# Elevidys

delandistrogene moxeparvovec-rokl

suspension for intravenous infusion

# TREATMENT GUIDE

## FOR US HEALTHCARE PROVIDERS

**Please see Important Safety Information on pages 3-4 and full Prescribing Information.**

# Introduction to ELEVIDYS

ELEVIDYS (delandistrogene moxeparvovec-rokl) is an adeno-associated virus vector-based gene therapy indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. ELEVIDYS is administered as a single intravenous infusion over 1 to 2 hours, but there are essential steps to treatment before and after infusion day.

## This guide provides information on:



**INDICATION**
ELEVIDYS is indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. This indication is approved under accelerated approval based on the expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

**IMPORTANT SAFETY INFORMATION**
**CONTRAINDICATION**
ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Important Safety Information

## INDICATION

ELEVIDYS is indicated for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the *DMD* gene. This indication is approved under accelerated approval based on the expression of ELEVIDYS micro-dystrophin observed in patients treated with ELEVIDYS. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

## IMPORTANT SAFETY INFORMATION

## CONTRAINDICATION

ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene.

## WARNINGS AND PRECAUTIONS

**Acute serious liver injury**

Acute serious liver injury has been observed with ELEVIDYS, and administration may result in elevations of liver enzymes (such as GGT, GLDH, ALT, AST) or total bilirubin, typically seen within 8 weeks.

Patients with preexisting liver impairment, chronic hepatic condition, or acute liver disease (eg, acute hepatic viral infection) may be at higher risk of acute serious liver injury. Postpone ELEVIDYS administration in patients with acute liver disease until resolved or controlled.

Prior to ELEVIDYS administration, perform liver enzyme test and monitor liver function (clinical exam, GGT, and total bilirubin) weekly for the first 3 months following ELEVIDYS infusion. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam, GGT, and total bilirubin levels return to near baseline levels).

Systemic corticosteroid treatment is recommended for patients before and after ELEVIDYS infusion. Adjust corticosteroid regimen when indicated. If acute serious liver injury is suspected, consultation with a specialist is recommended.

**Please see additional Important Safety Information on page 4 and full Prescribing Information.**

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# **Important Safety Information** (continued)

## WARNINGS AND PRECAUTIONS (CONTINUED)

**Immune-mediated myositis**

In clinical trials, immune-mediated myositis has been observed approximately 1 month following ELEVIDYS infusion in patients with deletion mutations involving exon 8 and/or exon 9 in the *DMD* gene. Symptoms of severe muscle weakness, including dysphagia, dyspnea, and hypophonia, were observed.

Limited data are available for ELEVIDYS treatment in patients with mutations in the *DMD* gene in exons 1 to 17 and/or exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction.

Advise patients to contact a physician immediately if they experience any unexplained increased muscle pain, tenderness, or weakness, including dysphagia, dyspnea, or hypophonia, as these may be symptoms of myositis. Consider additional immunomodulatory treatment (immunosuppressants [eg, calcineurin-inhibitor] in addition to corticosteroids) based on the patient's clinical presentation and medical history if these symptoms occur.

**Myocarditis**

Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials.

Monitor troponin-I before ELEVIDYS infusion and weekly for the first month following infusion, and continue monitoring if clinically indicated. More frequent monitoring may be warranted in the presence of cardiac symptoms, such as chest pain or shortness of breath.

Advise patients to contact a physician immediately if they experience cardiac symptoms.

**Preexisting immunity against AAVrh74**

In AAV-vector based gene therapies, preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels. Following treatment with ELEVIDYS, all subjects developed anti-AAVrh74 antibodies.

Perform baseline testing for presence of anti-AAVrh74 total binding antibodies prior to ELEVIDYS administration.

ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers greater than or equal to 1:400.

**Adverse reactions**

The most common adverse reactions (incidence ≥5%) reported in clinical studies were vomiting, nausea, liver function tests increased, pyrexia, and thrombocytopenia.

