



The only nonsteroidal treatment indicated for Duchenne muscular dystrophy in people 6 years of age and older, regardless of their genetic variant or ability to walk.\*

# PROTECTION

OF MUSCLE STRENGTH AND FUNCTION†

\*The DUVYZAT clinical trial did not include people who were not able to walk on their own.

†As measured by the 4-stair climb compared to steroids alone.



Ryan, taking DUVYZAT for 4+ years.

## Indication

### What is DUVYZAT?

DUVYZAT is a prescription medicine for the treatment of Duchenne muscular dystrophy [DMD] in people 6 years of age and older. It is not known if DUVYZAT is safe and effective in children under age 6.

### Important Safety Information

#### What is the most important information I should know about DUVYZAT?

- **Low platelet counts in your blood (thrombocytopenia).** Platelets are important for blood clotting, so having fewer can increase your risk of bleeding or bruising. Your doctor will check your blood count before you start DUVYZAT and regularly during treatment for signs of thrombocytopenia. Call your doctor right away if you notice unusual bleeding or small red or purple spots on the skin.

Please see full Important Safety Information on pages 14–15.

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# Understanding Duchenne muscular dystrophy

## What is Duchenne?



**Duchenne muscular dystrophy (often called DMD or Duchenne) is a rare genetic disorder that mainly affects boys.** Duchenne symptoms begin to appear in early childhood. People with Duchenne have a mutation (or change) in a specific gene that causes this disease. This leads to a lack of dystrophin and histone deacetylase (HDAC) overactivity. Dystrophin is a protein that plays a role in muscle strength, and HDAC is an enzyme that—when overactive—interferes with the body’s ability to repair damaged muscle.



**When someone has Duchenne, their muscles weaken over time.** This makes it more difficult for them to walk, lift their arms, and perform daily tasks. Eventually, people with Duchenne lose the ability to walk. In addition to physical symptoms, people with Duchenne may also have intellectual differences, including difficulties with attention, memory, and learning.



**Duchenne is a progressive condition,** meaning symptoms get worse over time and cannot be reversed. For that reason, early diagnosis and care are important. Although there is no cure yet, treatment options are available to help those with Duchenne. Some treatments may help manage symptoms while others may slow the progression of the disease.



**Diagnosing Duchenne as early as possible is important because early treatment can help preserve muscle function.**

## Important Safety Information (cont’d)

**What is the most important information I should know about DUVYZAT? (cont’d)**

- **Increased levels of fat (triglycerides) in your blood.** You may not have any symptoms, so your doctor will do blood tests before you start DUVYZAT and regularly during treatment to check your triglyceride levels.

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*“We’re able to overcome our fears about the diagnosis even though we’re doing things very differently.”*

*— Gisela and Carlos, parents to Naythan, taking DUVYZAT™ (givinostat) for 4+ years.*





# DUVYZAT is an HDAC inhibitor

DUVYZAT is not a steroid and works differently than other types of medicines for Duchenne. It can be used for all genetic variants.

## A deeper dive into HDAC inhibition



### How HDAC should work in healthy muscle

HDACs are enzymes located in the body's cells that play a key role in maintaining and repairing muscles. They do this by telling proteins how to repair muscle fiber.



### How HDAC works in Duchenne

In Duchenne, the HDAC enzymes become overactive. The increased HDAC activity causes chronic muscle inflammation, decreased muscle repair, and replacement of muscle with fat and scar tissue.

## Important Safety Information (cont'd)

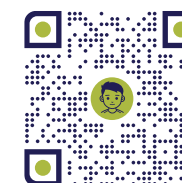
### What is the most important information I should know about DUVYZAT? (cont'd)

- **Frequent watery loose stools (diarrhea) and vomiting.** DUVYZAT can cause vomiting and moderate to severe diarrhea. If diarrhea occurs, you should keep track of the frequency and severity of your diarrhea symptoms, drink plenty of fluids, and contact your doctor.



