantly below the wholesale list price, taking into account the considerable uncertainties in the information on the effectiveness and safety of the treatment. In addition, Zolgensma is recommended as an option for treating presymptomatic younger siblings of an SMA1 patient if they have been genetically tested and if the treatment is considered justified based on a clinical assessment and if the younger siblings have a missing SMN1 gene and two copies of the SMN2 gene.

Onasemnogene abeparvovec (trade name Zolgensma) is recommended for treating patients who have spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and either a clinical diagnosis for SMA1 or up to three copies of the SMN2 gene. Onasemnogene abeparvovec is administered as a single-dose intravenous infusion. Earlier, nusinersen was approved for treating SMA in Europe in 2017.

The main effectiveness and safety evidence for onasemnogene abeparvovec comes from small single-arm studies without comparison treatment groups. These studies enrolled patients with the severest form of SMA, i.e., patients with two copies of the SMN2 gene. There is interim evidence from studies involving presymptomatic patients with 2–3 copies of the SMN2 gene. The evidence shows that SMA1 patients (with two copies of the SMN2 gene) who were treated with Zolgensma had a longer lifespan and their motor functioning

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improved compared to outcomes for best supportive care during a short-term follow up of 14 months and 18 months. Because there is limited long-term evidence, it is also uncertain whether the motor milestones achieved with Zolgensma are maintained throughout the patient's lifespan.

Zolgensma has a very high one-off cost, amounting to around EUR 2 million. The cost-effectiveness comparisons of Zolgensma and nusinersen are highly uncertain.

Significant ethical issues are associated with both Zolgensma and nusinersen, particularly with regard to uncertainties about long-term effects and outcomes.

Spinal muscular atrophy (SMA) is a rare neuromuscular genetic condition that causes spinal motor neurones to deteriorate and fail to stimulate muscle fibres. SMA can grouped into a number of types, based on the number of copies of the SMA2 gene, clinical picture, and age of onset. Type 1 SMA is a serious condition and results in death in early childhood. The prevalence of SMA is estimated to be approximately 9.4 cases per 100,000 children born alive. It is estimated that 60% of all new SMA cases are of type 1.
This is a summary of a recommendation adopted by the Council for Choices in Health

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

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