Report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

**Please see additional Important Safety Information on page 3 and full Prescribing Information.**

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# Patient counseling information

## It's important to review the potential risks of ELEVIDYS treatment with caregivers. Below are key topics to discuss:

**Acute serious liver injury**

ELEVIDYS can increase certain liver enzyme levels and cause acute serious liver injury. Patients will receive oral corticosteroid medication before and after infusion with ELEVIDYS. Weekly blood tests will be required to monitor liver enzyme levels for 3 months after treatment. Contact a healthcare provider immediately if the patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of corticosteroid or vomits it up.

**Immune-mediated myositis**

Immune-mediated myositis (an immune response affecting muscles) was observed in patients with a deletion mutation in the *DMD* gene that is contraindicated. Contact a physician immediately if the patient experiences any unexplained increased muscle pain, tenderness, or weakness, including difficulty swallowing, difficulty breathing, or difficulty speaking, as these may be symptoms of myositis.

**Myocarditis**

Myocarditis (inflammation of the heart) has been observed within days following ELEVIDYS infusion. Weekly monitoring of troponin-I for the first month after treatment is required. Contact a healthcare provider immediately if the patient begins to experience chest pain and/or shortness of breath.

**Vaccination**

Patient's immunizations should be up-to-date with current immunization guidelines prior to initiation of the corticosteroid regimen required before ELEVIDYS infusion. Vaccinations should be completed at least 4 weeks prior to initiation of the corticosteroid regimen.

**Concurrent infections**

Due to the concomitant administration of corticosteroids, an infection (eg, cold, flu, gastroenteritis, otitis media, bronchiolitis, etc) before or after ELEVIDYS infusion could lead to more serious complications. Contact a healthcare provider immediately if symptoms suggestive of infection are observed (eg, coughing, wheezing, sneezing, runny nose, sore throat, or fever).

**Vector shedding**

Vector shedding of ELEVIDYS occurs primarily through body waste. Practice proper hand hygiene, such as hand washing, when coming into direct contact with patient body waste. Place potentially contaminated materials that may have the patient's bodily fluids/waste in a sealable bag, and dispose into regular trash. These precautions should be followed for 1 month after ELEVIDYS infusion.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Determining who is eligible for ELEVIDYS

## Review the genetic test report to confirm the patient's *DMD* mutation

- ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the *DMD* gene due to the increased risk for a severe immune-mediated myositis reaction
- Limited data are available for ELEVIDYS treatment in patients with mutations in the *DMD* gene in exons 1 to 17 and/or exons 59 to 71. Patients with deletions in these regions may be at risk for a severe immune-mediated myositis reaction

## Perform testing for anti-AAVrh74 total binding antibody levels

- Preexisting anti-AAV antibodies may impede transgene expression at desired therapeutic levels
- Select patients for treatment who have anti-AAVrh74 total binding antibody titers <1:400
- Measure baseline anti-AAVrh74 antibody titers using a total binding antibody enzyme-linked immunosorbent assay (ELISA)
- The safety and efficacy of ELEVIDYS in patients with elevated anti-AAVrh74 total binding antibody titer (≥1:400) have not been evaluated
- ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers (≥1:400)
- For more information on how to access this testing, please call SareptAssist at 1-888-SAREPTA (1-888-727-3782)

## Perform baseline testing for:

- Liver function (clinical exam, GGT,* and total bilirubin)
- Platelet counts
- Troponin-I levels

The Sarepta Gene Therapy Enrollment Form is required to verify eligibility for ELEVIDYS and initiate the process to receive therapy. Submit the fully completed form to **SareptAssist** via fax at **1-800-621-5203** or via email at **SareptAssist@Sarepta.com**.

SAREPTASSIST

If you have questions, SareptAssist Case Managers are available to assist.

1-888-SAREPTA (1-888-727-3782)

Monday through Friday, 8:30am – 6:30pm ET

* GGT=gamma-glutamyl transferase.

