translarna™
ataluren

SYMPOSIUM

Translarna (ataluren): Long-term treatment experience in patients with nmDMD

PTC Therapeutics symposium at the 17th International Congress on Neuromuscular Diseases (ICNMD), Brussels, Belgium. Wednesday, 6 July 2022, 13:00–14:00 CEST (Silver Hall)

Join our expert faculty in an interactive, peer-to-peer discussion on the key long-term and real-world results of Translarna use in treating patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). Through the use of real-world clinical case studies, the challenges patients face in transitioning from paediatric to adult care will also be discussed, alongside the key outcomes of treatment with Translarna for these patients.

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Time (CEST)	Topic	Speaker
13:00–13:05	Welcome and introductions	Dr. med. Christian Werner (Chair) PTC Therapeutics
13:05–13:25	Key results of long-term and real-world Translarna use in nmDMD	Prof. Luca Bello
13:25–13:45	Case studies: Understanding how patients with nmDMD can successfully transition from paediatric to adult care	Prof. Ros Quinlivan
13:45–13:55	Panel discussion and Q&A session	All speakers (moderated by the Chair)
13:55–14:00	Summary with key takeaways and close of the symposium	Dr. med. Christian Werner PTC Therapeutics

Expert faculty members

Dr. med. Christian Werner (Chair)
Executive Director,
Global Medical Affairs – Global DMD Lead,
PTC Therapeutics

Prof. Luca Bello
Associate Professor of Neurology,
Department of Neurosciences DNS,
University of Padova, Italy

Prof. Ros Quinlivan
MRC Centre for Neuromuscular Disease, National Hospital for Neurology and Neurosurgery, London, UK

This promotional symposium has been organised and funded by PTC Therapeutics for healthcare professionals only. This presentation was approved by the Scientific Program Committee as an independent activity held in conjunction with the 17th International Congress on Neuromuscular Diseases. This presentation is not sponsored or endorsed by ICNMD 2022.

Translarna prescribing information can be found on the reverse side of this flyer.

PTC THERAPEUTICS™

Abbreviated Prescribing Information Indication: Translarna™ (active ingredient: ataluren) is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene (nmDMD), in ambulatory patients aged 2 years and older. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing. **Posology and administration:** Translarna is available as granules for oral suspension in sachets of 125 mg, 250 mg or 1000 mg. The recommended dose is 10 mg/kg body weight in the morning, 10 mg/kg body weight at midday, and 20 mg/kg body weight in the evening (for a total daily dose of 40 mg/kg body weight). Patients should not take a double or extra dose if a dose is missed. It is important to administer the correct dose. Increasing the dose above the recommended dose may be associated with reduced effectiveness. Treatment of patients with severe renal impairment (eGFR <30 ml/min) or end-stage renal disease is not recommended. The safety and efficacy of Translarna in children <12kg and aged 6 months to 2 years have not yet been established. Treatment with Translarna should only be initiated by specialist physicians with experience in the management of DMD. **Ingredients:** Active ingredient: ataluren. **Excipients:** polydextrose (E1200), macrogol, poloxamer, mannitol (E421), crospovidone, hydroxyethyl cellulose, artificial vanilla flavour (maltodextrin, artificial flavours and propylene glycol), silica, colloidal anhydrous (E551), magnesium stearate. **Contraindications:** Patients with hypersensitivity to the active substance or to any of the excipients; concomitant use of intravenous aminoglycosides. **Special warnings and precautions for use:** Patients who do not have a nonsense mutation should not receive Translarna. Patients with severe renal impairment or end-stage renal disease should be treated with ataluren only if the anticipated clinical benefit outweighs the potential risk, and should be closely monitored for possible metabolite toxicity and decrease in efficacy. A lower ataluren dose should be considered. Treatment should not be initiated in previously untreated patients with eGFR <30 ml/min. It is recommended that total cholesterol, LDL, HDL, triglycerides be measured annually, and serum creatinine, BUN, cystatin C be measured every 6 to 12 months. Resting systolic and diastolic blood pressure should be monitored every 6 months in patients receiving Translarna concomitantly with corticosteroids. All clinical measures and/or laboratory testing may be conducted more frequently as needed based on clinical status. See precaution for use with other medicines in next "interactions" section. **Interactions:** Translarna should not be co-administered with intravenous aminoglycosides, and concomitant use of other nephrotoxic agents is not recommended. Caution should be exercised when Translarna is co-administered with medicinal products that are inducers of UGT1A9, or substrates of OAT1, OAT3 or OATP1B3 and when co-administered with adefovir. Based on in vitro studies Translarna is not expected to be an inducer of P450 isoenzymes. **Fertility, pregnancy and lactation:** It is recommended to avoid the use of Translarna in pregnancy. Breast-feeding should be discontinued during treatment with Translarna. Non-clinical data revealed no hazard for humans based on standard male and female fertility study in rats. **Effects on ability to drive and use machines:** Patients who experience dizziness should use caution when driving, cycling or using machines. Adverse reactions: Adverse events reported in clinical trials of predominantly paediatric nmDMD patients treated at the recommended dose of 10-, 10-, 20mg/kg/day according to frequency: Very common (≥1/10): vomiting. Common (≥1/100 to <1/10): decreased appetite, hypertriglyceridemia, headache, hypertension, cough, epistaxis, nausea, upper abdominal pain, flatulence, abdominal discomfort, constipation, rash erythematous, pain in extremity, musculoskeletal chest pain, haematuria, enuresis, pyrexia, weight decreased. Events with unknown frequency due to low numbers: increased blood urea nitrogen, cholesterol, creatinine, cystatin C, triglycerides. **Marketing Authorisation number and holder:** EU/1/13/902/001-002-003. PTC Therapeutics International Limited, 5th Floor, 3 Grand Canal Plaza, Grand Canal Street Upper, Dublin 4, Ireland. Please consult the SmPC before prescribing. **Date of Preparation:** June 2021.

▼ This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions via the national reporting system. Adverse events should also be reported to PTC Therapeutics at pharmacovigilance@ptcbio.com.

Translarna▼(ataluren) is indicated for the treatment of Duchenne muscular dystrophy resulting from a nonsense mutation in the dystrophin gene, in ambulatory patients aged 2 years and older in the European Member States and Iceland, Liechtenstein, Norway, Great Britain, Northern Ireland, Kazakhstan, Israel, Republic of Korea, Belarus, Russia, and Brazil, and aged 5 years and older in Chile and the Kingdom of Saudi Arabia, and Ukraine (under special state registration). In Brazil, the indication is specific to male paediatric patients. The presence of a nonsense mutation in the dystrophin gene should be determined by genetic testing (Translarna Summary of Product Characteristics (SmPC) for respective countries). Translarna received a conditional marketing authorization in the European Member States and Iceland, Liechtenstein, Norway, Great Britain and Northern Ireland.

Registrations conditions differ internationally, always consult local prescribing information and/or Summary of Product Characteristics before prescribing any product.

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