



Naythan, an actual DUVYZAT patient for 4+ years.

DUVYZAT makes it possible to offer your patients

PROTECTION

AGAINST DMD PROGRESSION^{1,*}

DMD, Duchenne muscular dystrophy.

*As measured by change from baseline to month 18 vs placebo in 4-stair climb (4SC).

INDICATION

DUVYZAT is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 6 years of age and older.

IMPORTANT SAFETY INFORMATION

Warnings and Precautions

Hematological Changes: DUVYZAT can cause dose-related thrombocytopenia and other signs of myelosuppression. Monitor blood count every 2 weeks for the first 2 months, at month 3, and every 3 months thereafter. Modify the dosage for confirmed thrombocytopenia. Discontinuation may be needed if abnormalities worsen.

Please see full Important Safety Information on page 16 and full Prescribing Information for additional safety information.

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Ryan, an actual DUVYZAT patient for 4+ years.

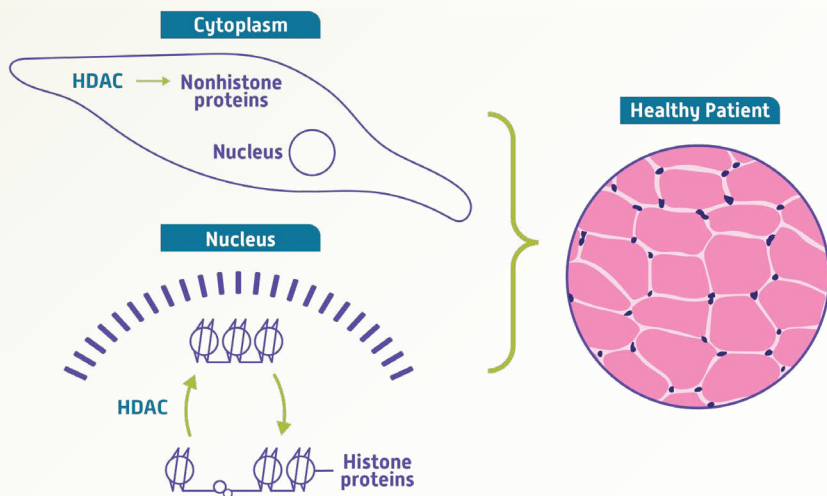
IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

Increased Triglycerides: DUVYZAT can cause elevations in triglycerides. Monitor triglycerides at 1 month, 3 months, 6 months, and then every 6 months thereafter. Modify the dosage if fasting triglycerides are verified >300 mg/dL. Treatment with DUVYZAT should be discontinued if triglycerides remain elevated despite adequate dietary intervention and dosage adjustment.

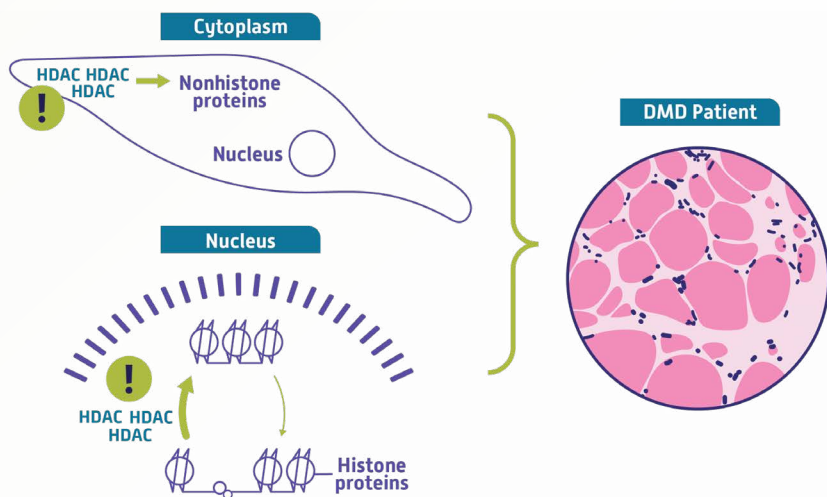
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HDAC may play a key role in regulating muscle homeostasis¹⁻⁶



The potential role of HDAC in normal muscle repair²⁻⁴

HDACs are thought to be important for regulating muscle homeostasis. Through nuclear and cytoplasmic enzymatic activity, HDACs modify both histone and nonhistone proteins. They may play a key role in maintaining and repairing muscle tissue by modifying proteins that regulate muscle fiber repair pathways.



HDAC overactivity in DMD^{3,5,6}

In DMD, HDAC is overactive. Increased HDAC activity, in combination with the structural instability of dystrophin-deficient muscle cells, may disrupt the process of normal muscle repair and accelerate the deterioration of tissue.

