Patient leaflet in accordance with the Pharmacists' Regulations (Preparations) – 1986

This medicine is dispensed with a doctor's prescription only

ELEVIDYS
$1.33x10^{13}$ vector genomes/mL
Solution for infusion
Composition:
Each vial contains:
delandistrogene moxeparvovec
$1.33x10^{13}$ vector genomes/mL

For inactive ingredients see section 6 'Additional information'.

Read the 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. Keep this leaflet. You may need to read it again.

This medicine has been prescribed to treat your illness. 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.

In addition to the patient information leaflet, Elevidys also has a patient card and a caregiver guide. These documents contain important safety information that you need to know and follow before starting and during treatment with Elevidys. Read them and the patient information leaflet before starting this medicine. Keep the documents in case you need to read them again.

1) What is this medicine intended for?

Elevidys is intended to treat children aged 4 to 5 years old who can walk (ambulatory) and have Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.

Therapeutic group: gene therapy.

Elevidys is a gene therapy medicine that contains the active substance delandistrogene moxeparvovec. Elevidys delivers a gene into the body that contains the information needed for muscles to make the Elevidys micro-dystrophin protein.

Your child will be tested by the doctor before starting treatment with Elevidys to confirm that their disease is suitable for treatment with Elevidys, see section 2.

How Elevidys works:
DMD is caused by a change (a mutation) in the DMD gene that makes a protein called dystrophin. Dystrophin is needed for the muscles to work properly. Patients with DMD are not able to make an active version of this protein.
The gene for making Elevidys micro-dystrophin protein is transferred to muscle cells using a modified virus that does not cause disease. Elevidys helps your

child's body to make Elevidys micro-dystrophin protein, a shortened version of dystrophin that helps to protect muscles from damage.

2) Before using this medicine

Do not use this medicine if:

- your child is sensitive (allergic) to delandistrogene moxeparvovec or to any of the other ingredients of this medicine (see section 6 – 'Additional information'). If you think your child may be sensitive, ask their doctor or nurse for advice.
- genetic testing shows that your child's DMD gene is missing any part of a specific section of the gene (exon 8 and/or exon 9).

If you have, or your child has, any concerns whether they need to be given Elevidys, talk to their doctor or nurse.

Special warnings about using this medicine

Before deciding on Elevidys treatment:
To help decide if Elevidys is suitable for your child, your child's doctor:

- will do, or have already done, a type of medical test that identifies changes in genes
- will check for specific antibodies (a protein produced by the body's immune system).

Tell your child's doctor or nurse before Elevidys is given if:

- your child has had any liver problems. This is important because your child may have a higher risk of developing new or worse liver problems after Elevidys treatment.
- your child is taking any medication or dietary supplements
- your child has signs of an infection (such as fever, cough, runny nose, sore throat). An infection before receiving Elevidys treatment may lead to more serious side effects.

For additional information see the section 'Tests and follow-up – Before treatment with Elevidys' and the section 'Vaccinations'.

After treatment with Elevidys, tell your child's doctor or nurse straight away if:

your child gets any of the following signs or symptoms:

Liver problems

- This medicine can lead to an increase in liver enzymes (proteins found within the body) in the blood, which may mean the liver is injured or inflamed.
- Signs or symptoms may include jaundice (yellowing of the skin or the whites of the eyes), pain in the belly, feeling tired or vomiting (see section 4 for more information).

Inflammation of the muscles

After being given Elevidys your child may experience:

- Myositis (inflammation of the muscles):
  - Signs or symptoms could include unusual muscle weakness, muscle pain or tenderness, and problems with talking (weak voice), swallowing, or breathing (see section 4 for more information).
  - This effect was seen in patients who have a specific section of the gene (exon 8 and/or exon 9) missing (see section 2 'Do not use this medicine if').
  - A risk exists for patients with other changes (known as mutations) in the DMD gene.
  - Your child's doctor may check whether the child's heart is affected.
- Myocarditis (inflammation of the heart muscle), signs or symptoms could include:
  - being short of breath, feeling tired, chest pain or discomfort, swelling of the ankles or legs, irregular heartbeat, and fainting (see section 4 for more information).

Infection

- An infection after Elevidys treatment may lead to more serious side effects.
- Signs or symptoms may include coughing, wheezing, sneezing, runny nose, sore throat, or fever.

Talk to your child's doctor or nurse if your child develops **any** side effect (see section 4 for more information).

Children and adolescents

Elevidys is intended to treat children aged 4 to 5 years old who can walk (ambulatory) and have Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene.

See the section 'Additional information for patients/parents/caregivers' for further information.

Tests and follow-up

Before treatment with Elevidys

Your child will have a blood test to check:
- liver function
- number of platelets (blood cells that prevent and stop bleeding) in their blood
- level of troponin-I (a protein that is released when the heart muscle is damaged) in their blood.

After treatment with Elevidys

It is very important to go to all follow-up visits and tests. After treatment, your child will have regular blood tests as often as directed by your child's doctor to monitor for:

- liver function, including increases in liver enzymes.
- changes in the number of platelets.
- changes in the levels of the heart protein (troponin-I).

