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 preparing an oral suspension or for swallowing with food

Each sachet of Translarna 125 mg contains 125 mg ataluren.
Each sachet of Translarna 250 mg contains 250 mg ataluren.
Each sachet of Translarna 1000 mg contains 1000 mg ataluren.

For inactive ingredients and allergens 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.
This medicine is intended to treat patients who are over 2 years old and are able to walk.

Attention:
- **This medicine is intended for patients with Duchenne muscular dystrophy, who have been diagnosed with a mutation in the dystrophin gene using a genetic test, are two years old or older, and are able to walk.**
- **Do not use this medicine if you have not been diagnosed with a dystrophin gene mutation.**
- **Do not use this medicine if you are receiving aminoglycoside antibiotics such as gentamicin, tobramycin or streptomycin by injection into a vein.**
- **If you have kidney or liver problems, you must be under regular medical supervision.**
- **If you are also being treated with steroids, there may be an increase in blood pressure, so monitoring your blood pressure is recommended.**
- **Take this medicine with food mixed with water, yogurt or apple sauce. See preparation instructions in section 3.**
- **During treatment you may also be referred for of a kidney function test and blood lipid profile.**
- **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.**

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, as listed in section 6.
- You are receiving aminoglycoside antibiotics such as gentamicin, tobramycin or streptomycin by injection into a vein.
- You have not been diagnosed with the dystrophin gene mutation.

Special warnings about using this medicine:

- If you have kidney problems, you must be under regular medical supervision.
- It has been found that changes occurred in the blood lipid profiles of patients treated with Translarna, so testing total cholesterol, LDL, HDL, and triglycerides once a year is recommended.
- If you are also being treated with steroids, there may be an increase in blood pressure, so monitoring your blood pressure every 6 months or more often is recommended, depending on your condition.

Children and adolescents

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.

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 this may affect your kidney function.
- Phenobarbital, rifampicin, adefovir, captopril, furosemide, methotrexate, oseltamivir, acyclovir, bumetanide, ciprofloxacin, famotidine, benzylpenicillin, sitagliptin, pravastatin, rosuvastatin, atorvastatin, pitavastatin, valsartan, olmesartan. 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 with food mixed with water, yogurt or apple sauce.
See preparation instructions in section 3.

Pregnancy and breastfeeding

If you are pregnant, think you might be pregnant, are planning a pregnancy or if you are breastfeeding, 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 passes into breast milk, so the risk to breastfed infants cannot be excluded.

Driving and using machines

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

3. How to use this medicine?

Always use 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.
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 granules 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 food can be increased if required.

Tests and follow-up:
During the course of treatment you may be referred to tests for kidney function, total cholesterol, LDL, HDL, and triglycerides.

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 have accidentally taken a higher dose, you may experience mild headache, nausea, vomiting or diarrhea.

If you forget to take this medicine at the scheduled time:
If you are late 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. 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 a 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 (frequency higher than 1:10): vomiting.

Common side effects (frequency is up to 1:10): decreased appetite, high level of triglycerides in the blood, headache, nausea, weight loss, elevated 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 whose frequency is unknown: increased blood lipids, increase in kidney function tests.

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 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 at 30°C or below.

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:
polydextrose, mannitol, poloxamer 407, polyethylene glycol 3350, hydroxyethyl cellulose, crospovidone, artificial vanilla flavor, colloidal silicon dioxide, magnesium stearate.

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.
P.O. Box 7090, Petach Tikva.

Manufacturer's name and address:
PTC Therapeutics International Limited
5th Floor, 3 Grand Canal Plaza, Grand Canal Street Upper, Dublin 4, D04 EE70 Ireland.

This leaflet was reviewed and approved by the Ministry of Health in March 2017 and revised in November 2019 in accordance with Ministry of Health guidelines.

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

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