HDAC upregulation may drive^{3,5,6}:

- Activation of chronic inflammatory pathways
- Impairment of muscle repair
- Fibrogenesis and adipogenesis
- Muscular atrophy

HDAC, histone deacetylase.

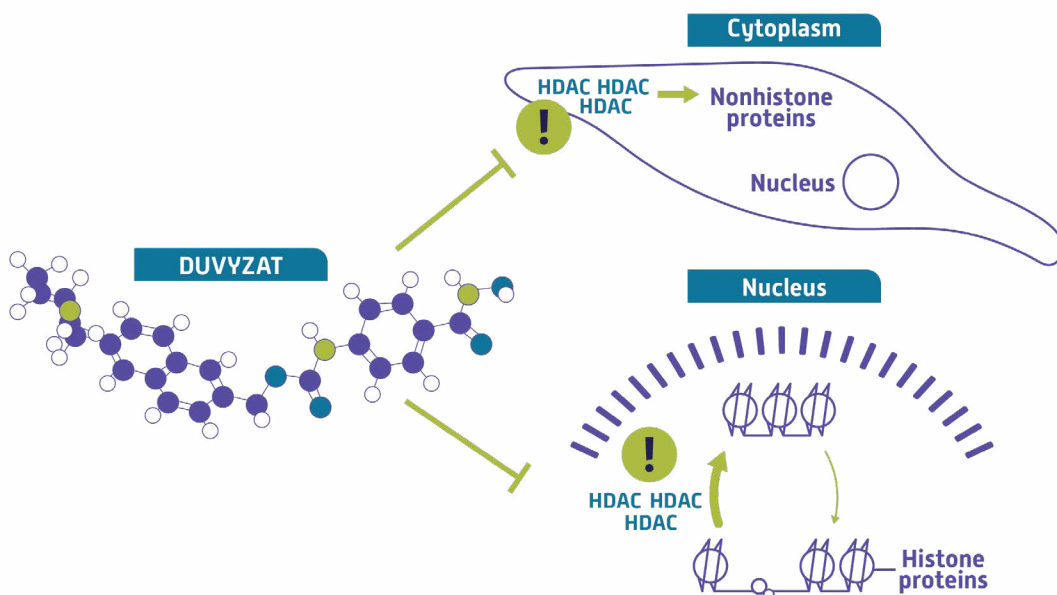
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While the exact mechanism is unknown,
**DUVYZAT targets HDAC overactivity—
thought to be a key pathologic process
in DMD¹⁻⁶**



How DUVYZAT—a pan-HDAC inhibitor—may work^{1,6}

While its exact mechanism of action is unknown, DUVYZAT is designed to target HDAC, reduce its enzymatic activity, and address the key pathologic process of HDAC overactivity.



**As the only HDAC inhibitor indicated to treat DMD, DUVYZAT offers a
unique, mutation-agnostic approach to treatment.^{1,3,5}**

IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

Gastrointestinal Disturbances: Gastrointestinal disturbances, including diarrhea, nausea/vomiting, and abdominal pain were common adverse reactions in DUVYZAT clinical trials. Antiemetics or antidiarrheal medications may be considered during treatment with DUVYZAT. Modify the dosage of DUVYZAT in patients with moderate or severe diarrhea and discontinue treatment if significant symptoms persist.

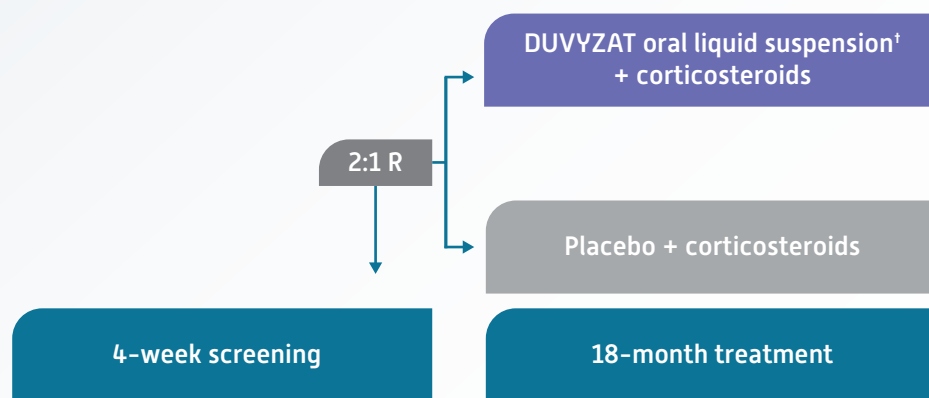
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DUVYZAT was studied in EPIDYS, one of the most inclusive phase 3 DMD studies to date^{5,*}
EPIDYS was a global, 18-month, double-blind, randomized, placebo-controlled trial^{1,5}



179 patients were randomized 2:1 to receive oral DUVYZAT + corticosteroids (n=118) or matching placebo + corticosteroids (n=61).^{1,2}

120 of these 179 patients in the study were included in the efficacy analysis (Group A).



