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 is an adeno-associated virus vector-based gene therapy indicated for the treatment of Duchenne muscular dystrophy (DMD) in both ambulatory and non-ambulatory patients who are at least 4 years of age with a confirmed mutation in the *DMD* gene.

Use in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin (or "micro-dystrophin") in skeletal muscle. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

ELEVIDYS is administered as a single intravenous infusion over 1 to 2 hours or longer, 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 Duchenne muscular dystrophy (DMD) in individuals at least 4 years of age:

- For patients who are ambulatory and have a confirmed mutation in the *DMD* gene
- For patients who are non-ambulatory and have a confirmed mutation in the *DMD* gene.

The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin (noted hereafter as "micro-dystrophin") in skeletal muscle. 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 Duchenne muscular dystrophy (DMD) in individuals at least 4 years of age:

- For patients who are ambulatory and have a confirmed mutation in the *DMD* gene
- For patients who are non-ambulatory and have a confirmed mutation in the *DMD* gene.

The DMD indication in non-ambulatory patients is approved under accelerated approval based on expression of ELEVIDYS micro-dystrophin (noted hereafter as "micro-dystrophin") in skeletal muscle. 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:

Infusion-related Reactions:

- Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred during or up to several hours following ELEVIDYS administration. Closely monitor patients during administration and for at least 3 hours after the end of infusion. If symptoms of infusion-related reactions occur, slow or stop the infusion and give appropriate treatment. Once symptoms resolve, the infusion may be restarted at a lower rate.
- ELEVIDYS should be administered in a setting where treatment for infusion-related reactions is immediately available.
- Discontinue infusion for anaphylaxis.

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 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.
- If a patient experiences myocarditis, those with pre-existing left ventricle ejection fraction (LVEF) impairment may be at higher risk of adverse outcomes. 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 patients 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 injury, 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 patients or caregivers. Below are key topics to discuss:

Infusion-related reactions	Infusion-related reactions including hypersensitivity and anaphylaxis have occurred during and after ELEVIDYS infusion. Possible symptoms of infusion-related reactions are fast heart rate, fast breathing, swollen lips, being short of breath, nostrils widening, hives, red and blotchy skin, itchy or inflamed lips, rash, vomiting, nausea, chills, and fever. Contact a healthcare provider immediately if the patient experiences such a reaction.
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 is available to assist.

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

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

Spanish-speaking Case Managers and interpreters for other languages are also available.

GGT=gamma-glutamyl transferase.

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

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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 1.33×10^{14} vector genomes per kilogram (vg/kg) of body weight (or 10 mL/kg body weight) for patients weighing less than 70 kg or 9.31×10^{15} vg total fixed dose for patients weighing 70 kg or greater. There is limited safety data available in non-ambulatory patients weighing 70 kg or greater who received the maximum dose of ELEVIDYS, 9.31×10^{15} vg, in clinical trials
- Calculate the dose as follows:

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

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

  Example: Calculation of volume needed for a 19.5 kg patient

  19.5 kg rounded to the nearest kilogram = 20 kg

  20 kg × 10 = 200 mL

  Number of ELEVIDYS vials needed = 200 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

- Measure baseline anti-AAVrh74 antibody titers using a Total Binding Antibody enzyme-lined immunosorbent assay (ELISA). 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*	Peri-ELEVIDYS infusion corticosteroid dose (prednisone equivalent)†	Recommended maximum total daily dose (prednisone equivalent)†
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

* Patient continues to receive this dose.

† Corticosteroids other than prednisone and prednisolone have not been studied 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
   - Thawed ELEVIDYS in vials or syringes is stable for up to 24 hours at room temperature (up to 25°C [77°F])

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

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 with a large surface area. To avoid the risk of occlusions, the use of smaller pediatric in-line filters (eg, less than 10 cm^2 surface area) is not recommended
- PVC (non-DEHP) IV infusion tubing, and polyurethane catheter

Administer ELEVIDYS as a single-dose intravenous infusion through a peripheral venous catheter.

- ELEVIDYS should be administered in a setting where treatment for infusion-related reactions is immediately available. Do not infuse ELEVIDYS at a rate of 10 mL/kg/hour or faster.
- 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 with 0.9% Sodium Chloride Injection 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 12 hours after drawing into syringe. Discard the ELEVIDYS-containing syringe(s) if infusion of the drug has not been completed within the 12-hour timeframe

4. In the event of an infusion-related reaction during administration
   - Slow or stop the infusion based on patient's clinical presentation
   - Discontinue infusion for anaphylaxis
   - Administer treatment as needed to manage infusion-related reaction
   - ELEVIDYS infusion may be restarted at a lower rate after the infusion-related reaction has resolved at the discretion of the physician, based on severity of patient's clinical presentation
   - If the ELEVIDYS infusion needs to be stopped and restarted, ELEVIDYS should be infused within 12 hours after drawing into the syringe

5. 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.

