BlueCross BlueShield Arizona

An Independent Licensee of the Blue Cross Blue Shield Association

PHARMACY COVERAGE GUIDELINE

AGAMREE® (vamorolone) oral
Deflazacort oral
DUVYZAT™ (givinostat) oral
EMFLAZA™ (deflazacort) oral
Generic Equivalent (if available)

This Pharmacy Coverage Guideline (PCG):
- Provides information about the reasons, basis, and information sources we use for coverage decisions
- Is not an opinion that a drug (collectively "Service") is clinically appropriate or inappropriate for a patient
- Is not a substitute for a provider's judgment (Provider and patient are responsible for all decisions about appropriateness of care)
- Is subject to all provisions e.g. (benefit coverage, limits, and exclusions) in the member's benefit plan; and
- Is subject to change as new information becomes available.

Scope
- This PCG applies to Commercial and Marketplace plans
- This PCG does not apply to the Federal Employee Program, Medicare Advantage, Medicaid or members of out-of-state Blue Cross and/or Blue Shield Plans

Instructions & Guidance
- To determine whether a member is eligible for the Service, read the entire PCG.
- This PCG is used for FDA approved indications including, but not limited to, a diagnosis and/or treatment with dosing, frequency, and duration.
- Use of a drug outside the FDA approved guidelines, refer to the appropriate Off-Label Use policy.
- The "Criteria" section outlines the factors and information we use to decide if the Service is medically necessary as defined in the Member's benefit plan.
- The "Description" section describes the Service.
- The "Definition" section defines certain words, terms or items within the policy and may include tables and charts.
- The "Resources" section lists the information and materials we considered in developing this PCG
- **We do not accept patient use of samples as evidence of an initial course of treatment, justification for continuation of therapy, or evidence of adequate trial and failure.**
- Information about medications that require prior authorization is available at www.azblue.com/pharmacy. You must fully complete the request form and provide chart notes, lab workup and any other supporting documentation. The prescribing provider must sign the form. Fax the form to BCBSAZ Pharmacy Management at (602) 864-3126 or email it to Pharmacyprecert@azblue.com.

Criteria:

AGAMREE (vamorolone)
Deflazacort
EMFLAZA (deflazacort)

- **Criteria for initial therapy:** Agamree (vamorolone), Emflaza (deflazacort), deflazacort, and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

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1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist

2. Individual is 2 years of age or older

3. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD) with documented mutation of the dystrophin gene or the presence of abnormal dystrophin

4. **Additional criteria for Agamree only**: Individual is ambulatory without the need of an assistive device (cane, walker, wheelchair, etc.)

5. Individual has documented failure (after 6 months of use), contraindication per FDA label, intolerance, or is not a candidate for **BOTH** of the following:
    a. Prednisone
    b. Prednisolone

6. **For brands Agamree (vamorolone) and Emflaza (deflazacort):** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for **generic deflazacort** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

7. **For Agamree (vamorolone):** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

8. Individual does not have severe hepatic impairment (Child-Pugh Class C)

9. Will not be simultaneously used with live or live attenuated vaccines

10. **For deflazacort (brand Emflaza and generic deflazacort) only:** Individual is not using moderate or strong CYP3A4 inducers such as efavirenz, modafinil, nafcillin, rifabutin, rifampin, carbamazepine, phenytoin, phenobarbital, and others

Initial approval duration: 12 months

- **Criteria for continuation of coverage (renewal request):** Agamree (vamorolone), Emflaza (deflazacort), deflazacort, and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy)**:

    1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist

    2. Individual's condition has responded while on therapy with response defined as **TWO** of the following:

a. Achieved and maintains an improvement in muscle strength over baseline
b. Achieved and maintains an improvement in muscle function over baseline as demonstrated by **THREE** of the following:
    i. Reduced falls
    ii. Able to stand
    iii. Able to balance
    iv. Improved time to walk or run 30 feet
    v. Improved time to climb 4 stairs
    vi. Improved time to stand from supine position
c. Achieved and maintains ability to independently perform activities of daily living
d. Achieved and maintains ambulation without need for wheelchair
e. Achieved and maintains an improved 6-minute walking distance
f. Improvement in forced vital capacity (FVC) or maximum voluntary ventilation (MVV)

