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Translarna: renewal of conditional marketing authorisation

The CHMP completed its scientific assessment of the annual renewal of the conditional marketing authorisation for **Translarna** (ataluren) and recommended that the conditional marketing authorisationbe renewed. As part of the CHMP assessment, the Committee reviewed all available data, including the results of a study performed by the marketing authorisation holder as a requirement of the conditional marketing authorisation after initial approval. Although the data available to date continue toindicate that Translarna slows the progression of the disease and there are no major safety concerns, the Committee considered that further comprehensive data are still needed to fully confirm that the benefit-risk balance of the medicine is positive. The CHMP has therefore requested that the marketing authorisation holder for Translarna conducts a new 18-month randomised,placebo-controlledstudy in patients withDuchenne muscular dystrophy,followed by an 18-month period where all patients will be switched to Translarna. The study results are expected to be available in the first quarter of 2021.

Translarna is used to treat patients aged five years and older with Duchenne muscular dystrophy, a serious and rare condition for which no authorised treatments are currently available. The medicine is intended for use in patients who are able to walk and whose disease is caused by a specific genetic defect (called a 'nonsense mutation') in the gene for the muscle protein dystrophin.

Conditional approval allows EMA to recommend a medicine for marketing authorisation where the benefit to public health of its immediate availability on the market outweighs the risk inherent in the fact that additional data are still required. These medicines are subject to specific post-authorisation obligations that aim to generatecomprehensivedata on the medicine. Conditional marketing authorisations are valid for one year and can be renewed or converted to a standardfive-year marketing authorisation when the additional data generated confirm that the benefit-risk balance of the medicine is positive.

The assessment report on the renewal of the conditional marketing authorisation for Translarna will be published after the European Commission issues its decision on the renewal.

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