R, randomization.

[†]Dose adjustments permitted dependent on protocol version at randomization.⁵

The EPIDYS clinical trial also includes an open-label extension phase that is ongoing.⁵

The 2:1 randomization ratio was chosen because of the rapidly progressive nature of DMD as well as the rarity of the disease. This ratio maximized the number of patients exposed to active treatment while maintaining study integrity.^{1,5}

Primary Endpoint¹

Change from baseline to month 18 in time to complete 4SC for DUVYZAT + corticosteroids compared to placebo + corticosteroids

NSAA, North Star Ambulatory Assessment; VLFF, vastus lateralis fat fraction.

*EPIDYS is an acronym for Epigenetic Rescue of Dystrophin Dysfunction trial.⁵

Key Secondary Endpoints⁵

Change from baseline to month 18 in:


- Motor function and muscle strength assessed by the NSAA
- VLFF
- 6-minute walking test
- Knee extension and elbow flexion

IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

QTc Prolongation: DUVYZAT can cause prolongation of the QTc interval. Avoid use of DUVYZAT in patients who are at an increased risk for ventricular arrhythmias (including torsades de pointes), such as those with congenital long QT syndrome, coronary artery disease, electrolyte disturbance or in patients taking concomitant medicinal products known to cause QT prolongation. Obtain ECGs prior to initiating treatment with DUVYZAT in patients with underlying cardiac disease or in patients who are taking concomitant medications that cause QT prolongation.

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 **DUVYZAT was studied in EPIDYS, one of the most inclusive phase 3 DMD studies to date^{5,*}**



Patient population

- Ambulant boys aged ≥ 6 years with genetically confirmed DMD (median age of 9.8 years)^{1,5}
- No restrictions on genetic mutations, but these were generally balanced across both groups⁵
- All patients completed two 4SC assessments with a mean of 8 seconds or less, were ambulatory, and had a time-to-rise of at least 3 seconds but less than 10 seconds⁵
- Patients were on a stable dose and regimen of corticosteroids, which were continued throughout the study⁵

Recruitment was prespecified in 2 groups:

Group A Efficacy and safety analysis group, n=120

Baseline VLFF $>5\%$ but $\leq 30\%$ ⁵

Composed of patients unlikely to lose mobility suddenly but expected to show measurable decline in function, strength, and fat fraction when on placebo + corticosteroids.

Group B Safety analysis only, n=59

Baseline VLFF $\leq 5\%$ or $>30\%$ ⁵

Recruited to assess the safety of DUVYZAT + corticosteroids in a broader population of patients with DMD.

Participants attended study site visits every 12 weeks for 18 months.

Magnetic resonance spectroscopy (MRS) of the right upper leg was done at screening and after 48 and 72 weeks for the calculation of VLFF.⁵

*EPIDYS is an acronym for Epigenetic Rescue of Dystrophin Dysfunction trial.⁵

IMPORTANT SAFETY INFORMATION (cont'd)

Adverse Reactions

The most common adverse reactions reported in $>5\%$ of patients treated with DUVYZAT are diarrhea [37%], abdominal pain [34%], thrombocytopenia [33%], nausea/vomiting [32%], hypertriglyceridemia [23%], pyrexia [13%], myalgia [9%], rash [9%], arthralgia [8%], fatigue [8%], constipation [7%], and decreased appetite [7%].

