Patient leaflet in accordance with the Pharmacists' Regulations (Preparations) - 1986
This medicine is dispensed with a doctor's prescription only

Translarna 125 mg
Translarna 250 mg
Translarna 1000 mg
Granules for oral suspension

Name and quantity of active ingredients
Translarna 125 mg
Each sachet of granules contains 125 mg of ataluren.

Translarna 250 mg
Each sachet of granules contains 250 mg of ataluren.

Translarna 1000 mg
Each sachet of granules contains 1000 mg of ataluren.

For inactive ingredients in this product, see section 6 **'Additional information'** in this leaflet.

Read this entire leaflet carefully before you start using this medicine. This leaflet contains concise information about this medicine. If you have any further questions, consult your doctor or pharmacist.

This medicine has been prescribed for you. Do not pass it on to others. It may harm them, even if it seems to you that their medical condition is similar to yours.

1. What is this medicine intended for?
Translarna is intended for treating Duchenne muscular dystrophy caused by a mutation in the gene that allows normal muscle function. Duchenne disease is caused by a problem in a muscle protein called dystrophin. As a result, the muscles do not work properly.
Translarna enables the production of normal dystrophin and in this way helps muscles work properly.
This medicine is intended for patients with Duchenne muscular dystrophy who have been shown to carry a mutation in the dystrophin gene based on a genetic test, are aged 2 years or older, and are able to walk.

2. Before using this medicine
Do not use this medicine if:

- You are sensitive (allergic) to ataluren or to any of the other ingredients this medicine contains, see section 6 '**Additional information'**.
- You are receiving aminoglycoside antibiotics such as gentamicin, tobramycin or streptomycin by injection into a vein.

Special warnings about using this medicine:
- If you have kidney problems, you must be under regular medical supervision.
- 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 establish if treatment with Translarna is suitable for you.

Children
This medicine is not intended for children under 2 years old or children who weigh less than 12 kg, because it has not been studied in this group of patients.

Tests and follow-up:
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.

Drug interactions
If you are taking or have recently taken other medicines, including nonprescription medications and dietary supplements, tell your doctor or pharmacist. Particularly inform your doctor or pharmacist if you are taking any of the following medicines:
Do not use Translarna with aminoglycoside antibiotics such as gentamicin, tobramycin or streptomycin given by injection, because these may affect your kidney function.

- Acyclovir – usually prescribed for treatment of chickenpox [varicella],
- Adefovir – usually prescribed for treatment of chronic hepatitis B and/or HIV,
- Atorvastatin, pitavastatin, pravastatin, rosuvastatin – usually prescribed for lipid lowering,
- Benzylpenicillin – usually prescribed for severe infections,
- Bumetanide, captopril, furosemide, valsartan – usually prescribed for treatment or prevention of congestive heart failure,
- Ciprofloxacin – usually prescribed for treatment of infections,
- Famotidine – usually prescribed for treatment of active duodenal ulcer, gastroesophageal reflux disease,
- Methotrexate – usually prescribed for rheumatoid arthritis, psoriasis,
- Olmesartan – usually prescribed for essential hypertension in adults,
- Oseltamivir - usually prescribed for prevention of influenza,
- Phenobarbital – usually prescribed for sleep-inducing, prevention of seizures,
- Rifampicin – usually prescribed for treatment for tuberculosis,
- Sitagliptin – usually prescribed for type 2 diabetes.
- Some of these medicines have not been studied when given with Translarna so you must be closely monitored by your doctor.

Using this medicine and food
Take this medicine after it has been mixed with liquid or semi-solid food (yogurt or apple sauce).
See preparation instructions in section 3.

Pregnancy and breast-feeding
If you are pregnant or breast-feeding, think you might be pregnant or are planning to become pregnant, consult your doctor before taking this medicine.
If you become pregnant while taking Translarna, consult your doctor immediately as it is recommended not to take Translarna during pregnancy.
It is unknown whether the active substance in Translarna is excreted in human milk, so the risk to breastfed infants cannot be excluded. Breast-feeding should be discontinued during treatment with ataluren.

Driving and using machines
If you feel dizzy, you must not drive, cycle or operate machines.

3. How to use this medicine?
Always use this medicine according to your doctor's instructions. Check with your doctor or pharmacist if you are not sure about your dose or about how to take this medicine.
Only your doctor will determine your dose and how you should take this medicine.

