



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

22 May 2014  
EMA/CHMP/310130/2014  
Committee for Medicinal Products for Human Use (CHMP)

## Summary of opinion<sup>1</sup> (initial authorisation)

---

### Translarna ataluren

On 22 May 2014, following a re-examination procedure, the Committee for Medicinal Products for Human Use (CHMP), adopted a positive opinion, recommending the granting of a conditional marketing authorisation for the medicinal product Translarna intended for the treatment of Duchenne muscular dystrophy (DMD).

Translarna was designated as an orphan medicinal product on 27 May 2005. The applicant for this medicinal product is PTC Therapeutics Limited.

The active substance of Translarna is ataluren. It is to be available as 125, 250 and 1000 mg granules for oral suspension.

Duchenne muscular dystrophy can be caused by a number of genetic abnormalities. Translarna is for use in patients whose disease is due to the presence of certain defects (called nonsense mutations) in the dystrophin gene which prematurely stop the production of a normal dystrophin protein, leading to a shortened dystrophin protein that does not function properly. Translarna is thought to work in these patients by enabling the protein-making apparatus in cells to skip over the defect, allowing the cells to produce a functional dystrophin protein.

Translarna was investigated in a pivotal study involving 174 patients with DMD. The results showed some evidence of efficacy of Translarna in slowing down the loss of walking ability in DMD patients. However, as part of the conditional marketing authorisation, the company will be required to provide comprehensive data on the efficacy of Translarna from an ongoing confirmatory study. Regarding safety, Translarna is generally well tolerated with the most common side effects being headache, nausea and vomiting.

As for all medicinal products, a pharmacovigilance plan for Translarna will be implemented as part of the marketing authorisation.

---

<sup>1</sup> Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.



The recommended indication is: "the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 5 years and older. Efficacy has not been demonstrated in non-ambulatory patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing." Treatment with Translarna should only be initiated by specialist physicians with experience in the management of Duchenne/Becker muscular dystrophy.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and will be available in all official European Union languages after the marketing authorisation has been granted by the European Commission.