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# Proper storage and handling of ELEVIDYS

ELEVIDYS is shipped and delivered frozen (≤-60°C [-76°F]) in 10 mL vials

ELEVIDYS can be refrigerated for up to 14 days when stored at 2°C to 8°C (36°F to 46°F) in the upright position

Do not refreeze

Do not shake

Do not place back in the refrigerator once brought to room temperature

Follow local guidelines on handling of biological waste

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Preparing for infusion: Planning

## Testing

- Ensure all baseline testing has been completed for liver function (clinical exam, GGT,* and total bilirubin), platelet count, and troponin-I levels

## Weight

- The recommended dose of ELEVIDYS is $1.33 \times 10^{14}$ vector genomes per kilogram (vg/kg) of body weight (or 10 mL/kg body weight)
- Calculate the dose as follows:

  ELEVIDYS dose (in mL) = patient body weight (in kilogram) × 10

  *The multiplication factor 10 represents the per kilogram dose ($1.33 \times 10^{14}$ vg/kg) divided by the amount of vector genome copies per mL of the ELEVIDYS suspension ($1.33 \times 10^{13}$ vg/mL)*
- Number of ELEVIDYS vials needed = ELEVIDYS dose (in mL) divided by 10 (round to the nearest number of vials)

  Example: Calculation of volume needed for a 19.6 kg patient

  19.6 kg × 10 = 196 mL

  Number of ELEVIDYS vials needed = 196 divided by 10, rounded to the nearest number of vials = 20 vials
- For additional information on the number of vials required, please refer to page 12. For additional information on dosage and administration, please see the Prescribing Information

## Antibody test completion

- Complete a confirmatory AAVrh74 Antibody Test for the presence of anti-AAVrh74 total binding antibodies. ELEVIDYS administration is not recommended in patients with elevated anti-AAVrh74 total binding antibody titers (≥1:400)

**REMINDER**

Due to the increased risk of serious systemic immune response, postpone ELEVIDYS in patients with infections until the infection has resolved. Clinical signs or symptoms of infection should not be evident at the time of ELEVIDYS administration.

* GGT=gamma-glutamyl transferase.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Preparing for infusion: Premedication

## Corticosteroid treatment

- Immune responses to the AAVrh74 vector can occur after the administration of ELEVIDYS. Administer corticosteroids starting at least 1 day before ELEVIDYS infusion to reduce the risk associated with an immune response. Follow the appropriate schedule below
- Note: This regimen is recommended for a minimum of 60 days after the infusion, unless earlier tapering is clinically indicated (see page 16 for more information)

**Recommended pre- and post-infusion corticosteroid dosing**

| Baseline corticosteroid dosing[a] | Peri-ELEVIDYS infusion corticosteroid dose (prednisone equivalent)[b] | Recommended maximum total daily dose (prednisone equivalent)[b] |
|---|---|---|
| Daily or intermittent dose | Start **1 day** prior to infusion: 1 mg/kg/day (and continue baseline dose) | 60 mg/day |
| High dose for 2 days per week | Start **1 day** prior to infusion: 1 mg/kg/day taken on days without high-dose corticosteroid treatment (and continue baseline dose) | 60 mg/day |
| Not on corticosteroids | Start **1 week** prior to infusion: 1.5 mg/kg/day | 60 mg/day |

[a] Patient continues to receive this dose.

