EXONDYS 51
(eteplirsen) Injection

DOSING & ADMINISTRATION GUIDE

EXONDYS51.com

MEET DALTON.

DALTON IS A YOUNG MAN WITH DMD
(DELETION OF EXONS 48-50)

INDICATION[1]

EXONDYS 51 (eteplirsen) is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the *DMD* gene that is amenable to exon 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

IMPORTANT SAFETY INFORMATION

Hypersensitivity reactions, including bronchospasm, chest pain, cough, tachycardia and urticaria, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

The most common adverse reactions from observational clinical studies (N=163) seen in greater than 10% of patients were headache, cough, rash, and vomiting.

Please see the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

EXONDYS 51
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MEET BILLY.

BILLY IS A BOY WITH DMD
(DELETION OF EXON 52)

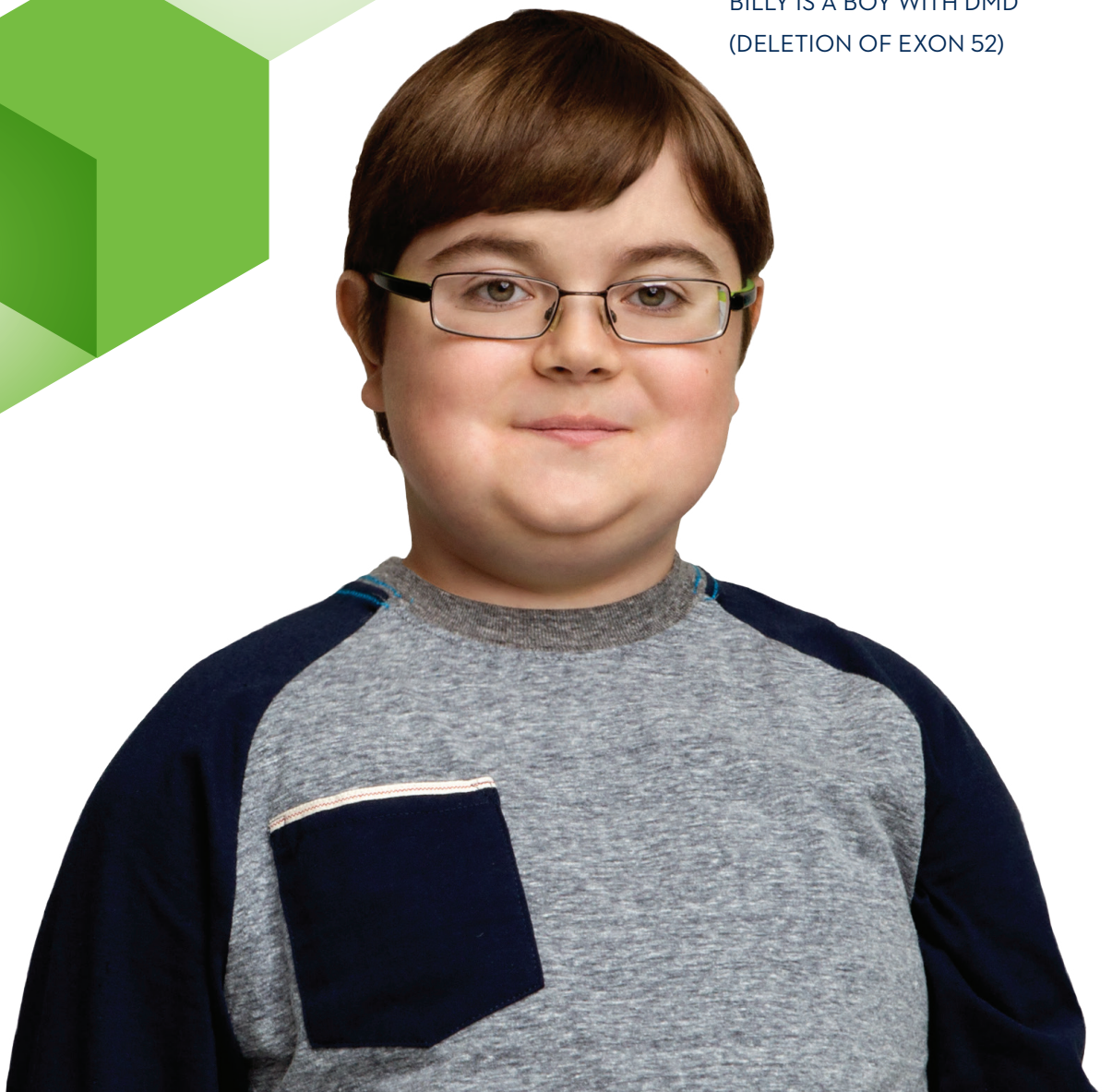


TABLE OF CONTENTS

INTRODUCTION[1]

The recommended dose of EXONDYS 51 is 30 mg/kg administered once weekly as a 35 to 60-minute intravenous infusion via an in-line 0.2 micron filter. If a dose of EXONDYS 51 is missed, it may be administered as soon as possible after the scheduled time.

Application of a topical anesthetic cream to the infusion site prior to administration of EXONDYS 51 may be considered.

If a hypersensitivity reaction occurs, consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Advise patients and/or caregivers that symptoms of hypersensitivity, including bronchospasm, chest pain, cough, tachycardia and urticaria, can occur with EXONDYS 51. Instruct them to seek immediate medical care should they experience signs and symptoms of hypersensitivity.

The 50 mg/kg once weekly dosing regimen is not recommended.

