

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

This medicine is dispensed with a doctor's prescription only

**ELEVIDYS<sup>®</sup>**

**1.33x10<sup>13</sup> vector genomes/mL**

Solution for infusion

Composition:

Each vial contains:

**delandistrogene moxeparvovec**

**1.33x10<sup>13</sup> 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 that you should follow before starting and during treatment with Elevidys. Read them and the patient information leaflet before starting to use 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 over the age of 4 years and below the age of 8 years 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 that contains the active ingredient delandistrogene moxeparvovec.

The doctor will test your child before starting treatment with Elevidys to confirm that this medicine is a suitable treatment, see section 2.

**How Elevidys works:**

DMD is caused by a change - called a mutation - in the DMD gene

- The DMD gene makes a protein called dystrophin.
- Dystrophin is needed for the muscles to work properly.

People with DMD are not able to make a version of this protein that works.

Elevidys delivers a gene into the body. This gene contains the information needed for muscles to make Elevidys micro-dystrophin protein. This is a shortened and working version of dystrophin. This helps to protect muscles from damage. The gene for making Elevidys micro-dystrophin protein is transferred to muscle cells using a modified virus that does not cause disease.

## **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 in this medicine (see section 6 – ‘Additional information’).
- genetic testing shows that your child's DMD gene is missing any part of a specific section of the gene (called exon 8 and/or exon 9).

### **Special warnings about using this medicine**

#### **Before deciding on Elevidys treatment:**

To help decide if Elevidys is suitable, your doctor:

- will have done a medical test that looks for specific changes in the DMD gene.
- will have checked for specific antibodies - a protein produced by the body's immune system.

#### **Tell your child's doctor or nurse before treatment with Elevidys 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.
- An infection before receiving Elevidys may lead to more serious side effects.
  - You need to look out for signs of an infection such as coughing, wheezing, sneezing, runny nose, sore throat or fever.
  - Tell your doctor or nurse straight away if your child has any of these signs or symptoms.

For additional information, see ‘Tests and follow-up – before treatment with Elevidys’ and ‘Vaccinations’.

#### **After treatment with Elevidys, tell your doctor or nurse straight away if:**

Elevidys can cause some side effects that you need to tell your doctor or nurse about straight away (see section 4 ‘Side effects’ for more information).

#### **Infusion-related reactions**

- Infusion-related side effects can happen during or after administration of Elevidys. See section 4 for details of the signs of infusion-related reactions.
- Depending on the symptoms, the infusion may be slowed down or stopped.
- Your doctor may decide that additional medication needs to be given.
- If the treatment can be restarted, it may be given at a slower rate.

### Liver problems

This medicine can lead to an increase in liver enzymes (proteins found within the body) in the blood. This may mean the liver is injured or inflamed. See section 4 for details of the signs of liver problems.

### Inflamed muscles (myositis)

- Inflamed muscles (called myositis) - in muscles that help you move - can occur after treatment with Elevidys. See section 4 for details of the signs of muscle problems.
- This effect was seen in patients who have a specific section of the DMD gene (called exon 8 or exon 9) missing. Patients with other changes (known as mutations) in the DMD gene may also be at risk.
- Your doctor may check whether the heart is affected.

### Inflamed heart muscle (myocarditis)

- Inflamed heart muscle (called myocarditis) can occur after treatment with Elevidys. See section 4 for details of the signs of heart muscle problems.
- Patients with 'cardiomyopathy' - a disease of the heart muscle affecting its function, which can affect the heart's pumping ability - may be at risk of more serious side effects.

Tell your doctor or nurse straight away if your child has any of these side effects during or after treatment with Elevidys:

### Infection

- An infection after Elevidys treatment may lead to more serious side effects.
- You need to look out for signs of an infection such as coughing, wheezing, sneezing, runny nose, sore throat, or fever.
- Tell your doctor or nurse straight away if your child has any of these signs or symptoms.

### **Children and adolescents**

Elevidys is intended to treat children over the age of 4 years and under the age of 8 years 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 health

- number of platelets - these are blood cells that prevent and stop bleeding.
- levels of troponin-I - a protein that is released in the blood when the heart muscle is damaged.

### **After treatment with Elevidys**

It is very important to go to all follow-up visits and tests. After treatment, there will be regular blood tests as often as directed by the doctor to check for:

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

### **Interactions with other medicines**

**If you are taking or have recently taken other medicines, including nonprescription medicines and dietary supplements, tell your doctor or pharmacist.** Particularly if you are taking:

#### Corticosteroids

- Your child will also be given another medication (corticosteroids). This will be for about 2 months or longer as part of Elevidys treatment.
- If your child already takes corticosteroid medicine, your doctor will tell you how the corticosteroid medicine dose will increase.
- The dose will depend on your child's weight. The doctor will work out the dose.
- Your child must receive the treatment according to the instructions.
- Corticosteroid medicine will help manage the increase in liver enzymes that may occur after treatment with Elevidys. The dose may be increased if needed.
- Contact your doctor or seek emergency help straight 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 doctor may decide to change the timing of vaccinations before and after Elevidys treatment.
- Consult your doctor or nurse if you have any questions.

### **Additional information for patients/parents/caregivers**

#### Good hand hygiene

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

- Dispose of soiled diapers and other waste in sealed double plastic bags in household waste.
- Consult the 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. Elevidys will be given in a medical center.

#### **Elevidys will be given ONCE only.**

The doctor will work out the amount of Elevidys that will be given according to your child's weight.

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

#### **Do not exceed the recommended dose.**

Carefully follow directions for treatment and follow-up as given by your 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 treatment with Elevidys – urgent medical treatment may be needed:

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

- Fast heartbeat (tachycardia), fast breathing, swollen lips, being short of breath, nostrils widening (nasal flaring), hives (urticaria), red and blotchy skin, itchy or inflamed lips, itchy spots - this may be a sign of infusion-related side effects (infusion related reactions).

**Uncommon side effects** (may affect up to 1 in 100 users):

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

**Side effects of unknown frequency** (the frequency of these effects has not been established yet):

- Yellowing of the skin or the whites of the eyes (jaundice), pain in the tummy, feeling tired or being sick(vomiting) – these may be signs of liver problems.
- 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 heart problems (myocarditis).

Consult your 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):

- nausea
- increase in liver enzymes seen in blood tests
- fever

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

- 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](http://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.
- 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.

- 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 it in the refrigerator.
  - After thawing, do not refreeze and do not shake.
- Before opening/preparation can be kept at temperature up to 25°C, up to 24 hours, when stored in the original package.
- Do not place the medicine back in the refrigerator once brought to room temperature.
- 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 via wastewater or household waste. Ask the pharmacist how to dispose of 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  $1.33 \times 10^{13}$  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 Elevidys 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 contains 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-4058 Basel, Switzerland

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