SUMMARY OF A RECOMMENDATION BY COHERE FINLAND ON CERLIPONASE ALFA IN THE TREATMENT OF NEURONAL CEROID LIPOFUSCINOSIS TYPE 2 (CLN2)

The Council for Choices in Health Care in Finland (COHERE Finland) approved the recommendation at its meeting on 1 September 2021.

According to the recommendation, cerliponase alfa is included in the national range of service choices for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease if the marketing authorisation holder and the buyer agree on a sufficient price reduction.

Cerliponase alfa is indicated for the treatment of a storage disease named neuronal ceroid lipofuscinosis type 2 (CLN2). Cerliponase alfa is a copy of the recombinant human tripeptidyl peptidase-1 (rhTPP1) protein that replaces this missing enzyme. Cerliponase alfa is administered once every other week by infusion using a surgically implanted intracerebroventricular access device. Treatment can be continued for as long as the patient benefits from it. Cerliponase alfa is currently the only accepted treatment for the CLN2 disease.

The safety and efficacy of cerliponase alfa has mainly been studied in a single open-label, phase 1/2 190-202 study (n=23) that had no control group. Comparisons have been made against patient data extracted from the international CLN disease database. Even though efforts were made to match patients, a lot of clinical data is missing concerning patients in the historical register database, and the reliability of the comparison cannot be ascertained. Based on the results, cerliponase alfa slows down the natural progression of the disease. 87% of the patients (n=20) achieved the set response when the median treatment

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duration was 116 weeks. Results concerning changes in health-related quality of life have only been reported to a limited extent. The effects on survival time cannot yet be assessed.

In the reasoning of the recommendation, it is noted that based on clinical evidence, cerliponase alfa slows down the progression of the disease, but research data on the extent and duration of the effect is uncertain. The method of administration involves significant risks. Treatment should be started as early as possible.

In the recommendation, it is required that the criteria for starting and ending treatment, as well as the evaluation criteria to be used, have been agreed upon in the units providing treatment before any treatments are started. The preconditions for continuing the treatment must be assessed on a regular basis. The treatment is extremely expensive, more than EUR 500,000 per patient a year. The costs at the public wholesale price would be unreasonable considering that the treatment is not curative but only slows down the natural progression of the disease and the expected health benefits of the treatment involve uncertainty.

Neuronal ceroid lipofuscinosis type 2 (CLN2) is a rare progressive disease, the first symptoms of which appear during the second and third year of life. The disease leads to death in childhood or adolescence. There would probably be one or at most a few patients eligible for the treatment in Finland.

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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 COHERE Finland website under Recommendations.

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

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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