EXONDYS 51 injection is supplied in single-dose vials containing 100 mg/2 mL (50 mg/mL) eteplirsen or 500 mg/10 mL (50 mg/mL) eteplirsen. The solution is clear and colorless, may have some opalescence, and may contain trace amounts of small, white to off-white amorphous particles.

Store EXONDYS 51 at 2°C to 8°C (36°F to 46°F). Do not freeze. Protect from light and store EXONDYS 51 in the original carton until ready for use.

IMPORTANT SAFETY INFORMATION

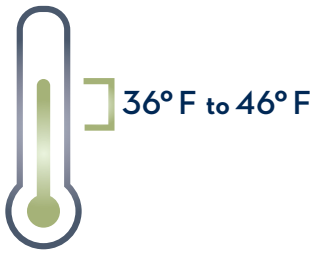
Hypersensitivity reactions, including bronchospasm, chest pain, cough, tachycardia and urticaria, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

BEFORE THE INFUSION[1]

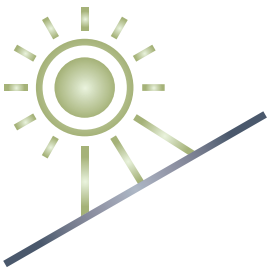
PROPER STORAGE AND HANDLING OF EXONDYS 51

Once you receive EXONDYS 51 at your facility, be sure to store it according to proper procedures:

Store EXONDYS 51 at 2° C to 8° C
(36° F to 46° F)



Protect from light and store in the original carton until ready for use



Do not freeze

Please see the Important Safety Information on page 3 and the accompanying full Prescribing Information for EXONDYS 51 (eteplirsen).

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EXONDYS 51 DOSING CALCULATIONS[1]

STEP 1 Calculate the patient dose in mg

Patient weight (kg) x 30 mg/kg = total dose in mg of EXONDYS 51

STEP 2 Calculate the volume in mL

Total mg of EXONDYS 51 needed ÷ **50** mg/mL = **Total volume in mL** of EXONDYS 51 needed

STEP 3 Calculate the number of single-use vials the patient needs

Total mL needed ÷ 10 = # of 10 mL vials

Add **# of 2 mL vials** up to needed dose

Depending on the patient's weight and the volume required, using a combination of 10 mL and 2 mL vials may help to avoid waste.