Interactions with other medicines
If you are taking or have recently taken other medicines, including non-prescription medicines and dietary supplements, tell your doctor or pharmacist. Particularly if you are taking:

Corticosteroids

- Your child will be given another medication (corticosteroids) for about 2 months or longer as part of Elevidys treatment.
- If your child already takes corticosteroid medicine your child's doctor will tell you how the corticosteroid medicine dose will increase.
- The dose will depend on your child's weight. Your child's doctor will work out the total dose to give.
- It is important that your child receives the treatment as directed.
- The corticosteroid medicine will help manage the increase in liver enzymes that your child could develop after being given Elevidys, and the dose may be increased if needed.
- Contact your child's doctor or seek emergency help right away if your child does not take or vomits straight after taking a dose of corticosteroids.

Vaccinations

- Corticosteroids can affect the body's immune (defense) system. Your child's doctor may decide to change the timing of vaccinations (before and after Elevidys treatment).
- Consult your child's doctor or nurse if you have any questions.

Additional information for patients/parents/caregivers

Read this leaflet, the Caregiver Guide, and the Patient Card after your child is given Elevidys treatment.

Hygiene care

- The AAV vector (a modified virus) in Elevidys will temporarily pass into your child's bodily waste (urine, feces, or saliva) after treatment. This is called "shedding".
- Parents and caregivers should follow good hand-hygiene for **one month** after Elevidys treatment.
  - Wear protective gloves when handling your child's bodily waste and fluids.
  - Wash your hands thoroughly with soap and running water after handling your child's bodily waste and fluids.

- Dispose of soiled diapers (nappies) and other waste in sealed double plastic bags in household waste.
- Talk to your child's doctor or nurse if you have any questions.

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.

Elevidys will be given by a doctor or nurse trained in the treatment of your child's DMD.

Your child's doctor will work out the amount of Elevidys your child will receive according to your child's weight.

Elevidys is given intravenously (into a vein) by a single infusion over about 1-2 hours or longer if needed.

Elevidys will be given once only.

Do not exceed the recommended dose.

Carefully follow directions for treatment and follow-up as given by your child's doctor.

Do not take medicines in the dark! Check the label and 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

As with any medicine, using Elevidys may cause side effects in some users. Do not be alarmed by this list of side effects; you may not experience any of them.

Seek urgent medical attention if your child has any of these signs or symptoms after receiving Elevidys:

Common side effects (may affect up to 1 in 10 users):

- Unusual muscle weakness, muscle pain or tenderness, and problems with talking (weak voice), swallowing, or breathing – these may be signs of inflammation of the muscles (myositis – see section 2).

Side effects whose frequency is unknown (effects whose frequency has not been established yet):

- Jaundice (yellowing of the skin or the whites of the eyes), pain in the belly, feeling tired or vomiting – these may be signs of injury to the liver.
- Being short of breath, feeling tired, chest pain or discomfort, swelling of the ankles or legs, irregular heartbeat, and fainting – these may be signs of possible problems with the heart.

Talk to your child's doctor or nurse if your child develops any other side effects. These can include:
Very common side effects (may affect more than 1 in 10 users):

- vomiting
- nausea
- increases in liver enzymes seen in blood tests
- fever
- decrease in the number of platelets seen in blood 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.

Some side effects could appear some time after treatment rather than immediately after it.

Reporting side effects

You can report side effects to the Ministry of Health by following the 'Reporting Side Effects of Drug Treatment' link on the Ministry of Health home page (www.health.gov.il), which opens an online form for reporting side effects, or 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 your doctor.

The following information is for the healthcare professionals who will prepare and give the medicine.

- Keep this medicine out of the reach and sight of children.
- Vials will be transported frozen (at or below -60°C). Upon receipt vials should be stored frozen (at or below -60°C).
- This medicine can be stored in a refrigerator at 2°C–8°C.
  - Once refrigerated, the medicine must be used within 14 days.
  - Write the new expiry date on the medicine's outer package before storing in the refrigerator.
  - After thawing, do not refreeze and do not shake.

- Do not place the medicine back in the refrigerator once brought to room temperature.
- Do not use this medicine after the expiry date which is stated on the vial label and outer package. The expiry date shown on the outer package refers to the medicine's shelf-life when stored at or below -60°C. The expiry date refers to the exact day of the month (DD-MM-YYYY).
- This medicine contains genetically modified organisms. Unused medicine and waste material must be disposed of in compliance with the local guidelines on handling biological waste.

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

6) Additional information

What does Elevidys contain?

- The active ingredient is delandistrogene moxeparvovec. Each mL of delandistrogene moxeparvovec contains 1.33×10^{13} vector genomes.

In addition to the active ingredients, this medicine also contains:
sodium chloride, tromethamine HCL, tromethamine, magnesium chloride, poloxamer 188 and water for injection.

What the medicine looks like and contents of the pack:

Elevidys is a clear, colorless liquid for infusion, which may have some opalescence and may contain white to off-white particles.

Elevidys is supplied in 10 mL vials. Each vial is for single use only.

Each pack will contain either 10 to 35 vials, or 36 to 70 vials.

Registration holder's name and address: Roche Pharmaceuticals (Israel) Ltd., POB 6391, Hod HaSharon 4524079.

Manufacturer's name and address: F. Hoffmann-La Roche Ltd., Grenzacherstrasse 124, CH-4070 Basel, Switzerland.

This leaflet was reviewed and approved by the Ministry of Health in June 2024.

Registration number of the medicine in the Ministry of Health's National Drug Registry:
176-89-37834-00