Package leaflet: Information for the patient

Translarna 125 mg granules for oral suspension
Translarna 250 mg granules for oral suspension
Translarna 1000 mg granules for oral suspension
ataluren

▼This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See the end of section 4 for how to report side effects.

Read all of this leaflet carefully before you start taking this medicine because it contains important information for you.
- Keep this leaflet. You may need to read it again.
- If you have any further questions, ask your doctor or pharmacist.
- This medicine has been prescribed for you only. Do not pass it on to others. It may harm them, even if their signs of illness are the same as yours.
- If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. See section 4.

What is in this leaflet

1. What Translarna is and what it is used for

Translarna is a medicine that contains the active substance ataluren.

Translarna is used to treat Duchenne muscular dystrophy resulting from a specific genetic defect that affects normal muscle function.

Translarna is used to treat patients aged 2 years and older, who are able to walk.

You or your child will have been tested by your doctor before starting treatment with Translarna, in order to confirm that your disease is suitable for treatment with this medicine.

How does Translarna work?

Duchenne muscular dystrophy is caused by genetic changes that result in an abnormality in a muscle protein called dystrophin which is needed for muscles to work properly. Translarna enables the production of working dystrophin and helps muscles work properly.

2. What you need to know before you take Translarna

Do not take Translarna
- If you are allergic to ataluren or any of the other ingredients of this medicine (listed in section 6).
- If you are receiving treatment with certain antibiotics, such as gentamicin, tobramycin, or streptomycin by injection into a vein.

Warnings and precautions
Your doctor must have done a blood test to confirm that your disease is suitable for treatment with Translarna. If you have any kidney problem, your doctor should check your kidney function regularly.

If you have severe kidney problems (eGFR <30 ml/min) or if you are receiving dialysis because your kidneys do not work (end-stage renal disease) your doctor will established if treatment with Translarna is suitable for you.

Your doctor will test the levels of lipids (fats such as cholesterol and triglycerides) in your blood and your kidney function every 6 to 12 months. Your doctor will monitor your blood pressure every 6 months, if you are taking a corticosteroid medicine.

Children and adolescents
Do not give this medicine to children under the age of 2 years or weighing less than 12 kg as it has not been tested in this group of patients.

Other medicines and Translarna
Tell your doctor if you are taking, have recently taken, or might take any other medicines. In particular do not take Translarna with the antibiotics gentamicin, tobramycin, or streptomycin given by injection. These may affect your kidney function.

Tell your doctor if you are taking any of the following medicines:

Medicine	**Usually prescribed for**
acyclovir	treatment of chickenpox [varicella]
adefovir	treatment of chronic hepatitis B and/or HIV
atorvastatin	lipid-lowering
benzylpenicillin	severe infections
bumetanide	treatment or prevention of congestive heart failure
captopril	treatment or prevention of congestive heart failure
ciprofloxacin	treatment of infections
famotidine	treatment of active duodenal ulcer, gastroesophageal reflux disease
furosemide	treatment or prevention of congestive heart failure
methotrexate	rheumatoid arthritis, psoriasis
olmesartan	essential hypertension in adults
oseltamivir	prevention of influenza
phenobarbital	sleep-inducing, prevention of seizures
pitavastatin	lipid-lowering
pravastatin	lipid-lowering
rifampicin	treatment for tuberculosis
rosuvastatin	lipid-lowering
sitagliptin	type 2 diabetes
valsartan	treatment or prevention of congestive heart failure

Some of these medicines were not tested together with Translarna and your doctor may decide to monitor you closely.

Pregnancy and breast-feeding
If you are pregnant or breast-feeding, think you may be pregnant or are planning to have a baby, ask your doctor for advice before taking this medicine. If you become pregnant while taking Translarna, consult your doctor immediately as it is recommended not to take Translarna while you are pregnant or breast-feeding.

Driving and using machines
If you feel dizzy, do not drive, cycle or use machines.

3. How to take Translarna

Always take this medicine exactly as your doctor or pharmacist has told you. Check with them if you are not sure.

Translarna is available in the following sachet strengths: 125 mg, 250 mg and 1000 mg of ataluren per sachet. Your doctor or pharmacist will tell you the exact number of sachets and what strength to take at each time.

Your dose of Translarna depends on your body weight. 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 (adding up to a total daily dose of 40 mg/kg body weight).

The medicine is taken by mouth mixed in liquid or semi-solid food.

Open the sachet only at the time you are taking the medicine and use the entire amount from the sachet. The full contents of each sachet should be mixed with at least 30 ml of liquid (water, milk, fruit juice) or 3 tablespoons of semi-solid food (yoghurt or apple sauce). Mix the prepared dose well before taking it. The amount of the liquid or semi-solid food can be increased based on your preference.