### DUVYZAT and HDAC inhibition

DUVYZAT is thought to work by inhibiting (blocking) HDAC overactivity. This is different from how other types of medicines work to help people with Duchenne.



**SEE WHAT MAKES DUVYZAT DIFFERENT  
THAN OTHER MEDICINES FOR DUCHENNE.**



Talk to your healthcare provider about DUVYZAT, a unique, nonsteroidal medicine for Duchenne.



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# How DUVYZAT was studied



The clinical trial was 18 months long



Participants received steroids along with either DUVYZAT or placebo

- During the trial, the DUVYZAT dose was based on weight and was reduced if certain side effects occurred



The clinical trial included 179 participants\* with a wide range of genetic variants of Duchenne:

- The effectiveness of DUVYZAT was studied in 120 participants
- The safety of DUVYZAT was studied in 179 participants

\*The median age was 9.8 years, meaning that half of the participants were younger than 9.8 years, and the other half were 9.8 years or older.



Ryan, taking DUVYZAT for 4+ years.

# Study measurements



Researchers recorded key data points that helped measure how far Duchenne had progressed in participants who took DUVYZAT compared to those who were only taking steroids.

## MAIN MEASURE



4-stair climb

How fast participants could **climb 4 stairs** before and after 18 months of using DUVYZAT and steroids, compared with steroids alone



NSAA<sup>1</sup>

Participants' **mobility and muscle function** across multiple muscle groups as measured by the **North Star Ambulatory Assessment (NSAA)**



Muscle strength

A **muscle strength test** that included how well participants could bend their elbow and straighten their knee



Fat tissue

How much **fat was in the muscle**, a measurement of muscle loss

<sup>1</sup>The NSAA is a test that helps healthcare providers understand how well people with Duchenne can move and do daily activities by watching them perform specific tasks.

## Important Safety Information (cont'd)

### What is the most important information I should know about DUVYZAT? (cont'd)

- If thrombocytopenia, increased triglycerides, or diarrhea cannot be managed, your doctor may change your dose or stop your treatment with DUVYZAT, if needed.

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## DUVYZAT protects from Duchenne progression

The clinical trial measured whether DUVYZAT + steroids more effectively preserved muscle strength and function than steroids alone (placebo).

### MAIN MEASURE



Participants taking DUVYZAT

**Completed 4-stair climb 1.78 seconds faster**

than participants taking steroids alone, **demonstrating significant protection.\***

\*This was measured by the average time it took participants in both groups to climb 4 stairs at the end of 18 months.

### Additional measurements of protection

After 18 months, participants taking DUVYZAT + steroids:



- **Maintained motor function abilities longer** compared with those who took placebo, based on NSAA scores

– Those who took DUVYZAT + steroids saw an average of 1.91 fewer points of decline on the NSAA than those who took just placebo



- **Maintained more ability** to bend their elbow or straighten their knee, a measure of muscle strength

In this study, people taking DUVYZAT showed improvements in NSAA scores and flexibility. While these results are encouraging, this analysis wasn't able to confirm improvements were the result of treatment.



**DUVYZAT also reduced how much fat was in the muscle, a measurement of muscle loss. Similar to NSAA scores and muscle flexibility, the analysis wasn't able to prove that this result was due to the treatment.**

## Possible side effects of DUVYZAT

The DUVYZAT clinical trial included 179 participants in the safety analysis. Common side effects are listed below. Talk to your healthcare provider about how to best manage side effects if they do occur.



### Most common side effects in the phase 3 clinical trial<sup>†</sup>

Diarrhea [37%], abdominal pain [34%], low platelet levels [33%], nausea/vomiting [32%], high triglyceride levels [23%], elevated temperature/fever [13%], muscle aches [9%], rash [9%], joint pain [8%], fatigue [8%], constipation [7%], and decreased appetite [7%]

For patients who experienced diarrhea, it usually occurred in the first few weeks of treatment. Most of the time it was mild and manageable. Very few people had severe cases. Only one person needed to pause their treatment due to diarrhea, but no one stopped taking DUVYZAT because of it

<sup>†</sup>Experienced by more than 5% of participants in the clinical trial.