[b] Deflazacort is not recommended for use as a peri-ELEVIDYS infusion corticosteroid.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Administering ELEVIDYS

## General precautions

- Prepare ELEVIDYS using aseptic technique
- Verify the required dose of ELEVIDYS based on the patient's body weight
- Confirm that the kit contains sufficient number of vials to prepare the ELEVIDYS infusion for the patient
- Visually inspect parenteral drug products for particulate matter and discoloration prior to administration, whenever suspension and container permit. ELEVIDYS may contain white to off-white particles

## Recommended supplies and materials

- 60 mL siliconized polypropylene syringes
- 21-gauge or smaller stainless steel needles (eg, 22- or 23-gauge needles are acceptable)

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# **Administering ELEVIDYS** (continued)

## Preparing ELEVIDYS infusion

1. Thaw ELEVIDYS before use
   - When thawed in the refrigerator, ELEVIDYS vials are stable for up to 14 days in the refrigerator (2°C to 8°C [36°F to 46°F]) when stored in the upright position
   - Frozen ELEVIDYS vials will thaw in approximately 2 hours when placed at room temperature (up to 25°C [77°F]) when removed from original packaging

2. Inspect vials to ensure no ice crystals are present prior to preparation

3. When thawed, swirl gently
   - Do not shake
   - Do not refreeze
   - Do not place back in the refrigerator

4. Visually inspect each vial of ELEVIDYS. ELEVIDYS is a clear, colorless liquid that may have some opalescence. ELEVIDYS may contain white to off-white particles
   - Do not use if the suspension in the vials is cloudy or discolored

5. Remove the plastic flip-off cap from the vials and disinfect the rubber stopper with a sterilizing agent (eg, alcohol wipes)


6. Withdraw 10 mL of ELEVIDYS from each vial provided in the customized ELEVIDYS kit (refer to the table on page 12)
   - Do not use filter needles during preparation of ELEVIDYS
   - Multiple syringes will be required to withdraw the required volume
   - Remove air from the syringes and cap the syringes

7. Maintain syringes at room temperature prior to and during administration
   - Sealed ELEVIDYS thawed vials are stable up to 24 hours at room temperature (up to 25°C [77°F]) when stored in upright position

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Administering ELEVIDYS (continued)

## ELEVIDYS multivial kits

| Patient Weight (kg) | Total Vials per Kit | Total Dose Volume per Kit (mL) | NDC Number |
|---|---|---|---|
| 10.0 - 10.4 | 10 | 100 | 60923-501-10 |
| 10.5 - 11.4 | 11 | 110 | 60923-502-11 |
| 11.5 - 12.4 | 12 | 120 | 60923-503-12 |
| 12.5 - 13.4 | 13 | 130 | 60923-504-13 |
| 13.5 - 14.4 | 14 | 140 | 60923-505-14 |
| 14.5 - 15.4 | 15 | 150 | 60923-506-15 |
| 15.5 - 16.4 | 16 | 160 | 60923-507-16 |
| 16.5 - 17.4 | 17 | 170 | 60923-508-17 |
| 17.5 - 18.4 | 18 | 180 | 60923-509-18 |
| 18.5 - 19.4 | 19 | 190 | 60923-510-19 |
| 19.5 - 20.4 | 20 | 200 | 60923-511-20 |
| 20.5 - 21.4 | 21 | 210 | 60923-512-21 |
| 21.5 - 22.4 | 22 | 220 | 60923-513-22 |
| 22.5 - 23.4 | 23 | 230 | 60923-514-23 |
| 23.5 - 24.4 | 24 | 240 | 60923-515-24 |
| 24.5 - 25.4 | 25 | 250 | 60923-516-25 |
| 25.5 - 26.4 | 26 | 260 | 60923-517-26 |
| 26.5 - 27.4 | 27 | 270 | 60923-518-27 |
| 27.5 - 28.4 | 28 | 280 | 60923-519-28 |
| 28.5 - 29.4 | 29 | 290 | 60923-520-29 |
| 29.5 - 30.4 | 30 | 300 | 60923-521-30 |
| 30.5 - 31.4 | 31 | 310 | 60923-522-31 |
| 31.5 - 32.4 | 32 | 320 | 60923-523-32 |
| 32.5 - 33.4 | 33 | 330 | 60923-524-33 |
| 33.5 - 34.4 | 34 | 340 | 60923-525-34 |
| 34.5 - 35.4 | 35 | 350 | 60923-526-35 |
| 35.5 - 36.4 | 36 | 360 | 60923-527-36 |
| 36.5 - 37.4 | 37 | 370 | 60923-528-37 |
| 37.5 - 38.4 | 38 | 380 | 60923-529-38 |
| 38.5 - 39.4 | 39 | 390 | 60923-530-39 |
| 39.5 - 40.4 | 40 | 400 | 60923-531-40 |

