translarna™
ataluren

Advancing the management of Duchenne muscular dystrophy: the disease continuum and transition from pediatric to adult care

A virtual symposium at ICNMD 2021

Friday 28 May 2021; 16:15–17:00 (CEST)

Please join our expert faculty in a peer-to-peer discussion on the transition from pediatric to adult care for patients with nonsense mutation Duchenne muscular dystrophy (nmDMD).

This virtual symposium will explore the following areas:

- The disease continuum of DMD and the importance of multidisciplinary care
- Importance of early and continued intervention for slowing disease progression
- Challenges encountered by patients with nmDMD who are transitioning from pediatric to adult care
- Management of patients with nmDMD using Translarna ▼ (ataluren)* throughout the disease continuum

Dr. med. Christian Werner *(Chair)*
PTC Therapeutics

Prof. Tracey Willis *(Speaker)*
Consultant Pediatric Neurologist
The Robert Jones and Agnes Orthopaedic Hospital Gobowen, Oswestry, England

Prof. Thomas Sejersen *(Speaker)*
Professor in Neuropediatrics
University Hospital and Karolinska Institute Stockholm, Sweden

16:15–16:20	**Welcome and introduction** Dr. med. Christian Werner *(Chair)*
16:20–16.30	**DMD as a disease continuum: a succession of management milestones** Prof. Tracey Willis *(Speaker)*
16:30–16:40	**The transition from pediatric to adult care for patients with nmDMD** Prof. Thomas Sejersen *(Speaker)*
16:40–17:00	**Panel discussion and Q&A: managing patients with nmDMD throughout the disease continuum** *Moderated by the Chair:* Dr. med. Christian Werner

This ICNMD 2021 virtual symposium is organized and funded by PTC Therapeutics International Ltd, contains product information and is intended for healthcare professionals only.

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This presentation was approved by the Program Committee as an independent activity held in conjunction with ICNMD 2021.
This presentation is not sponsored or endorsed by ICNMD 2021.

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***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, and Republic of Korea, and aged 5 years and older in Chile, Brazil, and Ukraine *(under special state registration)*. 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)*.

In Russia, Translarna is indicated for congenital malignant DMD resulting from a nonsense mutation in the dystrophin gene in adults and children over the age of 2 years *(Translarna Instructions for Use [IFU] – Russia)*.

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 reactions reported in clinical trials of 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. A higher frequency of malaise (7.1%), pyrexia (42.9%), ear infection (28.6%), and rash (21.4%) were reported in patients aged 2-5 years compared with patients 5 years of age and older. **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:** August 2020.

▼ 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 at: **pharmacovigilance@ptcbio.com**.

Registrations conditions differ internationally, always consult local prescribing information and/or Summary of Product Characteristics before prescribing any product. For the EU Translarna Summary of Product Characteristics, **please click here**.

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