3. Individual has been adherent with the medication

4. **For Emflaza (deflazacort):** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for **generic deflazacort** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

5. **For Agamree (vamorolone):** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

6. **For deflazacort (brand Emflaza and generic deflazacort) only:** Individual is not using moderate or strong CYP3A4 inducers such as efavirenz, modafinil, nafcillin, rifabutin, rifampin, carbamazepine, phenytoin, phenobarbital, and others

Renewal duration: 12 months

- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-Cancer Medications**

2. **Off-Label Use of Cancer Medications**

DUVYZAT (givinostat)

- **Criteria for initial therapy:** Duvyzat (givinostat) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met:

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1. Prescriber is a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist

2. Individual is 6 years of age or older

3. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD) with documented mutation of the dystrophin gene

4. Individual has DMD characteristic clinical symptoms or signs (e.g., proximal muscle weakness, Gowers' maneuver, elevated serum creatinine kinase level)

5. Individual is ambulatory without the need of an assistive device (cane, walker, wheelchair, etc.)

6. Individual has received and completed **ALL** the following **baseline tests** before initiation of treatment and with continued monitoring of the individual as clinically appropriate:
    a. Platelet count is greater than or equal to 150 x 10^9/L
    b. Triglyceride level is 300 mg/dL or less
    c. Electrocardiogram in individual with underlying cardiac disease or taking medication that causes QT prolongation
    d. Ambulation demonstrated by **TWO** of the following:
        i. Two 4-stair climb test of 8 seconds or less (with less than 1 second variance) [Note this only counts as **ONE** demonstration]
        ii. Time to rise from floor is at least 3 seconds but less than 10 seconds
        iii. Able to complete a 6-minute walk test of 350 to 400 meters

7. Individual has used a stable dose of a systemic corticosteroid for a minimum of 6 months and that the corticosteroid will be continued

8. Individual **is not on concurrent** genetic exon-skipping therapies **OR** vector-based gene therapy [e.g., Amondys 45 (casimersen), Exondys 51 (eteplirsen), Viltepso (viltolarsen), Vyondys 53 (golodirsen), delandistrogene moxeparvovec (Elevidys)]

9. Individual **has not received** any prior gene exon-skipping therapies and **is not being considered for treatmen**t with any other gene therapy

10. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

11. Individual does not have **ANY** of the following risk for ventricular arrhythmia:
    a. Torsades de pointe
    b. Congenital long QT syndrome or QTcF of greater than 450 msec
    c. Coronary artery disease
    d. Electrolyte disturbances

12. Individual is not using drugs that prolong the QT interval

13. Individual does not have hepatic impairment

Initial approval duration: 6 months

- **Criteria for continuation of coverage (renewal request):** Duvyzat (givinostat) and/or generic equivalent (if available) is considered ***medically necessary*** and will be approved when **ALL** the following criteria are met **(samples are not considered for continuation of therapy)**:

1. Individual continues to be seen by a physician specializing in the patient's diagnosis or is in consultation with a Pediatric Neurologist or Neurologist

2. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD)

3. Individual's condition has responded while on therapy with response defined as **TWO** of the following:
    a. Achieved and maintains an improvement in muscle strength over baseline
    b. Achieved and maintains an improvement in muscle function over baseline as demonstrated by **THREE** of the following:
        i. Reduced falls
        ii. Able to stand
        iii. Able to balance
        iv. Improved time to walk or run 30 feet
        v. Improved time to climb 4 stairs
        vi. Improved time to stand from supine position
    c. Achieved and maintains ability to independently perform activities of daily living
    d. Achieved and maintains ambulation without need for wheelchair
    e. Achieved and maintains an improved 6-minute walking distance
    f. Improvement in forced vital capacity (FVC) or maximum voluntary ventilation (MVV)

