A summary of a COHERE Finland recommendation

The summary is related to the recommendation "Criteria for continued treatment with nusinersen" repealed by section 4 February 2022. Link to the updated recommendation.

Criteria for continued treatment with nusinersen

Recommendation approved at the meeting of Council for Choices in Health Care in Finland (COHERE Finland) on 4 September 2019. According to the recommendation, the medicine should improve the patient's ability to function during the first year of treatment, and thereafter the situation should remain at least stable in order to continue the treatment.

On 15 March 2018, COHERE Finland issued a recommendation on the treatment with nusinersen in spinal muscular atrophy (SMA). SMA is a rare disease for which there is currently no cure. Nusinersen is the first medicine that can affect the course of the disease. COHERE Finland concluded that the initiation of treatment with nusinersen could be included in the national range of services for the treatment of SMA if:

- the SMA diagnosis has been confirmed for the patient before the age of two years,
- the symptoms observed by the patient's doctor have started before the age of 20 months,
- the patient is no more than 17 years old,
- the patient is not in need of permanent respiratory support and
- there is no other medical impediment to the treatment.

In addition, COHERE Finland stated that the nusinersen medicinal product cannot be taken into use unless its price is reduced.

COHERE Finland stated that, due to insufficient research data, it is also important to set the criteria for continued treatment, on which COHERE Finland makes this recommendation.

COHERE Finland states that the prerequisites for continued treatment with nusinersen will be assessed for the first time 12 months after the start of treatment. Continued treatment requires that it can be reliably established that the medicine has improved the patient's ability to function during the first year of treatment, taking into account the patient's age, structural changes limiting the patient's ability to function, and the natural course of the disease.

After the first year of treatment, continued treatment with nusinersen is evaluated every 12 months. Maintaining the response to treatment achieved during the first year of treatment is the minimum requirement for medical justification to continue the treatment.

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When assessing the prerequisites for continuing the pharmacotherapy, a national expert group should be consulted, which enables making the treatment decision on equal and objective grounds. The actual treatment decision is made by the attending physician in accordance with, in particular, sections 6 and 7 of the Act on the Status and Rights of Patients.

It is medically necessary to ensure that the pharmacotherapy produces a response that affects the course of the disease. This protects patients from the adverse effects of medicines and ensures the correct allocation of healthcare resources. Research data on nusinersen effects in clinical patient groups is very limited. However, an individual patient's ability to function may improve significantly during the first year of treatment. It is possible that, even in these cases, the response to treatment will change over time to maintain the patient's ability to function or the response to treatment will be lost.

Studies with carefully selected patient groups have demonstrated that the younger the patient was when the treatment was started, the more likely the response to treatment is clinically significant with regard to improved gross motoric performance. No medical grounds have been found in Finland for not initiating treatment with nusinersen in the target group of the recommendation. Therefore, the clinical patient group has been significantly weaker in the initial phase of treatment than those involved in the drug trials and, for several patients, it has not been possible to use the same methods for evaluating the ability to function that were used in the drug trials. Changes in the ability to function have been very individual, ranging from qualitative changes (better overall coping) to such significant changes improving independent ability to function and reducing the need for assistance that would not have been achievable without the treatment with nusinersen, taking into account the normal course of the disease.

Updating the recommendation will start by 2021 at the latest, taking into account practical experience of the implementation of the recommendation and of the effectiveness of treatment with nusinersen.

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: www.palveluvalikoima.fi.