Posology table

Weight Range (kg)		**Number of Sachets**								
		Morning			**Midday**			**Evening**		
		125 mg sachets	**250 mg sachets**	**1000 mg sachets**	**125 mg sachets**	**250 mg sachets**	**1000 mg sachets**	**125 mg sachets**	**250 mg sachets**	**1000 mg sachets**
12	14	1	0	0	1	0	0	0	1	0
15	16	1	0	0	1	0	0	1	1	0
17	20	0	1	0	0	1	0	0	1	0
21	23	0	1	0	0	1	0	1	1	0
24	26	0	1	0	0	1	0	0	2	0
27	31	0	1	0	0	1	0	1	2	0
32	35	1	1	0	1	1	0	1	2	0
36	39	1	1	0	1	1	0	0	3	0
40	44	1	1	0	1	1	0	1	3	0
45	46	0	2	0	0	2	0	1	3	0
47	55	0	2	0	0	2	0	0	0	1
56	62	0	2	0	0	2	0	0	1	1
63	69	0	3	0	0	3	0	0	1	1
70	78	0	3	0	0	3	0	0	2	1
79	86	0	3	0	0	3	0	0	3	1
87	93	0	0	1	0	0	1	0	3	1
94	105	0	0	1	0	0	1	0	0	2
106	111	0	0	1	0	0	1	0	1	2
112	118	0	1	1	0	1	1	0	1	2
119	125	0	1	1	0	1	1	0	2	2

Take Translarna by mouth 3 times per day; in the morning, midday and evening. There should be 6 hours between morning and midday doses, 6 hours between midday and evening doses, and 12 hours between the evening dose and the first dose on the next day. For example, you might take Translarna

at 7:00 AM in the morning with breakfast, at 1:00 PM in the afternoon with lunch, and again at around 7:00 PM in the evening with dinner.

Drink water or other liquids regularly to avoid dehydration while taking Translarna.

If you take more Translarna than you should
Contact your doctor if you take more than the recommended dose of Translarna.
You may experience mild headache, nausea, vomiting or diarrhoea.

If you forget to take Translarna
If you are late in taking Translarna by less than 3 hours after the morning or midday doses, or by less than 6 hours after the evening dose, take the dose. Remember to take the next dose on time.
If you are late by more than 3 hours after the morning or midday doses, or by more than 6 hours after the evening dose, do not take the dose. But, take the next doses on time.

Do not take a double dose to make up for a forgotten dose. It is important to take the correct dose. Translarna may not be as effective in treating your symptoms if you take more than the recommended dose.

If you stop taking Translarna
Do not stop taking Translarna without talking to your doctor.

If you have any further questions on the use of this medicine, ask your doctor.

4. Possible side effects

Like all medicines, this medicine can cause side effects, although not everybody gets them. You may have one or more of the following side effects after taking Translarna:

Very common side effects (may affect more than 1 in 10 people):
- Vomiting

Common side effects (may affect up to 1 in 10 people):
- Decreased appetite
- High blood triglyceride levels
- Headache
- Feeling sick
- Weight loss
- High blood pressure
- Cough
- Nosebleed
- Constipation
- Wind
- Stomach discomfort
- Stomach pain
- Rash
- Arm or leg pain
- Chest pain
- Involuntary urination
- Blood in urine
- Fever

Frequency not known (frequency cannot be estimated from the available data):
- Increases in blood lipids
- Increases in test for kidney function

Reporting of side effects
If you get any side effects, talk to your doctor or pharmacist. This includes any possible side effects not listed in this leaflet. You can also report side effects directly via:

Ireland
HPRA Pharmacovigilance
Earlsfort Terrace
IRL - Dublin 2
Tel: +353 1 6764971
Fax: +353 1 6762517
Website: www.hpra.ie
e-mail: medsafety@hpra.ie

United Kingdom
Yellow Card Scheme
Website: www.mhra.gov.uk/yellowcard or search for MHRA Yellow Card in the Google Play or Apple App Store

5. How to store Translarna

Keep this medicine out of the sight and reach of children.

Do not use this medicine after the expiry date, which is stated on the carton and sachet after 'EXP'. The expiry date refers to the last day of that month.

This medicine does not require any special storage conditions.

Take each prepared dose immediately after preparation. Discard the prepared dose if not taken within 24 hours of preparation if kept refrigerated (2 – 8 °C), or within 3 hours at room temperature (15 – 30 °C).

Do not throw away any medicines via wastewater or household waste. Ask your pharmacist how to throw away medicines you no longer use. These measures will help protect the environment.

6. Contents of the pack and other information

What Translarna contains
Translarna is available in 3 strengths, each containing 125 mg, 250 mg and 1000 mg of the active substance, called ataluren. The other ingredients are: polydextrose (E1200), macrogol, poloxamer, mannitol (E421), crospovidone, hydroxyethyl cellulose, artificial vanilla flavour (maltodextrin, artificial flavours and propylene glycol), silica, colloidal anhydrous (E551), magnesium stearate.

What Translarna looks like and contents of the pack
Translarna is white to off-white granules for oral suspension in sachets.
Translarna is available in packs containing 30 sachets.

Marketing Authorisation Holder
PTC Therapeutics International Limited
5th Floor
3 Grand Canal Plaza
Grand Canal Street Upper
Dublin 4
D04 EE70
Ireland

Manufacturer
Almac Pharma Services
22 Seagoe Industrial Estate
Craigavon BT63 5QD
United Kingdom

PTC Therapeutics International Limited
5th Floor
3 Grand Canal Plaza
Grand Canal Street Upper
Dublin 4
D04 EE70
Ireland

Almac Pharma Services (Ireland) Limited
Finnabair Industrial Estate
Dundalk, Co. Louth, A91 P9KD
Ireland

This leaflet was last revised in 06/2019

This medicine has been given 'conditional approval'. This means that there is more evidence to come about this medicine.
The European Medicines Agency will review new information on this medicine at least every year and this leaflet will be updated as necessary.

Detailed information on this medicine is available on the European Medicines Agency web site: http://www.ema.europa.eu. There are also links to other websites about rare diseases and treatments.