4. Individual has been adherent with the medication

5. Individual **is not on concurrent** genetic exon-skipping therapies **OR** vector-based gene therapy [e.g., Amondys 45 (casimersen), Exondys 51 (eteplirsen), Viltepso (viltolarsen), Vyondys 53 (golodirsen), delandistrogene moxeparvovec (Elevidys)]

6. Individual **has not received** any prior gene exon-skipping therapies and **is not being considered for treatmen**t with any other gene therapy

7. **If available:** Individual has failure after adequate trial, contraindication per FDA label, intolerance, or is not a candidate for a **generic equivalent** [Note: Failure, contraindication or intolerance to the generic should be reported to the FDA] (see Definitions section)

8. Individual continues to receive concomitant corticosteroid regimen

9. Individual has not developed any significant adverse drug effects that may exclude continued use such as:
    a. Thrombocytopenia despite dose modification
    b. Myelosuppression including anemia and neutropenia despite dose modification
    c. Elevated triglyceride of greater than 300 mg/dL despite diet intervention and dose adjustment
    d. Persistent moderate to severe diarrhea
    e. QTc interval greater than 500 ms or a change from baseline of greater than 60 ms
    f. Any adverse reaction that persists after two dose modification

10. Individual does not have **ANY** of the following risk for ventricular arrhythmia:
    a. Torsades de pointe
    b. Congenital long QT syndrome or QTcF of greater than 450 msec
    c. Coronary artery disease
    d. Electrolyte disturbances

11. Individual is not using drugs that prolong the QT interval

12. Individual does not have hepatic impairment

Renewal duration: 12 months

- Criteria for a request for non-FDA use or indication, treatment with dosing, frequency, or duration outside the FDA-approved dosing, frequency, and duration, refer to one of the following Pharmacy Coverage Guideline:

1. **Off-Label Use of Non-Cancer Medications**

2. **Off-Label Use of Cancer Medications**

Description:

Deflazacort (brand Emflaza and generic deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in individuals 2 years of age and older. Deflazacort (brand and generic) is a prodrug whose active metabolite (21-desDFZ) binds to glucocorticoid receptors to exert immunosuppressive and anti-inflammatory effects. Deflazacort (brand and generic) is a chemical modification of prednisolone. The precise mechanism by which deflazacort (brand and generic) exerts its therapeutic effects in DMD is unknown.

Agamree (vamorolone) is a corticosteroid indicated for the treatment of DMD in individuals 2 years of age and older. Vamorolone acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which vamorolone exerts its effect in patients with DMD is unknown.

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Duvyzat (givinostat), a histone deacetylase inhibitor, is indicated for the treatment of DMD in individuals 6 years of age and older. The precise mechanism by which givinostat exerts its effect in individuals with DMD is unknown. Givinostat promotes muscle formation and reduces fibrosis, fatty replacement, and inflammation in animal models of DMD and muscle histology of boys with DMD. It can be used in combination with glucocorticoid and its use is not restricted to the type of pathogenic gene variant.

DMD is a rare, genetic, X-linked, recessive neuromuscular disorder that typically afflicts young boys; however, female-manifesting carriers are reported. The disorder is caused by mutations of the dystrophin gene that leads to a disruption in messenger ribonucleic acid resulting in an absence or near absence of dystrophin within muscle cells. Dystrophin is thought to maintain the structural integrity of muscle cell, cushioning it from the stress and strain of repeated contraction and relaxation. Absence of dystrophin leads to muscle damage, with fibrotic and adipose tissue deposition.

In DMD there is significant deterioration of muscle strength and function with individuals experiencing frequent falls; difficulty in walking, standing, and balance; and difficulty in getting up from a lying or sitting position. A child is typically diagnosed with DMD between the ages of 2-5 years of age. There is progressive loss in the ability to perform activities independently, eventually leading to loss of ambulation (LoA) occurring by the teenage years in untreated patients. Other major complications of DMD that occur as the disease progresses include scoliosis, respiratory failure, and cardiomyopathy.