- Your healthcare provider will monitor you or your loved one for changes in blood counts
- If side effects do occur, your healthcare provider can adjust the dose to help reduce them



**Talk to your healthcare provider about possible side effects of DUVYZAT.**

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## Twice-daily dosing

### How to take DUVYZAT



- DUVYZAT is a liquid medicine that can be taken at home, twice a day
- The starting dose for DUVYZAT is based on weight



DUVYZAT can be used on its own or along with steroids. Talk to your healthcare provider about DUVYZAT and any other medications you or your loved one are taking. See full Important Safety Information.

### Important Safety Information (cont'd)

Before taking DUVYZAT, tell your doctor about all of your medical conditions, including:

- any heart problems or medicines you take that could increase your chance for irregular heart rhythms.
- any bleeding problems.

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10 Please see full Prescribing Information and Medication Guide in pocket.

## Establish a daily routine with DUVYZAT

### The three S's of success:



**S**ame time (morning and night)



**S**et dose reminders



**S**nack on! (can be taken with food)



*I have a reminder on my phone [to remember to take it]...*


– Naythan, taking DUVYZAT for 4+ years.






# What to expect when starting DUVYZAT

Before starting DUVYZAT, your healthcare provider will perform:



Lab tests

**Blood tests** to check levels of platelets (tiny cell fragments that help blood clot) and triglycerides (a type of fat in your blood).



Heart test

An **electrocardiogram** for those who have an underlying heart condition or who are taking medicines that might affect heart rhythm.

Once you have started taking DUVYZAT, your healthcare provider will closely monitor your health through regular blood tests. This will allow your healthcare team to ensure DUVYZAT is working as expected. If side effects occur, contact your healthcare provider.

## Important Safety Information (cont'd)

**Tell your doctor about all of the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements.







Taking DUVYZAT with certain other medicines may affect each other. Taking DUVYZAT with other medicines can cause serious side effects. Do not start or stop other medicines without talking to your doctor.


# Regular testing with DUVYZAT




Blood tests you can expect after starting DUVYZAT:

- Blood cell count (complete blood count [CBC] + differential) to check the levels of different types of cells in your blood
- Triglycerides to check the level of a type of fat in your blood

Monitoring scheduling												
Month	1*	2*	3	4	5	6	7	8	9	10	11	12
Blood test												
*Blood tests are every 2 weeks for the first 2 months.												



These tests are more frequent at the beginning of treatment.  
Over time, they will become less frequent.



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- **Increased levels of fat (triglycerides) in your blood.** You may not have any symptoms, so your doctor will do blood tests before you start DUVYZAT and regularly during treatment to check your triglyceride levels.
- **Frequent watery loose stools (diarrhea) and vomiting.** DUVYZAT can cause vomiting and moderate to severe diarrhea. If diarrhea occurs, you should keep track of the frequency and severity of your diarrhea symptoms, drink plenty of fluids, and contact your doctor.
- If thrombocytopenia, increased triglycerides, or diarrhea cannot be managed, your doctor may change your dose or stop your treatment with DUVYZAT, if needed.

### Before taking DUVYZAT, tell your doctor about all of your medical conditions, including:

- any heart problems or medicines you take that could increase your chance for irregular heart rhythms.
- any bleeding problems.

**Tell your doctor about all of the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

Taking DUVYZAT with certain other medicines may affect each other. Taking DUVYZAT with other medicines can cause serious side effects. Do not start or stop other medicines without talking to your doctor.