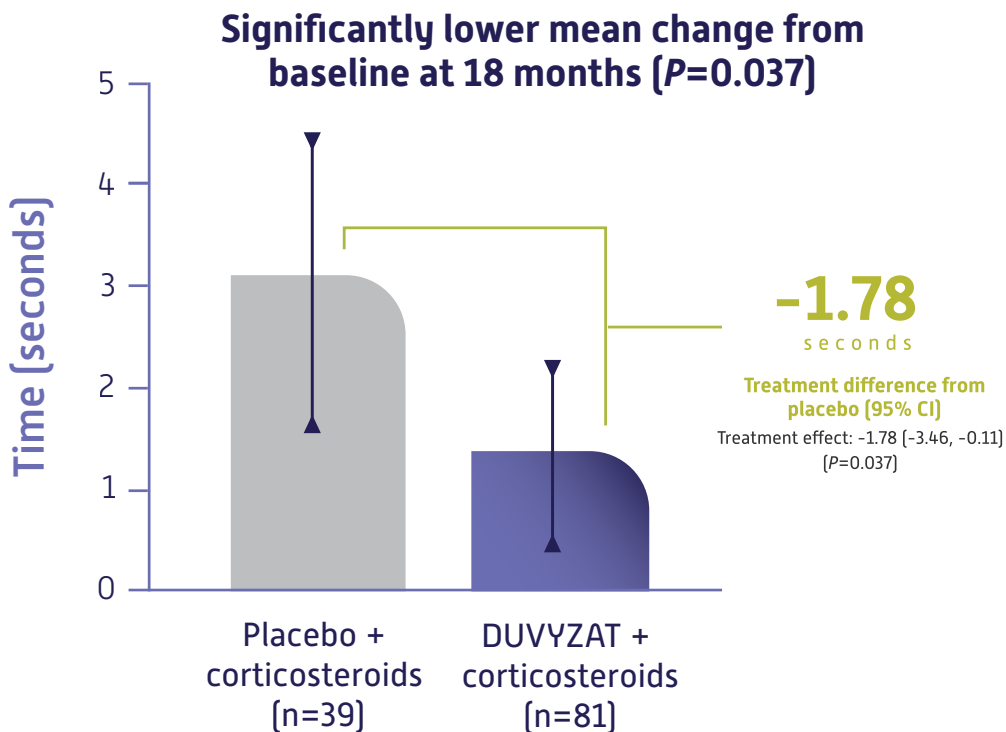
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PROTECTION against DMD progression with DUVYZAT¹



Patients treated with DUVYZAT were able to complete 4SC faster than patients taking placebo¹

PRIMARY ENDPOINT: Change in time to perform 4SC vs placebo



Estimates compared across multiple real-world and clinical trial data sources indicate that change ≥ 1.3 seconds to complete the 4SC is clinically meaningful.⁷

IMPORTANT SAFETY INFORMATION (cont'd)

Drug Interactions

Closely monitor when DUVYZAT is used in combination with an oral CYP3A4 sensitive substrate or a sensitive substrate of the OCT2 transporter, for which a small change in substrate plasma concentrations may lead to serious toxicities.

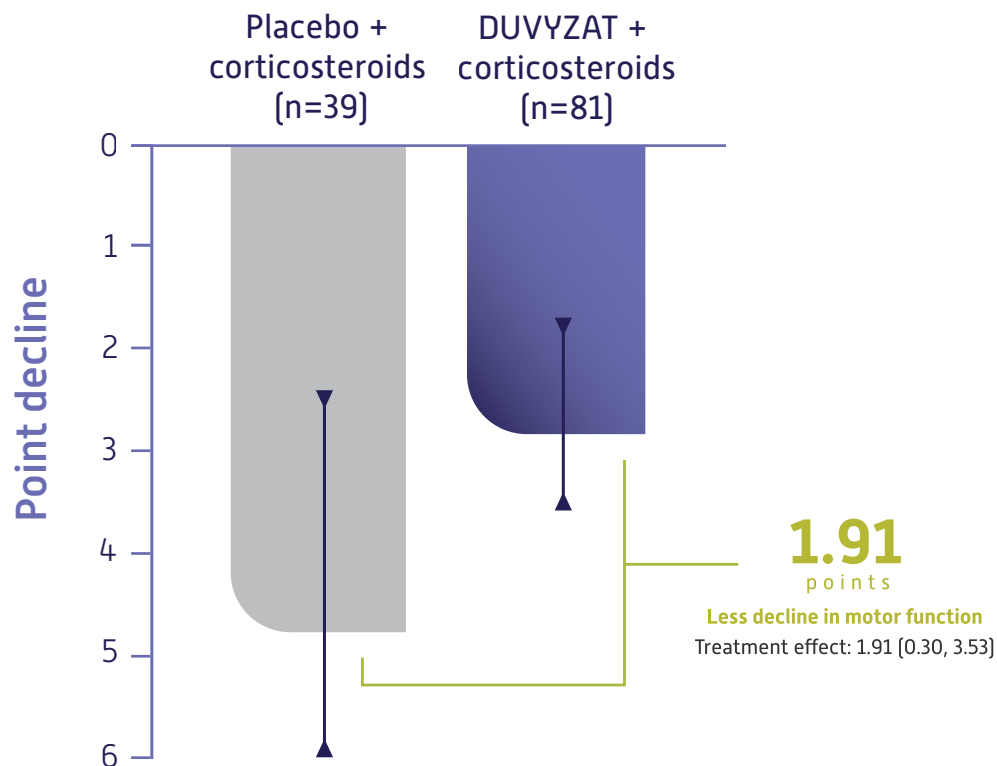
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PROTECTION against DMD progression with DUVYZAT¹