| Patient Weight (kg) | Total Vials per Kit | Total Dose Volume per Kit (mL) | NDC Number |
|---|---|---|---|
| 40.5 - 41.4 | 41 | 410 | 60923-532-41 |
| 41.5 - 42.4 | 42 | 420 | 60923-533-42 |
| 42.5 - 43.4 | 43 | 430 | 60923-534-43 |
| 43.5 - 44.4 | 44 | 440 | 60923-535-44 |
| 44.5 - 45.4 | 45 | 450 | 60923-536-45 |
| 45.5 - 46.4 | 46 | 460 | 60923-537-46 |
| 46.5 - 47.4 | 47 | 470 | 60923-538-47 |
| 47.5 - 48.4 | 48 | 480 | 60923-539-48 |
| 48.5 - 49.4 | 49 | 490 | 60923-540-49 |
| 49.5 - 50.4 | 50 | 500 | 60923-541-50 |
| 50.5 - 51.4 | 51 | 510 | 60923-542-51 |
| 51.5 - 52.4 | 52 | 520 | 60923-543-52 |
| 52.5 - 53.4 | 53 | 530 | 60923-544-53 |
| 53.5 - 54.4 | 54 | 540 | 60923-545-54 |
| 54.5 - 55.4 | 55 | 550 | 60923-546-55 |
| 55.5 - 56.4 | 56 | 560 | 60923-547-56 |
| 56.5 - 57.4 | 57 | 570 | 60923-548-57 |
| 57.5 - 58.4 | 58 | 580 | 60923-549-58 |
| 58.5 - 59.4 | 59 | 590 | 60923-550-59 |
| 59.5 - 60.4 | 60 | 600 | 60923-551-60 |
| 60.5 - 61.4 | 61 | 610 | 60923-552-61 |
| 61.5 - 62.4 | 62 | 620 | 60923-553-62 |
| 62.5 - 63.4 | 63 | 630 | 60923-554-63 |
| 63.5 - 64.4 | 64 | 640 | 60923-555-64 |
| 64.5 - 65.4 | 65 | 650 | 60923-556-65 |
| 65.5 - 66.4 | 66 | 660 | 60923-557-66 |
| 66.5 - 67.4 | 67 | 670 | 60923-558-67 |
| 67.5 - 68.4 | 68 | 680 | 60923-559-68 |
| 68.5 - 69.4 | 69 | 690 | 60923-560-69 |
| 69.5 and above | 70 | 700 | 60923-561-70 |

**For questions, contact Sarepta Medical Information (1-888-727-3782, option 2).**

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# **Administering ELEVIDYS** (continued)

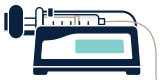
## Recommended supplies and materials

- Syringe infusion pump
- 0.2-micron PES* in-line filter
- PVC† (non-DEHP‡), polyurethane IV infusion tubing, and catheter

**Administer ELEVIDYS as a single-dose intravenous infusion through a peripheral venous catheter. ELEVIDYS should be infused at a rate of less than 10 mL/kg/hour.**

- Consider application of a topical anesthetic to the infusion site prior to administration of IV insertion
- Recommend inserting a back-up catheter

1. Flush the intravenous access line prior to the ELEVIDYS infusion at the same infusion rate



2. Administer ELEVIDYS via intravenous infusion using a syringe infusion pump with an in-line 0.2-micron filter at a duration of approximately 1 to 2 hours, or longer at care team discretion, through a peripheral limb vein