For individuals that are still ambulatory, the goal of treatment is to preserve ambulation and minimize future respiratory, cardiac, and orthopedic complications. For individuals that are not ambulatory, the goal of treatment is to maintain respiratory status, cardiac function, and to improve complications from scoliosis.

Glucocorticoids are the mainstay of pharmacologic treatment for DMD. Glucocorticoids medications slow the decline in muscle strength and function in DMD delaying the loss of ambulation and improve motor function; they also reduce the risk of scoliosis and stabilize pulmonary function and possibly for delay progression of cardiomyopathy and improve survival. Genetic therapies (casimersen [Amondys 45], eteplirsen [Exondys 51], golodirsen [Vyondys 53], and viltolarsen [Viltepso]) are available; these therapies increase dystrophin expression, but clinical benefit has not been established. Delandistrogene moxeparvovec (Elevidys) a nonreplicating, recombinant adeno-associated virus serotype rh74 vector-based gene therapy that delivers a normal copy of the gene encoding a micro-dystrophin protein is also available. Duvyzat (givinostat) appears to slow DMD disease progression.

Prednisone, prednisolone, deflazacort, and vamorolone are believed to work similarly. The choice of which glucocorticoid to use depends on availability, formulation, strengths available, cost, and perceived adverse effect profile. Limited evidence suggests that deflazacort might be preferred to prednisone or prednisolone for some individuals because of a lower risk of weight gain in the first years of treatment. With longer period of prednisone use the weight gain was no longer significantly different. Deflazacort possibly increases the risk of cataracts over prednisone, although they are not vision-impairing.

Prednisone and prednisolone, depending on agent chosen, are available in several different formulations such as tablets, delayed-release tablets, disintegrating tablets, and oral liquid forms. Prednisone strengths include 1 mg, 2 mg, 2.5 mg, 5 mg, 10 mg, 20 mg, and 50 mg. Prednisolone strengths include 5 mg, 10 mg, 15 mg, 20 mg 25 mg

and 30 mg. Deflazacort is available as oral tablet and oral suspension; strengths include 6 mg, 18 mg, 22.75 mg, 30 mg, and 35 mg. Vamorolone is available as a 40 mg/mL oral suspension.

Duvyzat (givinostat) is available as an 8.86 mg/mL oral suspension.

Definitions:

U.S. Food and Drug Administration (FDA) MedWatch Forms for FDA Safety Reporting
MedWatch Forms for FDA Safety Reporting | FDA

	Approximate Equivalent dose	Anti-inflammatory potency
Deflazacort	6.5 mg	N/A
Prednisone	5 mg	4 mg
Prednisolone	5 mg	4 mg
Vamorolone	N/A	N/A
N/A*: not available*		

Timed 4-stair climb (4SC) test:

- A widely used tool to assess motor function in Duchenne muscular dystrophy (DMD), measuring how quickly someone can climb four stairs
- The 4SC test is used to measure functional capacity and assess the progression of muscle weakness
- The test involves timing how long it takes a person to climb four stairs, it can be used to monitor changes in motor function over time
- Importance in DMD:
    - DMD is characterized by progressive muscle weakness, making the 4SC test a valuable tool for monitoring disease progression and evaluating the effectiveness of treatments

North Star Ambulatory Assessment (NSAA):

- Evaluates the ability to perform various functional skills, such as standing up, climbing stairs, or getting up from a chair
- A 17-item scale is used to assess gross motor abilities in ambulant boys, with a total score ranging from 0 to 34, where higher scores indicate better function
    - Each item is scored on a scale of 0 (unable), 1 (completes independently but with modifications), and 2 (completed without compensation)
- NSAA is clinician-reported outcome measure specifically designed to assess ambulatory function in DMD