DUVYZAT demonstrated greater preservation of motor function vs placebo as shown by NSAA^{5,*}

SECONDARY ENDPOINT: Change from baseline in total NSAA item score over 18 months^{5,†}



[Review post hoc test analysis](#)

*Nominally significant but not statistically significant based on the prespecified multiplicity adjustment.⁵

[†]The NSAA is a DMD-specific assessment scale measuring lower limb function in ambulant children with DMD, comprising 17 items scored on a scale of 0 to 2. A score of 2 indicates the activity is performed without difficulty, 1 indicates the activity is performed with some compensation, and 0 indicates the activity cannot be performed independently.^{1,5,8}

IMPORTANT SAFETY INFORMATION (cont'd)

Drug Interactions (cont'd)

Avoid concomitant use with other drugs that prolong the QTc interval; monitor ECG if concomitant use cannot be avoided. If concomitant use cannot be avoided, obtain ECGs when initiating, during concomitant use, and as clinically indicated. Withhold DUVYZAT if the QTc interval is >500 ms or the change from baseline is >60 ms.

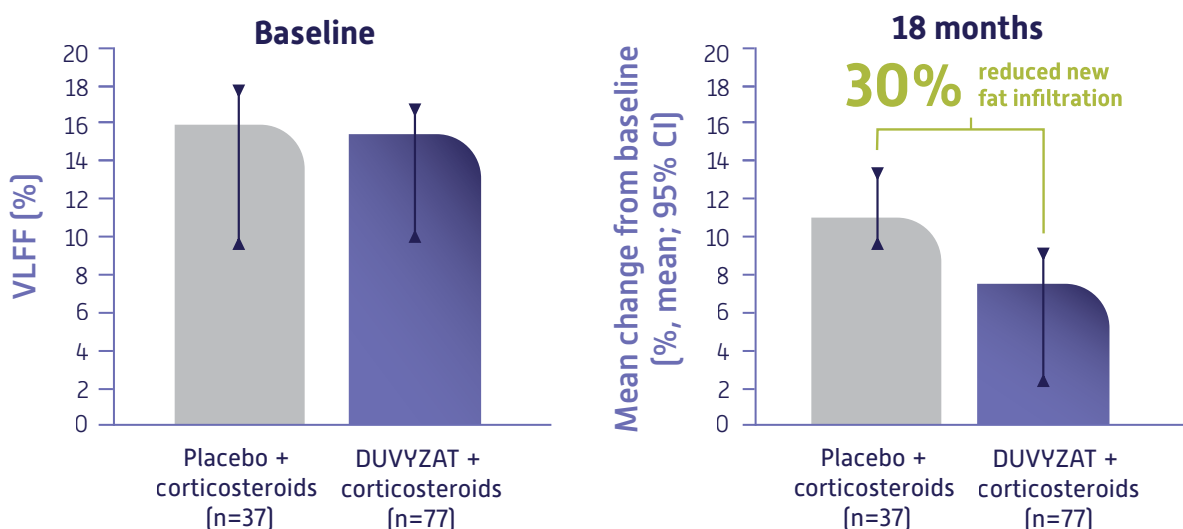
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Compared with placebo at 18 months,
DUVYZAT reduced new fat infiltration in key muscle groups required for ambulation^{1,5,9,*}



Patients treated with DUVYZAT had lower increase in mean VLFF over 18 months vs patients taking placebo

SECONDARY ENDPOINT: Changes in mean VLFF over 18 months from baseline^{5,9}



At 18 months, for the patients with VLFF baseline in the range of >5% to ≤30%, a mean increase (absolute difference from baseline levels) of VLFF was 7.48% in the DUVYZAT-treated patients compared to a 10.89% increase in patients who received placebo.¹

*Nominally significant but not statistically significant based on the prespecified multiplicity adjustment.⁵

IMPORTANT SAFETY INFORMATION

Warnings and Precautions

Hematological Changes: DUVYZAT can cause dose-related thrombocytopenia and other signs of myelosuppression. Monitor blood count every 2 weeks for the first 2 months, at month 3, and every 3 months thereafter. Modify the dosage for confirmed thrombocytopenia. Discontinuation may be needed if abnormalities worsen.