3. Infuse at a rate of less than 10 mL/kg/hour
   - Do not administer ELEVIDYS as an intravenous push
   - Do not infuse ELEVIDYS in the same intravenous access line with any other product
   - Use ELEVIDYS within 6 hours after drawing into syringe. Discard the ELEVIDYS-containing syringe(s) if infusion of the drug has not started within the 6-hour timeframe

4. Flush the intravenous access line with 0.9% Sodium Chloride Injection after the ELEVIDYS infusion
   - Discard unused ELEVIDYS
   - Dispose of the needle and syringe

**REMINDER**

Due to the increased risk of serious systemic immune response, postpone ELEVIDYS administration in patients with infections until the infection has resolved. Clinical signs or symptoms of infection should not be evident at the time of ELEVIDYS administration.

* PES=polyether sulfone.

† PVC=polyvinyl chloride.

‡ DEHP=di(2-ethylhexyl)phthalate.

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# Postinfusion: Safety

## Safety considerations for ELEVIDYS

The most common adverse reactions (incidence ≥5%) reported in clinical studies were vomiting, nausea, liver function test increased, pyrexia, and thrombocytopenia.

Adverse reactions were typically seen within the first 2 weeks (nausea, vomiting, thrombocytopenia, pyrexia), or within the first 2 months (immune-mediated myositis, liver function test increased). Vomiting may occur as early as on the day of the infusion.

The safety data described in this section reflect exposure to a one-time intravenous infusion of ELEVIDYS in 85 male subjects with a confirmed mutation of the *DMD* gene in 3 ongoing clinical studies, including 2 open-label studies and 1 study that included a double-blind, placebo-controlled period. Prior to ELEVIDYS infusion, patients in the ELEVIDYS treatment group had a mean age of 7.08 years (range: 3 to 20) and mean weight of 25.91 kg (range: 12.5 to 80.1). Seventy-three subjects received the recommended dose of $1.33 \times 10^{14}$ vg/kg, and 12 received a lower dose. The table below presents adverse reactions from these 3 clinical studies.

**Adverse reactions (incidence ≥5%) following treatment with ELEVIDYS in clinical studies**

| Adverse reactions | ELEVIDYS (n=85) % |
|---|---|
| Vomiting | 61 |
| Nausea | 40 |
| Liver function test increased[a] | 37 |
| Pyrexia | 24 |
| Thrombocytopenia[b] | 12 |

[a] Includes: AST* increased, ALT† increased, GGT‡ increased, GLDH§ increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased.
[b] Transient, mild, asymptomatic decrease in platelet counts.

**Adverse reactions occurring in ELEVIDYS-treated subjects and at least 10% more frequently than in placebo in Study 1, Part 1**

| Adverse reactions | ELEVIDYS (n=20) % | Placebo (n=21) % |
|---|---|---|
| Vomiting | 65 | 33 |
| Nausea | 35 | 10 |
| Liver function test increased[a] | 25 | 0 |
| Pyrexia | 20 | 5 |

[a] Includes: AST* increased, ALT† increased, GGT‡ increased, GLDH§ increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased.

Report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Sarepta Therapeutics at 1-888-SAREPTA (1-888-727-3782).

* AST=aspartate transferase.
† ALT=alanine transaminase.
‡ GGT=gamma-glutamyl transferase.
§ GLDH=glutamate dehydrogenase.

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# Postinfusion: Monitoring

## Posttreatment assessment schedule

After infusion, monitor your patient weekly for at least 3 months. Assess liver function, platelet count, and troponin-I levels, and refer to the table below for a schedule of assessments. Continue monitoring if clinically indicated. The post-ELEVIDYS monitoring form on page 18 may be a helpful reference for each patient.

| | Month 1 | | | | Month 2 | | | | Month 3 | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | WEEK 1 | WEEK 2 | WEEK 3 | WEEK 4 | WEEK 1 | WEEK 2 | WEEK 3 | WEEK 4 | WEEK 1 | WEEK 2 | WEEK 3 | WEEK 4 |
| LIVER FUNCTION | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ | ✓ |
| PLATELET COUNT | ✓ | ✓ | | | | | | | | | | |
| TROPONIN-I LEVELS | ✓ | ✓ | ✓ | ✓ | | | | | | | | |

Continue monitoring if clinically indicated.