DOSING CALCULATION EXAMPLE BASED ON 33.5 KG PATIENT

STEP 1 Calculate the dose in mg:
33.5 kg x 30 mg/kg = 1,005 mg

STEP 2 Calculate the volume in mL:
1,005 mg ÷ 50 mg/mL = 20.1 mL

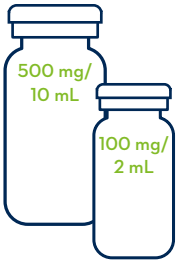
STEP 3 Calculate the number of single-use vials:
20.1 mL = two 10 mL vials AND **one 2 mL vial**

See page 14 of this brochure for a weight-based chart to help you determine how many 10 mL and 2 mL vials of EXONDYS 51 you will need.

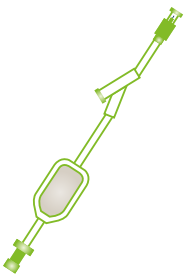
NECESSARY SUPPLIES[1,2]

To infuse EXONDYS 51, you will need:

EXONDYS 51
10 mL or 2 mL
single-dose vials



0.9% sodium chloride injection, USP,
(normal saline solution) infusion bag
(volume of 100-150 mL)



0.2 micron filter

A syringe fitted with a 21-gauge
or smaller non-coring needle

PREPARING EXONDYS 51[1-3]

PREPARE EXONDYS 51 ACCORDING TO THESE STEPS

STEP 1

Complete the dosing calculation.
See page 8 of this brochure for information on how to complete the dosing calculation.

STEP 2

EXONDYS 51 is administered intravenously via an in-line 0.2 micron filter. Flush the patient's intravenous access with sodium chloride 0.9% injection, USP, prior to and after infusion.

Application of a topical anesthetic cream to the infusion site prior to administration of EXONDYS 51 may be considered.

STEP 3

Assess the patient's IV or port for patency prior to removing any vials from the refrigerator. If IV access is adequate, remove the appropriate number of vials from the refrigerator and allow them to warm to room temperature. Do not microwave vials.

STEP 4

Once at room temperature, mix the contents of each vial by gently inverting 2 or 3 times. Do not shake. Visually inspect each vial of EXONDYS 51. EXONDYS 51 is a clear, colorless solution that may have some opalescence, and may contain trace amounts of small, white to off-white amorphous particles. Do not use if the solution in the vials is cloudy, discolored or contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles. If there is an issue with the solution, please report the issue to Sarepta at 1-888-SAREPTA (1-888-727-3782).

STEP 5

With a syringe fitted with a 21-gauge or smaller non-coring needle, withdraw the calculated volume of EXONDYS 51 from the appropriate number of vials.

STEP 6

Before adding the calculated volume of EXONDYS 51 to the saline bag, remove an equal volume of normal saline solution from the bag. Next, inject the bag with the syringe containing the calculated patient dose of EXONDYS 51. Gently invert infusion bag to ensure equal distribution of product. Contents can be mixed through 2-3 gentle inversions. Avoid agitation during preparation. Do not shake. Visually inspect the diluted solution. Do not use if the solution is cloudy, discolored or contains extraneous particulate matter other than trace amounts of small, white to off-white amorphous particles.

EXONDYS 51 contains no preservatives and should be administered immediately after dilution. Complete infusion within 4 hours of dilution. If immediate use is not possible, the diluted product may be stored for up to 24 hours at 2° C to 8° C (36° F to 46° F).

INFUSING EXONDYS 51[1,3,4]

ADMINISTER EXONDYS 51 ACCORDING TO THESE STEPS

STEP 7

- Prime the intravenous access line with normal saline solution.
- Infuse the diluted EXONDYS 51 solution over 35 to 60 minutes via an in-line 0.2 micron filter.
- If a hypersensitivity reaction occurs, consider slowing the infusion or interrupting the EXONDYS 51 therapy.
- Do not mix other medications with EXONDYS 51 or infuse other medications concomitantly via the same intravenous access line with EXONDYS 51.
- After completion of the infusion, flush the intravenous access line with sodium chloride 0.9% injection, USP, to allow the entire dose, including the contents of the intravenous access line, to be administered.
- In cases where EXONDYS 51 is administered into a venous access port, after administration of the drug and flushing with normal saline, the port may be flushed with heparin prior to de-access.