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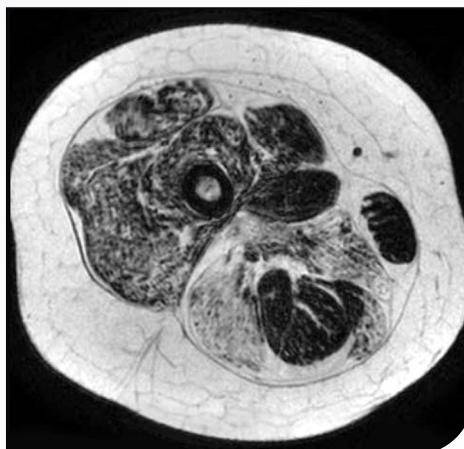
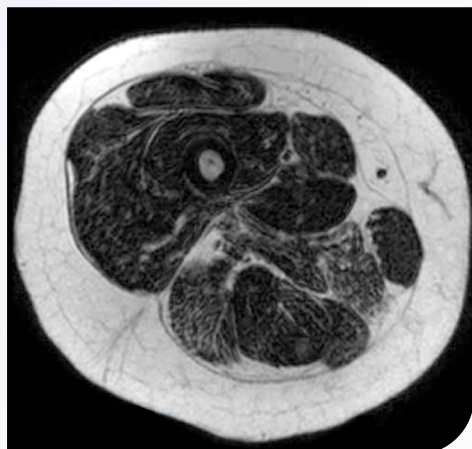
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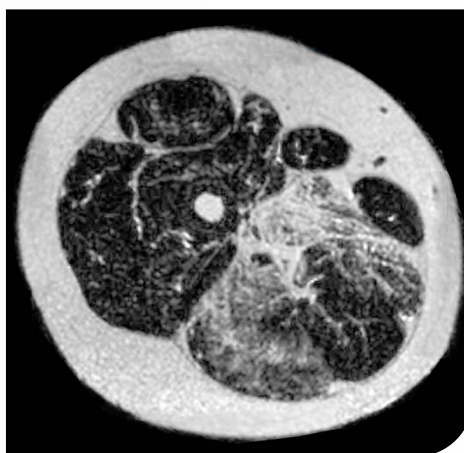
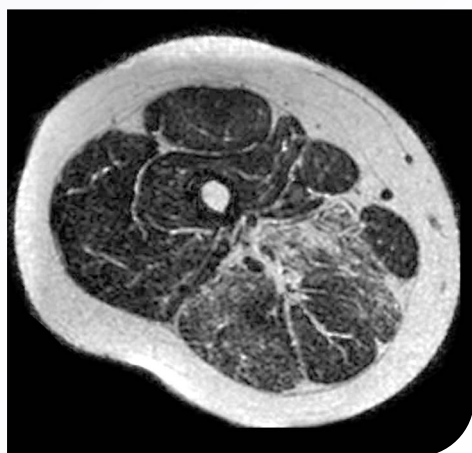
EXPLORATORY ANALYSIS: DUVYZAT reduced new fat infiltration in the thigh muscle^{1,5,9}

Baseline

18 months



Patient taking placebo + corticosteroids



Patient taking DUVYZAT + corticosteroids

MRS imaging showing VLFF increase from baseline after 18 months.⁵

Images are from single individuals and may not be representative of all patients' results.

IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

Increased Triglycerides: DUVYZAT can cause elevations in triglycerides. Monitor triglycerides at 1 month, 3 months, 6 months, and then every 6 months thereafter. Modify the dosage if fasting triglycerides are verified >300 mg/dL. Treatment with DUVYZAT should be discontinued if triglycerides remain elevated despite adequate dietary intervention and dosage adjustment.

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DUVYZAT safety¹

DUVYZAT safety is based on the pivotal EPIDYS 18-month study in a total of 179 ambulant DMD patients aged 6 years or older on concomitant corticosteroid treatment



Adverse reactions reported in >5% of DUVYZAT-treated patients

	DUVYZAT + corticosteroids (n=118) %	Placebo + corticosteroids (n=61) %
Diarrhea	37	20
Abdominal pain	34	25
Thrombocytopenia*	33	0
Nausea/vomiting	32	18
Hypertriglyceridemia	23	7
Pyrexia	13	8
Myalgia	9	3
Rash	9	2
Arthralgia	8	2
Fatigue	8	0
Constipation	7	2
Decreased appetite	7	0

 **Diarrhea, the most common adverse reaction, usually occurred within the first few weeks of treatment and resolved with continued dosing.^{1,10}**

*Thrombocytopenia includes platelet count decreased and thrombocytopenia.