North Star Ambulatory Assessment (NSAA)			
Activity	Score: 2 Completed without compensation	Score: 1 Completes independently but with modifications	Score: 0 Unable
1. Stand	Stands upright, still and symmetrically, without compensation (with heels flat and legs in neutral)	Stands still with some degree of compensation (e.g., on toes or with legs abducted or with bottom	Cannot stand still or independently, need support (even minimal)

	for minimum count of 3 seconds	stuck out) for minimum count of 3 seconds	
2. Walk	Walks with heel-toe or flat-footed gait pattern	Persistent or habitual toe walker, unable to heel-toe consistently	Loss of independent ambulation – may use KAFOs or walk short distances with assistance
3. Stand up from chair	Keeping arms folded, starting position 90^{o} hips and knees, feet on floor/supported on a box step	With help from thighs or push on chai or prone turn	Unable
4. Stand on one leg - right	Able to stand in a relaxed manner (no fixation) for count of 3 seconds	Stands but either momentarily or needs a lot of fixation e.g., by knees tightly adducted or other trick	Unable
5. Stand on one leg - left	Able to stand in a relaxed manner (no fixation) for count of 3 seconds	Stands but either momentarily or needs a lot of fixation e.g., by knees tightly adducted or other trick	Unable
6. Climb box step - right	Faces step – no support needed	Goes up sideways or needs support	Unable
7. Climb box step - right	Faces step – no support needed	Goes up sideways or needs support	Unable
8. Descend box step - Right	Faces forward, climbs down controlling weight bearing leg. No support needed	Sideways, skips down or needs support	Unable
9. Descend box step - Right	Faces forward, climbs down controlling weight bearing leg. No support needed	Sideways, skips down or needs support	Unable
10. Gets to sitting	Starts in supine – may use one hand to assist	Self-assistance e.g. – pulls on legs or uses head-on hands or head flexed to floor	Unable
11. Rise from floor	From supine – no evidence of Gowers' maneuver*	Gowers' evident. Turns towards the floor (generally into a four-point kneeling position) to place hands on the floor to assist rising, walks	a) NEEDS to use external support object e.g. chair OR (b) Unable

		hands back in towards him then uses arms to 'climb' up legs to achieve upright standing. A wide base of support is often assumed through the phases of rising from the floor	
12. Lifts head	In supine, head must be lifted in mid-line. Chin moves towards chest	Head is lifted but through side flexion or with no neck flexion	Unable
13. Stands on heels	Both feet at the same time, clearly standing on heels only (acceptable to move a few steps to keep balance) for count of 3	Flexes hip and only raises forefoot	Unable
14. Jump	Both feet at the same time, clear the ground simultaneously	One foot after the other (skip)	Unable
15. Hop right leg	Clears forefoot and heel off floor	Able bend knee and raise heel, no floor clearance	Unable
16. Hop left leg	Clears forefoot and heel off floor	Able bend knee and raise heel, no floor clearance	Unable
17. Run (10m)	Both feet off the ground (no double stance phase during running)	'Duchenne jog' not a true run (there probably IS a double support phase), but more than a walk. Typically characterized by excessive use of arms, trunk rotation, substantial 'waddle'. No real 'push off'	Walk
			TOTAL = /34
2 – 'Normal' – no obvious modification of activity 1 - Modified method but achieves goal independent of physical assistance from another 0 - Unable to achieve independently			

Time to rise from floor (TRF):

- The sitting-rising test involves a person sitting on the floor (cross-legged or in a comfortable position) and then rising to a standing position without using their hands or knees for support
- How to perform the test:
  - Start from a standing position
  - Sit all the way to the floor without using your hands or knees for support

