All Wales Medicines Strategy Group

Grŵp Strategaeth Meddyginiaethau Cymru Gyfan

Final Appraisal Recommendation

Advice No: 0419 – March 2019

Ataluren (Translarna®) 125 mg, 250 mg and 1,000 mg granules for oral suspension

Limited submission by PTC Therapeutics Ltd

Recommendation of AWMSG

Ataluren (Translarna®) is recommended for use within NHS Wales for the treatment of Duchenne muscular dystrophy (DMD) resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years to less than 5 years.

This recommendation applies only in circumstances where the approved Patient Access Scheme (PAS) is utilised or where the list/contract price is equivalent or lower than the PAS price.

Additional note(s):

- Please refer to the Summary of Product Characteristics for the full licensed indication.
- AWMSG considered that ataluren (Translarna®) satisfied the AWMSG criteria for an ultra-orphan medicine.
- AWMSG recommend that ataluren (Translarna®) be used in accordance with Specialised Service Policy: CP118 Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene, and, National Institute of Health and Care Excellence highly specialised technology guidance HST3 for treating nonsense mutation DMD in children aged five years and older who can walk.

In reaching the above recommendation AWMSG has taken account of the appraisal documentation prepared by the AWMSG Secretariat (reference number 3911), which includes the AWMSG Secretariat Assessment Report (ASAR), the Preliminary Appraisal Recommendation (PAR) and the applicant company's response to the PAR, clinical expert opinion (where available), the views of patients/patient carers (where available) and the lay member perspective.

This recommendation has been ratified by Welsh Government and will be considered for review every three years.