EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

23 May 2014
EMA/312458/2014
Press Office

Press release

European Medicines Agency recommends first-in-class medicine for treatment of Duchenne muscular dystrophy

New medicine offers therapeutic innovation for rare disease with high unmet medical need

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has recommended granting a conditional marketing authorisation for Translarna (ataluren), an orphan-designated medicine for the treatment of Duchenne muscular dystrophy caused by nonsense mutations. Translarna is to be used in patients aged five years and older who are able to walk.

Duchenne muscular dystrophy is a genetic disease that gradually causes weakness and loss of muscle function. Patients with the condition lack normal dystrophin, a protein found in muscles. Because this protein helps to protect muscles from injury as muscles contract and relax, in patients with the disease the muscles become damaged and eventually stop working. There are currently no approved therapies available for this life-threatening condition and the current management of the disease is based on prevention and management of complications.

In the European Union (EU), approximately 18,600 people have Duchenne muscular dystrophy. The disease can be caused by a number of genetic abnormalities. Translarna is for use in the subgroup of 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.

In January 2014, the CHMP originally adopted a negative opinion for Translarna, but at the request of the applicant, the CHMP started a re-examination of its opinion. Following careful consideration of all available evidence, including a re-analysis of the clinical data submitted by the company, the Committee concluded that the data available are sufficient to recommend a conditional marketing authorisation. Under the terms of the authorisation, the company will be required to provide comprehensive data from an ongoing confirmatory study.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8427 **Facsimile** +44 (0)20 7418 8409
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

Conditional marketing authorisation is an early access mechanism which allows the Agency to recommend marketing authorisation for medicines that address an unmet medical need for patients suffering from life-threatening diseases even if comprehensive clinical data are not yet available.

The applicant for Translarna is PTC Therapeutics Limited. The company is registered as a micro-, small- or medium-sized-enterprise (SME), and as such benefited from support and incentives offered by the Agency's SME office.

Because Translarna has an orphan designation, the Agency provided free scientific advice to the applicant during the development of the medicine. Orphan designation and the associated incentives, such as free scientific advice or 'protocol assistance', are among the Agency's most important instruments to encourage the development of medicines for patients suffering from rare diseases.

The CHMP opinion on Translarna will now be sent to the European Commission for adoption of a decision on an EU-wide marketing authorisation.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 7418 8427

E-mail: press@ema.europa.eu