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SUMMARY OF COHERE FINLAND'S RECOMMENDATION FOR TOFERSEN IN THE TREATMENT OF AMYOTROPHIC LATERAL CLEROSIS (ALS) ASSOCIATED WITH SUPEROXIDE DISMUTASE 1 (SOD1) GENE MUTATION

The Council for Choices in Health Care in Finland (COHERE Finland) adopted the recommendation at its meeting on 5 February 2026.

Tofersen is not included in the national range of services for the treatment of amyotrophic lateral sclerosis (ALS) associated with superoxide dismutase 1 (SOD1) gene mutation.

In COHERE Finland's view, the therapeutic value of tofersen compared with current treatment has not been demonstrated. The data provided by the marketing authorisation holder did not include comparative data with the medicinal treatment used in current Finnish clinical practice. The patients participating in the studies were not representative of Finnish ALS patients. Tofersen is a very expensive drug. Treatment with tofersen is not cost-effective in Finland.

The evidence of the clinical efficacy and safety of tofersen in the recommended indication is mainly based on a phase III randomised, double-blind, placebo-controlled multicentre study and a subsequent open-label extension study.

In the phase III randomised controlled trial, no statistically significant differences were observed between tofersen and a placebo in physical functioning, respiratory function or muscle strength. Reductions in biomarkers measured in cerebrospinal fluid were observed, supporting the presumed biological effect of tofersen. The duration of the placebo-controlled follow-up was too short to reliably assess the long-term clinical efficacy of tofersen, including its effects on event-free survival, overall survival prognosis or the time before patients require ventilatory support. No comparison with standard care according to current clinical practice was conducted, and the efficacy or effectiveness of tofersen therapy or combination therapy with tofersen and riluzole therefore remained unproven.

Almost all patients (99.3%) receiving tofersen experienced an adverse event. A serious adverse event occurred in 44.2% of patients, and a treatment-related adverse event occurred in

66.7% of patients. Treatment with tofersen increases patients' dependence on hospital-based care. Finnish ALS patients typically suffer from a very slowly progressive form of the disease, in which the harms associated with tofersen treatment outweigh any potential benefits.

No analysis specific to the gene variant was conducted in the marketing authorisation study, and the study therefore did not provide information about whether treatment response differed between specific gene variants. The SOD1 gene variants found in the studied patients do not represent those occurring in Finland. The study results are not directly generalisable to Finnish patients.

Tofersen does not replace the standard treatment with riluzole. The standard treatment is moderate in cost (EUR 2,542 per patient per year) compared with tofersen (EUR 300,125 per patient per year in the first year and EUR 278,946 per patient per year in subsequent years) at public list prices. Tofersen is not cost-effective in any of the modelled scenarios in Finland (ICER EUR 1.3 million per QALY). Additional costs arise from the genetic testing associated with tofersen treatment, travel to and from hospital, the monitoring of treatment effects, and the management of adverse effects. The introduction of tofersen would also require some increase in personnel numbers.

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 [Valmiit suositukset](#).

The summary of the recommendation is also available in [Swedish](#) and [English](#) 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](#).