DEHP=di(2-ethylhexyl)phthalate; PES=polyether sulfone; PVC=polyvinyl chloride.

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

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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 injury, 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 injury). 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 156 male patients with a confirmed mutation of the *DMD* gene in 4 clinical studies, including 1 completed open-label study, and 1 ongoing, open-label study, and 2 studies that included a double-blind, placebo-controlled period. Prior to ELEVIDYS infusion, patients in the ELEVIDYS treatment group had a mean age of 6.7 years (range: 3 to 20) and mean weight of 24.6 kg (range: 12.5 to 80.1). One hundred forty-four patients received the recommended dose of 1.33×10^{14} vg/kg, and 12 received a lower dose. The table below presents adverse reactions from these 4 clinical studies.

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

Adverse reactions	ELEVIDYS (N=156) %
Vomiting	65
Nausea	43
Liver injury*	40
Pyrexia	28
Thrombocytopenia†‡	8

* Includes: AST increased, ALT increased, GGT increased, GLDH increased, GLDH level abnormal, hepatotoxicity, hepatic enzyme increased, hypertransaminasemia, liver function test increased, liver injury, transaminases increased, blood bilirubin increased.
† Includes: thrombocytopenia, platelet count decreased.
‡ Transient, mild, asymptomatic decrease in platelet counts.

Adverse reactions occurring in ELEVIDYS-treated patients at least twice more frequently than in placebo in Study 3 Part 1

Adverse reactions	ELEVIDYS (n=63) %	Placebo (n=62) %
Vomiting	64	19
Nausea	40	13
Liver injury*	41	8
Pyrexia	32	24
Thrombocytopenia†‡	3	0

* Includes: AST increased, ALT increased, GGT increased, GLDH increased, GLDH level abnormal, hepatotoxicity, hepatic enzyme increased, hypertransaminasemia, liver function test increased, liver injury, transaminases increased.
† Includes: platelet count decreased, thrombocytopenia.
‡ Transient, mild, asymptomatic decrease in platelet counts.

Postmarketing experience

The following adverse reactions have been identified during post-approval use of ELEVIDYS. Because these reactions are reported voluntarily from a population of uncertain size, it is not always possible to estimate their frequency or establish a causal relationship to drug exposure.

Immune System Disorders: Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred during or up to several hours following ELEVIDYS administration.

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).

ALT=alanine transaminase; AST=aspartate transferase; GGT=gamma-glutamyl transferase; GLDH=glutamate dehydrogenase.

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

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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.

Infusion-related Reactions

- Infusion-related reactions, including hypersensitivity reactions and anaphylaxis, have occurred during or up to several hours following ELEVIDYS administration
- Closely monitor patients during and for at least 3 hours after the end of infusion for signs and symptoms of infusion-related reactions including tachycardia, tachypnea, lip swelling, difficulty breathing, nasal flaring, urticaria, flushing, lip pruritus, rash, cheilitis, vomiting, nausea, rigors, and pyrexia

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
- If a patient experiences myocarditis, those with pre-existing left ventricle ejection fraction (LVEF) impairment may be at higher risk of adverse outcomes. Patients with moderate to severe LVEF impairment have not been studied in clinical trials with ELEVIDYS
- 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

ALT=alanine transaminase; GGT=gamma-glutamyl transferase.

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*

Peri-ELEVIDYS infusion corticosteroid dosing	Modified corticosteroid dose following ELEVIDYS infusion (prednisone equivalent)†	Recommended maximum total daily dose (prednisone equivalent)†
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

* 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.

† Corticosteroids other than prednisone and prednisolone have not been studied 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 patients or caregivers

Important risks

Speak with your patients or caregivers about the risk of infusion-related reactions, acute serious liver injury, immune-mediated myositis, and myocarditis

- Advise patients or caregivers to contact you immediately if:
  - The patient experiences fast heart rate, fast breathing, swollen lips, being short of breath, nostrils widening, hives, red and blotchy skin, itchy or inflamed lips, rash, vomiting, nausea, chills, or fever, as these may be signs of an infusion-related reaction
  - 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 patients or 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 patients or caregivers about weekly monitoring of liver function, troponin-I levels, and platelet counts postinfusion. Review the schedule for assessments on page 16

Concurrent infections

Speak with your patients or caregivers about infections due to the concomitant administration of corticosteroids before and after ELEVIDYS infusion

- Patients or 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 patients or 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 patients or caregivers about proper hand hygiene and proper waste disposal as parts of the vector in ELEVIDYS are eliminated via body waste

- Advise patients, 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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