### Liver function

- Acute serious liver injury has been observed with ELEVIDYS. Administration of ELEVIDYS may result in elevations of liver enzymes (eg, GGT,* ALT†) and total bilirubin, typically seen within 8 weeks
- Monitor liver function weekly for the first 3 months via clinical exam and analysis of GGT and total bilirubin levels. Continue monitoring if clinically indicated, until results are unremarkable (normal clinical exam and GGT and total bilirubin levels return to near baseline levels)
- If acute serious liver injury is suspected, a consultation with a specialist is recommended
- The recommended postinfusion corticosteroid regimen dose modification for patients with liver function abnormalities is detailed on the following page and in the full Prescribing Information

### Platelet count

- Transient, mild, asymptomatic decrease in platelet count has been observed following ELEVIDYS administration
- Monitor platelet count weekly for the first 2 weeks
- Continue monitoring if clinically indicated

### Myocarditis

- Acute serious myocarditis and troponin-I elevations have been observed following ELEVIDYS infusion in clinical trials
- Monitor troponin-I weekly for the first month following infusion. More frequent monitoring may be required in the presence of cardiac symptoms, such as chest pain or shortness of breath
- Continue monitoring if clinically indicated

* GGT=gamma-glutamyl transferase.
† ALT=alanine transaminase.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Postinfusion: Medication

## Postinfusion corticosteroid treatment

Immune responses to the ELEVIDYS vector (AAVrh74) can occur after administration of ELEVIDYS. To reduce the risk associated with an immune response, continue treatment with systemic corticosteroids for at least 60 days after the infusion, unless earlier tapering is clinically indicated. Follow the appropriate schedule in the table on page 9.

**Reminder:** The patient must start the regimen prior to ELEVIDYS infusion.

**Recommended corticosteroid regimen dose modification for liver function abnormalities following ELEVIDYS infusion[a]**

| Peri-ELEVIDYS infusion corticosteroid dosing | Modified corticosteroid dose following ELEVIDYS infusion (prednisone equivalent)[b] | Recommended maximum total daily dose (prednisone equivalent)[b] |
|---|---|---|
| Baseline + 1 mg/kg/day | Increase to 2 mg/kg/day (and continue baseline dose) | 120 mg/day |
| Baseline + 1 mg/kg/day taken on days without high-dose corticosteroid treatment | Increase to 2 mg/kg/day taken on days without high-dose corticosteroid treatment (and continue baseline dose) | 120 mg/day |
| 1.5 mg/kg/day | Increase from 1.5 mg/kg/day to 2.5 mg/kg/day | 120 mg/day |

[a] GGT≥150 U/L and/or other clinically significant liver function abnormalities (eg, total bilirubin >2 × ULN) following infusion. For GGT* or bilirubin elevations that do not respond to these oral corticosteroid increases, IV bolus corticosteroids may be considered.

[b] Deflazacort is not recommended for use as a peri-ELEVIDYS infusion corticosteroid.

## For patients previously taking corticosteroids at baseline:

Taper off the additional peri-ELEVIDYS corticosteroids (back to baseline corticosteroid dose) over 2 weeks, or longer, as needed.

## For patients not previously taking corticosteroids at baseline:

Taper the added peri-ELEVIDYS corticosteroids off (back to no corticosteroids) over 4 weeks, or longer, as needed, and the corticosteroids should not be stopped abruptly.

* GGT=gamma-glutamyl transferase.