The recommended dosage is usually: 10 mg per kg body weight in the morning, 10 mg per kg body weight at midday, and 20 mg per kg body weight in the evening (daily total of 40 mg per kg body weight).
Take this medicine 3 times a day at regular times. The interval between the morning dose and midday dose and between the midday dose and evening dose must be 6 hours. The interval between the evening dose and the morning dose on the following day must be 12 hours. 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.
The following table illustrates the number and strength of the sachets to be used according to body weight and time of day.

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

Be sure to drink plenty of liquids to avoid dehydration while taking this medicine.
Do not exceed the recommended dose.

How to use this medicine:
Take this medicine in a liquid or semi-solid food (such as yogurt or apple sauce).
Open the sachet only when you are about to take the medicine. Use the entire amount in the sachet. Mix the full contents of each sachet well in 30 ml of liquid (water, milk, fruit juice) or in 3 tablespoons of semi-solid food such as yogurt or apple sauce.
Mix well before use. The amount of the liquid or semi-solid food can be increased based on your preference.

If you have accidentally taken a higher dose, you may experience mild headache, nausea, vomiting or diarrhea. If you have taken an overdose, or if a child has accidentally

swallowed some medicine, immediately see a doctor or go to a hospital emergency room and bring the medicine package with you.

If you forget to take this medicine at the scheduled time:
If you are late in taking a dose by less than 3 hours after the morning or midday doses or by less than 6 hours after the evening dose, take the dose and 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, and make sure to 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 effective if you take more than the recommended dose.
Adhere to the treatment as recommended by your doctor.
Even if your health improves, do not stop taking this medicine without consulting your doctor or pharmacist.

Do not take medicines in the dark! Check the label and the dose every time you take medicine. Wear glasses if you need them. If you have any further questions about using this medicine, consult your doctor or pharmacist.

4. Side effects

Like with all medicines, using Translarna may cause side effects in some users. Do not be alarmed by this list of side effects; you may not experience any of them.

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 level of triglycerides in the blood, headache, nausea, weight loss, high blood pressure, cough, nosebleed, constipation, flatulence, abdominal discomfort, abdominal pain, rash, arm or leg pain, chest pain, involuntary urination, blood in urine, fever.

Side effects of unknown frequency: increases in blood lipids, increases in test for kidney function.

If you experience any side effect, if any side effect gets worse, or if you experience a side effect not mentioned in this leaflet, consult your doctor.

You can report side effects to the Ministry of Health by following the link 'Reporting Side Effects of Drug Treatment' on the Ministry of Health home page (www.health.gov.il) which links to an online form for reporting side effects. You can also use this link: https://sideeffects.health.gov.il

5. How to store the medicine?

Prevent poisoning! To prevent poisoning, keep this, and all other medicines, in a closed place, out of the reach and sight of children and/or infants. Do not induce vomiting unless explicitly instructed to do so by the doctor.
Do not use the medicine after the expiry date (exp. date) stated on the package. The expiry date refers to the last day of that month.
Store below 30°C.
Take immediately after preparation.
The prepared dose can be used within 24 hours if kept refrigerated (2-8°C), or within three hours if kept at room temperature (15-30°C). Otherwise, the dose must be discarded.
Do not dispose of medicines via household waste. Ask your pharmacist how to dispose of medicines that are no longer in use.

6. Additional information

In addition to the active ingredient, this medicine also contains:
mannitol, polydextrose, polyethylene glycol 3350, crospovidone, poloxamer 407, hydroxyethyl cellulose, colloidal silicon dioxide, magnesium stearate, artificial vanilla flavor.

What the medicine looks like and contents of the pack:
Translarna is a medicine in the form of white to off-white granules packaged in individual sachets. Each box contains 30 sachets.

Registration holder's name and address:
Medison Pharma Ltd., 10 Hashiloach St., P.O. Box 7090, Petach Tikva, Israel

Manufacturer's name and address:
PTC Therapeutics International Limited
Unit 1, 52-55 Sir John Rogerson's Quay,
Dublin 2, D02 NA07
Ireland

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Registration number of the medicine in the Ministry of Health's National Drug Registry:

Translarna 125 mg: 154-26-34264-00
Translarna 250 mg: 154-27-34266-00
Translarna 1000 mg: 154-28-34267-00