### What are the possible side effects of DUVYZAT?

- **DUVYZAT can cause serious side effects, including** changes in the electrical activity of your heart called QT prolongation. QT prolongation can increase the risk of developing a type of irregular heart rhythm known as Torsades de Pointes. Call your doctor right away if you feel faint, have an irregular heartbeat, feel dizzy, or lose consciousness. **See the section titled “What is the most important information I should know about DUVYZAT?” for more information about side effects.**



The most common side effects (occurring in >5% of DUVYZAT-treated patients) included diarrhea, abdominal pain, low platelet levels, nausea/vomiting, high triglyceride levels, elevated temperature/fever, muscle aches, rash, joint pain, fatigue, constipation and decreased appetite.

These are not all of the possible side effects of DUVYZAT. For more information, ask your doctor or pharmacist.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

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## Dedicated support through ITF ARC

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

Here are some of the ways ITF ARC may be able to help you access therapy:



### Insurance navigation:

- Insurance coverage benefits verification
- Prior authorization and appeal support, as needed
- Medication deliveries to home or preferred address



### Education and adherence:

- Personalized pharmacist support
- Translation services available as needed
- Education materials about how DUVYZAT may be able to help
- Coordination between families and healthcare providers, including refill reminders
- Patient support group resources

We believe everyone deserves access to the treatment they need, regardless of their financial situation or insurance status. That's why ITF ARC provides a variety of support options to address individual needs and ensure continuous access to therapy.

ITF ARC provides a variety of financial support options for eligible patients:



Copay Program



Patient Assistance Program



Temporary supply programs

## Getting started with ITF ARC\*

If you and your healthcare provider decide that DUVYZAT is right for you, ITF ARC is here to help.



To get the most out of ITF ARC, follow these tips:



As part of the prescription process, **ask your doctor to complete the Patient Start Form** to be enrolled in ITF ARC



**Provide your mobile number** for ease of communication and important updates



**Save the pharmacy's number in your phone.** Since calls from pharmacies can often appear as unknown numbers, this can help you make sure to answer or return calls as needed

If your healthcare provider has enrolled you in ITF ARC and you have not heard back about the status, reach out to a case manager at ITF ARC.



For more information, visit [duvyzat.com/ITFARC](https://duvyzat.com/ITFARC).



Have questions?  
Contact a case manager at ITF ARC.

1-855-4 ITF ARC (1-855-448-3272) 8 AM-8 PM ET, Monday-Friday

\*Each patient's eligibility for access programs is evaluated on an individual case-by-case basis. All programs are designed to comply with state and federal regulations. All programs may be modified or discontinued at any time for any reason, including eligibility criteria, state and federal laws, and program availability.

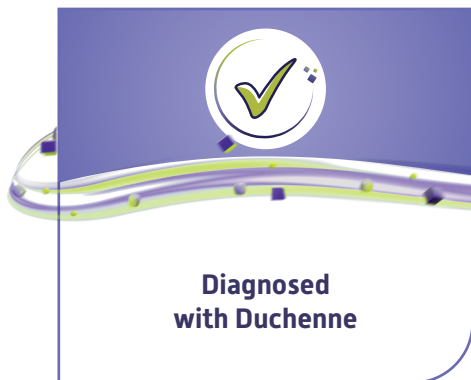
Restrictions apply. See full restrictions for ITF Therapeutics' Copay Program, Patient Assistance Program, and temporary supply programs at [duvyzat.com/support-and-resources/patient-support/](https://duvyzat.com/support-and-resources/patient-support/).

# Is DUVYZAT right for you or your loved one?

In a clinical trial, DUVYZAT + steroids more effectively preserved muscle strength and function than steroids alone (placebo).



**In Duchenne, every day makes a difference. Ask your healthcare provider about how DUVYZAT may preserve muscle strength and function if you or your loved one are:**



**SCAN TO LEARN MORE ABOUT DUVYZAT AND SIGN UP FOR UPDATES.**

## Important Safety Information (cont'd)

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