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PROTECTION with convenient dosing

DUVYZAT is an oral suspension administered twice daily [can be taken with food]¹



Weight-based dosing with DUVYZAT¹

Body weight	Dosage	Oral suspension volume
10 kg to <20 kg	22.2 mg twice daily	2.5 mL twice daily
20 kg to <40 kg	31 mg twice daily	3.5 mL twice daily
40 kg to <60 kg	44.3 mg twice daily	5 mL twice daily
≥60 kg	53.2 mg twice daily	6 mL twice daily

IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

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Please see [full Important Safety Information](#) on page 16 and [full Prescribing Information](#) for additional safety information.

Initial testing and ongoing monitoring¹



Obtain and evaluate baseline platelet counts and triglycerides prior to initiation of DUVYZAT.

- Do not initiate DUVYZAT in patients with a platelet count of $<150 \times 10^9/L$



In patients with underlying cardiac disease or taking concomitant medications that cause QT prolongation, obtain ECGs:

- When initiating treatment with DUVYZAT
- During concomitant use if unavoidable
- When clinically indicated



Ideally, avoid concomitant use with other drugs that prolong the QTc interval.



Closely monitor when DUVYZAT is used in combination with an oral CYP3A4 sensitive substrate or a sensitive substrate of the OCT2 transporter, for which a small change in substrate plasma concentration may lead to serious toxicities.

After prescribing DUVYZAT, ongoing monitoring with 2 blood tests helps to keep patients on track

Month	1	2	3	4	5	6	7	8	9	10	11	12
	Every 2 weeks											
CBC + differential	●	●	●			●			●			●
Triglycerides	●		●			●						●



CBC with differential

Monitor blood counts every 2 weeks for the first 2 months of treatment, at month 3, and then every 3 months thereafter



Triglycerides

Monitor at 1 month, 3 months, 6 months, and then every 6 months thereafter

After the first 3 months of treatment with DUVYZAT, your patient's blood monitoring will become less frequent

CBC, complete blood count; ECG, electrocardiogram; QTc, heart-rate corrected QT interval.

IMPORTANT SAFETY INFORMATION (cont'd)

Warnings and Precautions (cont'd)

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Dosage modifications¹



Dose can be modified for adverse reaction mitigation

Modifications may be needed for decreased platelet counts, diarrhea, and increased triglycerides, or QTc prolongation. Please see [full Prescribing Information](#) for more details about dosage modifications to mitigate adverse reactions.

Body weight	First dosage modification*		Second dosage modification†	
	Dosage	Oral suspension volume	Dosage	Oral suspension volume
10 kg to <20 kg	17.7 mg twice daily	2 mL twice daily	13.3 mg twice daily	1.5 mL twice daily
20 kg to <40 kg	22.2 mg twice daily	2.5 mL twice daily	17.7 mg twice daily	2 mL twice daily
40 kg to <60 kg	31 mg twice daily	3.5 mL twice daily	26.6 mg twice daily	3 mL twice daily
≥60 kg	39.9 mg twice daily	4.5 mL twice daily	35.4 mg twice daily	4 mL twice daily

*If the adverse reaction(s) persist after the first dosage modification, proceed to the second dosage modification.

†If the adverse reaction(s) persist after the second dosage modification, DUVYZAT should be discontinued.



Visit DUVYZATHCP.com to get your patients started today.



IMPORTANT SAFETY INFORMATION (cont'd)

Adverse Reactions

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ITF ARC offers dedicated support to help make it easier for your patients to access therapy

ITF ARC offers support that helps make insurance coverage navigation easier, helps address your patients' financial concerns, and encourages adherence to therapy.

ITF ARC can help your patients with:

- Insurance navigation
- Education and adherence support

ITF ARC also offers access solutions for your patients including:

- Copay assistance for eligible commercially insured patients whose health plan covers DUVYZAT® (givinostat)
- Education about third-party resources
- Patient assistance program for eligible uninsured and underinsured patients
- Temporary supply programs

Have questions?

Contact a case manager at ITF ARC.

[1-855-4 ITF ARC \(1-855-448-3272\)](tel:1-855-448-3272)

8 AM-8 PM ET, Monday-Friday



**To get your patients started today,
[download the Patient Start Form](#)**

Each patient's eligibility for access programs is evaluated on an individual basis. To be eligible, patients must first meet the FDA-approved indication. All programs may be modified or discontinued at any time based on eligibility, state and federal laws, and program availability.