    - Rise back up to standing without using your hands or knees for support
- Scoring:
    - Individual is timed, and success is considered as sitting and rising without any support
    - Points are deducted for using hands, knees, or other objects for support
    - People in their twenties can stand up from the floor in just a few seconds, for individuals over 50, is generally considered to be less than 3 seconds for fit individuals, less than 4 seconds for those in their sixties, and 5 and 6 seconds for those in their seventies and eighties
- Why it matters:
    - Functional Mobility: The test assesses a person's ability to transition from a seated to a standing position, which is a crucial movement for daily activities and independence
    - Longevity: Studies have shown a correlation between the ability to perform this test and overall survival, suggesting that those who struggle with it may have a higher risk of mortality
    - Musculoskeletal Fitness: The test can reveal information about a person's leg and core strength, balance, and flexibility
- In Duchenne muscular dystrophy (DMD), the time it takes to rise from the floor TRF can be a predictor of disease progression and loss of ambulation, with longer times indicating a faster decline in functional abilities
- Studies have shown that the TRF is a useful measure for assessing disease progression in DMD
- Correlation with 6-Minute Walk Test (6MWT):
    - TRF correlates with 6MWT performance, both can be used to predict changes in 6MWT over time
- TRF and Loss of Ambulation:
    - Boys with DMD who take longer to rise from the floor (e.g., more than 7 seconds) experience more decline in their 6MWT performance and may lose their ability to walk sooner
- TRF Ranges:
    - In one study, the time to rise from the floor ranged from 1.2 to 29.4 seconds
- The age at which a person with DMD loses ambulation (LOA) is linked to the TRF, with those taking longer to rise from the floor experiencing LOA at younger ages

6-minute walk test (6MWD):

- The primary outcome measure is the distance walked in meters during the 6 minutes
- For healthy adults, the normal range for the 6MWD is typically between 400 and 700 meters, but can vary based on age, gender, and other factors
- A change of at least 30 meters in 6MWD is considered a clinically meaningful
- Several factors can influence the 6MWD, including age, gender, height, and underlying health conditions
- Specific Conditions:
    - COPD: A 6MWD of 350 meters or less is associated with increased risk of exacerbation, hospitalization, and mortality
    - Heart Failure: A 6MWD of less than 300 meters is considered indicative of severe exercise intolerance
    - Pulmonary Arterial Hypertension: Patients capable of walking at least 400 m within the 6-minute timeframe generally exhibit a more favorable prognosis
    - Interstitial Lung Disease: Desaturation during a 6MWD is an important prognostic indicator for patients with ILD

- Duchenne Muscular Dystrophy (DMD): 6MWD distance of around 300-350 meters is often considered a lower limit of function, potentially indicating a risk for loss of ambulation

Resources:

Emflaza (deflazacort) tablet & oral suspension product information, revised by PTC Therapeutics, Inc. 06-2021. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 24, 2024.

Deflazacort oral suspension product information, revised by Tris Pharma Inc. 03-2024. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 24, 2024.

Agamree (vamorolone) oral suspension product information, revised by Catalyst Pharmaceuticals, Inc. 10-2023. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 24, 2024.

Duvyzat (givinostat) oral suspension product information, revised by Italfarmaco SPA. 03-2024. Available at DailyMed http://dailymed.nlm.nih.gov. Accessed June 24, 2024.

Darras BT. Duchenne and Becker muscular dystrophy: Glucocorticoid and disease-modifying treatment. In: UpToDate, Patterson MC, Dashe JF (Eds), UpToDate, Waltham MA.: UpToDate Inc. Available at http://uptodate.com. Literature current through May 2024. Topic last updated April 23, 2024. Accessed June 24, 2024.

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Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Report of the Guideline Development Subcommittee of the American Academy of Neurology. Re-affirmed January 22, 2022. Update: Corticosteroid Treatment of Duchenne Muscular Dystrophy (aan.com). Neurology 2016;86(5):465-472. Accessed October 30, 2020. Re-evaluated June 25, 2024.

ClinicalTrials.gov Bethesda (MD): National Library of Medicine (US). Identifier NCT02851797: PROTOCOL: Randomized, Double Blind, Placebo Controlled, Multicenter Study to Evaluate the Efficacy and Safety of Givinostat in Ambulant Patients With Duchenne Muscular Dystrophy. Available from: http://clinicaltrials.gov. Last update posted February 02, 2023. Last verified January 2023. Accessed June 24, 2024.

Mercuri E, Vilchez JJ, Boespflug-Tanguy O, et al.: Safety and efficacy of givinostat in boys with Duchenne muscular dystrophy (EPIDYS): a multicenter, randomized, double-blind, placebo-controlled, phase 3 trial. Lancet Neurology 2024; 23: 393-403. Accessed June 26, 2024.