AFTER THE INFUSION[1]

STEP 8

Once you have completed the infusion, discard any unused product.

To report suspected adverse reactions, contact Sarepta Therapeutics, Inc. at 1-888-SAREPTA (1-888-727-3782) or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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NUMBER OF EXONDYS 51 10 ML AND 2 ML VIALS REQUIRED[1]

EXAMPLES BASED ON EXACT WEIGHT SHOWN

Not all patient weights are included in the chart below. This is not meant to replace calculating the exact dose and number of vials needed for each individual patient.

Exact Weight (kg)	Total Dose Required (mg) = # kg x 30 mg/kg	Calculated Volume (mL) Before Rounding = Total Dose (mg) ÷ 50 mg/mL	Total Volume Required (Rounded to Next Higher mL)	Number of 10 mL Vials	Number of 2 mL Vials
1	30	0.6	1		1
2	60	1.2	2		1
3	90	1.8	2		1
4	120	2.4	3		2
5	150	3.0	3		2
6	180	3.6	4		2
7	210	4.2	5		3
8	240	4.8	5		3
9	270	5.4	6		3
10	300	6.0	6		3
11	330	6.6	7		4
12	360	7.2	8		4
13	390	7.8	8		4
14	420	8.4	9	1	
15	450	9.0	9	1	
16	480	9.6	10	1	
17	510	10.2	11	1	1
18	540	10.8	11	1	1
19	570	11.4	12	1	1
20	600	12.0	12	1	1
21	630	12.6	13	1	2
22	660	13.2	14	1	2
23	690	13.8	14	1	2
24	720	14.4	15	1	3
25	750	15.0	15	1	3

Exact Weight (kg)	Total Dose Required (mg) = # kg x 30 mg/kg	Calculated Volume (mL) Before Rounding = Total Dose (mg) ÷ 50 mg/mL	Total Volume Required (Rounded to Next Higher mL)	Number of 10 mL Vials	Number of 2 mL Vials
26	780	15.6	16	1	3
27	810	16.2	17	1	4
28	840	16.8	17	1	4
29	870	17.4	18	1	4
30	900	18.0	18	1	4
31	930	18.6	19	2	
32	960	19.2	20	2	
33	990	19.8	20	2	
34	1020	20.4	21	2	1
35	1050	21.0	21	2	1
36	1080	21.6	22	2	1
37	1110	22.2	23	2	2
38	1140	22.8	23	2	2
39	1170	23.4	24	2	2
40	1200	24.0	24	2	2
41	1230	24.6	25	2	3
42	1260	25.2	26	2	3
43	1290	25.8	26	2	3
44	1320	26.4	27	2	4
45	1350	27.0	27	2	4
46	1380	27.6	28	2	4
47	1410	28.2	29	3	
48	1440	28.8	29	3	
49	1470	29.4	30	3	
50	1500	30.0	30	3	

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SAREPTASSIST

Support, By Your Patients' Side

SareptAssist is a patient support program designed to provide your patients with information to help them navigate the process of starting and staying on EXONDYS 51 (eteplirsen).

For more information or to enroll your patients in the program:

- Call us anytime at 1-888-SAREPTA (1-888-727-3782)
  Case Managers are available
  Monday through Friday, 8:30 am – 6:30 pm ET
- Email us at SareptAssist@Sarepta.com
- Visit SareptAssist.com

References: 1. EXONDYS 51 Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc. **2.** Data on File. **3.** Infusion Nurses Society. *Infusion Therapy Standards of Practice 2016*, Online edition. *Journal of Infusion Nursing*. Available at: http://ins.tizrapublisher.com/hai13r/. Accessed July 2020. **4.** Society of Infusion Nurses. Policies and Procedures for Infusion Therapy. Ch. 5, Infusion-related Complications: Identification & Intervention. Available at: http://ins.tizrapublisher.com/hha7v4/; 152-153. Accessed July 2020.

SAREPTA THERAPEUTICS

09/23 C-SRP-51-US-0360