Restrictions apply. See full restrictions for ARC Copay Program, Patient Assistance Program, and temporary supply programs at <https://www.duvyzathcp.com/patient-support/#eligibilityModal>



INDICATION

DUVYZAT is a histone deacetylase inhibitor indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 6 years of age and older.

IMPORTANT SAFETY INFORMATION

Warnings and Precautions

Hematological Changes: DUVYZAT can cause dose-related thrombocytopenia and other signs of myelosuppression. Monitor blood count every 2 weeks for the first 2 months, at month 3, and every 3 months thereafter. Modify the dosage for confirmed thrombocytopenia. Discontinuation may be needed if abnormalities worsen.

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QTc Prolongation: DUVYZAT can cause prolongation of the QTc interval. Avoid use of DUVYZAT in patients who are at an increased risk for ventricular arrhythmias (including torsades de pointes), such as those with congenital long QT syndrome, coronary artery disease, electrolyte disturbance or in patients taking concomitant medicinal products known to cause QT prolongation. Obtain ECGs prior to initiating treatment with DUVYZAT in patients with underlying cardiac disease or in patients who are taking concomitant medications that cause QT prolongation.

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Drug Interactions

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Avoid concomitant use with other drugs that prolong the QTc interval; monitor ECG if concomitant use cannot be avoided. If concomitant use cannot be avoided, obtain ECGs when initiating, during concomitant use, and as clinically indicated. Withhold DUVYZAT if the QTc interval is >500 ms or the change from baseline is >60 ms.

To report SUSPECTED ADVERSE REACTIONS, contact ITF Therapeutics LLC at [1-833-582-4312](tel:1-833-582-4312) or FDA at [1-800-FDA-1088](tel:1-800-FDA-1088) or www.fda.gov/medwatch.

Please see [full Prescribing Information](#) for additional safety information.

PROTECT your patients against DMD progression



DUVYZAT is the only nonsteroidal treatment indicated for all patients with DMD 6 years of age or older, regardless of genetic mutation or ambulatory status^{1,5,*}



PROTECTION against DMD progression^{1,5}

DUVYZAT + corticosteroids demonstrated statistically significant preservation of muscle function as measured by 4SC results, and greater preservation of motor function as shown by NSAA scores (secondary endpoint),[†] compared to placebo + corticosteroids.

*The EPIDYS clinical trial did not include nonambulatory patients.⁵

[†]Nominally significant but not statistically significant based on the prespecified multiplicity adjustment.¹

IMPORTANT SAFETY INFORMATION (cont'd)

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References: 1. DUVYZAT. Prescribing information. ITF Therapeutics; 2024. 2. Yang C, Croteau S, Hardy P. Histone deacetylase (HDAC) 9: versatile biological functions and emerging roles in human cancer. *Cell Oncol [Dordr]*. 2021;44(5):997-1017. 3. Sandonà M, Cavioli G, Renzini A, et al. Histone deacetylases: molecular mechanisms and therapeutic implications for muscular dystrophies. *Int J Mol Sci*. 2023;24(5):4306. 4. Seto E, Yoshida M. Erasers of histone acetylation: the histone deacetylase enzymes. *Cold Spring Harb Perspect Biol*. 2014;6(4):a018713. 5. Mercuri E, Vilchez JJ, Boespflug-Tanguy O, et al; EPIDYS Study Group. Safety and efficacy of givinostat in boys with Duchenne muscular dystrophy (EPIDYS): a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial. *Lancet Neurol*. 2024;23(4):393-403. 6. Consalvi S, Saccone V, Giordani L, Minetti G, Mozzetta C, Puri PL. Histone deacetylase inhibitors in the treatment of muscular dystrophies: epigenetic drugs for genetic diseases. *Mol Med*. 2011;17(5-6):457-465. 7. Muntoni F, Signorovitch J, Sajeev G, et al. Meaningful changes in motor function in Duchenne muscular dystrophy (DMD): a multi-center study. *PLoS One*. 2024;19(7):e0304984. 8. Ayyar Gupta V, Pitchforth JM, Domingos J, et al. Determining minimal clinically important differences in the North Star Ambulatory Assessment (NSAA) for patients with Duchenne muscular dystrophy. *PLoS One*. 2023;18(4):e0283669. 9. Vandeborne K. Givinostat in DMD: results of the Epidys study with particular attention to MR measures of muscle fat fraction. Oral presentation at Muscular Dystrophy Association Clinical & Scientific Conference; March 19-22, 2023; Dallas, TX. 10. Data on file, ITF Therapeutics.