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Postinfusion guidance for caregivers

## Important risks

**Speak with your patient's caregiver about the risk of acute serious liver injury, immune-mediated myositis, and myocarditis**

- Advise caregivers to contact you immediately if:
  - The patient's skin and/or whites of the eyes appear yellowish, or if the patient misses a dose of corticosteroid or vomits it up
  - The patient experiences any unexplained increased muscle pain, tenderness, or weakness, including difficulty swallowing, difficulty breathing, or difficulty speaking, as these may be symptoms of myositis
  - The patient begins to experience chest pain and/or shortness of breath, as this may be a sign of myocarditis

## Corticosteroids

**Remind caregivers about the importance of corticosteroid treatment to help reduce the risk of an immune response to the ELEVIDYS vector (AAVrh74). Review the recommended postinfusion dosing schedule within this guide and within the full Prescribing Information**

## Monitoring

**Speak with your patient's caregiver about weekly monitoring of liver function, troponin-I levels, and platelet counts postinfusion. Review the schedule for assessments on page 15**

## Concurrent infections

**Speak with your patient's caregiver about infections due to the concomitant administration of corticosteroids before and after ELEVIDYS infusion**

- Caregivers should be aware that an infection (eg, cold, flu, gastroenteritis, otitis media, bronchiolitis, etc) before or after infusion could lead to more serious complications
- Advise caregivers to contact a healthcare provider immediately if symptoms suggestive of infection are observed (eg, coughing, wheezing, sneezing, runny nose, sore throat, or fever)

## Hand hygiene

**Speak with your patient's caregiver about proper hand hygiene and proper waste disposal as parts of the vector in ELEVIDYS are eliminated via body waste**

- Advise caregivers and family members to use proper hand hygiene, such as hand washing, when coming into direct contact with the patient's body waste. Place potentially contaminated materials that may have the patient's bodily fluids/waste in a sealable bag, and dispose into regular trash. These precautions should be followed for 1 month after ELEVIDYS infusion

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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# Post-ELEVIDYS monitoring

## After ELEVIDYS infusion, monitor your patient for at least 3 months

The chart below can help you track appointments and assessments. Continue monitoring liver function until results are unremarkable and return to near baseline levels. Adjust the corticosteroid regimen when indicated. Refer to page 16 in this guide or the full Prescribing Information.

PATIENT NAME: _____ MEDICAL RECORD NUMBER: _____

DATE OF ELEVIDYS INFUSION: _____ ELEVIDYS LOT NUMBER: _____

### Insert the date of each appointment:

**Month 1**

- Monitor liver function (clinical exam, GGT,* and total bilirubin) weekly
- Monitor platelet count weekly for the first 2 weeks
- Monitor troponin-I weekly. More frequent monitoring may be required

| | WEEK 1: _____ | WEEK 2: _____ | WEEK 3: _____ | WEEK 4: _____ |
|---|---|---|---|---|
| LIVER FUNCTION | | | | |
| PLATELET COUNT | | | | |
| TROPONIN-I LEVELS | | | | |

**Month 2**

- Monitor liver function (clinical exam, GGT, and total bilirubin) weekly
- Continue monitoring platelet count and troponin-I weekly if clinically indicated

| | WEEK 1: _____ | WEEK 2: _____ | WEEK 3: _____ | WEEK 4: _____ |
|---|---|---|---|---|
| LIVER FUNCTION | | | | |
| PLATELET COUNT | | | | |
| TROPONIN-I LEVELS | | | | |

**Month 3**

- Monitor liver function (clinical exam, GGT, and total bilirubin) weekly
- Continue monitoring platelet count and troponin-I weekly if clinically indicated

| | WEEK 1: _____ | WEEK 2: _____ | WEEK 3: _____ | WEEK 4: _____ |
|---|---|---|---|---|
| LIVER FUNCTION | | | | |
| PLATELET COUNT | | | | |
| TROPONIN-I LEVELS | | | | |

* GGT=gamma-glutamyl transferase.

**For questions, contact Sarepta Medical Information (1-888-727-3782, option 2).**

**Please see additional Important Safety Information on pages 3-4 and full Prescribing Information.**

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