



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.

Ryan, taking DUVYZAT for 4+ years.

Indication

What is DUVYZAT?

DUVYZAT is a prescription medicine that is used 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 6 years of age.

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, and a decrease in their numbers can lead to an increased risk of bleeding or bruising. Your healthcare provider will check your blood count before you start DUVYZAT and regularly during treatment for any signs of thrombocytopenia. Call your healthcare provider right away if you notice any unusual bleeding or small red or purple spots on the skin called petechiae. Your healthcare provider may change your dose of DUVYZAT if your blood platelet counts continue to be low or may stop your treatment with DUVYZAT.

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

Please see full Prescribing Information and Medication Guide in pocket.

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 muscles.



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)

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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: a unique oral treatment for Duchenne

DUVYZAT works differently than other types of medicines for Duchenne. It is not a steroid. DUVYZAT is a type of medicine known as an HDAC inhibitor. It can be used for all genetic variants.

How DUVYZAT protects muscle function



- In Duchenne, an enzyme called HDAC is overactive
- Overactive HDAC is like a faucet turned all the way to “hot,” with steam representing unchecked inflammation
- Just as the overly hot water can be harmful, ongoing inflammation contributes to muscle damage and interferes with muscle repair



- DUVYZAT works by inhibiting HDAC, which you can think of as turning down the hot water



- By turning down the HDAC (or hot water), DUVYZAT calms inflammation (or cools the temperature), helping preserve muscle strength and function, and increases muscle repair

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 healthcare provider. Your healthcare provider may change your dose of DUVYZAT if the diarrhea cannot be managed or does not go away. Your healthcare provider may also stop your treatment with DUVYZAT.



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.



The DUVYZAT difference

That's where DUVYZAT comes in—it is a unique treatment for Duchenne that works by inhibiting HDAC overactivity.



While the exact process is unknown, it is thought that DUVYZAT helps reduce inflammation, increase the body's ability to repair muscles, and slow muscle loss.



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DUVYZAT was studied in one of the largest Duchenne clinical trials to date



18 months long



Participants received steroids along with either DUVYZAT or placebo



Included 179 participants* with a wide range of genetic variants of Duchenne

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



Ryan, taking DUVYZAT for 4+ years.

Study measurements

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



4-stair climb

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



NSAA[†]

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 muscle was preserved compared to being replaced by fat, a measurement of muscle loss

Important Safety Information (cont'd)

Before taking DUVYZAT, tell your healthcare provider about all of your medical conditions, including if you:

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

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[†]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.

DUVYZAT protects from Duchenne progression

In the clinical trial, DUVYZAT and steroids were more effective at preserving muscle strength and function than steroids alone.



*DUVYZAT helped maintain muscle strength and motor function about 40% better than using steroids alone**

*As measured by the mean change in time from baseline to 18 months of 4-stair climb in DUVYZAT vs steroids alone (placebo).

More measurements of protection†

After 18 months, participants taking DUVYZAT and steroids:

- Maintained motor function abilities **longer** compared to those who took placebo, based on NSAA scores
- Maintained **more ability** to bend their elbow or straighten their knee, a measure of muscle strength

DUVYZAT also reduced how much muscle was replaced by fat, a measurement of muscle preservation.

†After clinical trials, researchers use statistics to see if the results are really from the new medicine and not just by chance. In this trial, people taking DUVYZAT showed improvements in NSAA scores and muscle flexibility. However, the analysis wasn't able to prove that these results were due to the treatment.

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Possible side effects of DUVYZAT

DUVYZAT was studied in multiple clinical trials involving 222 participants, many of whom were taking DUVYZAT for more than 2 years. Talk to your healthcare provider about how best to manage side effects if they do occur.



Most common side effects in the phase 3 clinical trial*

- Diarrhea (37%), abdominal pain (34%), low platelet levels (33%), nausea/vomiting (32%), high triglyceride levels (23%), and elevated temperature/fever (13%)
- 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

Your healthcare provider will monitor you or your loved one for changes in blood counts.

*Experienced by ≥10% of participants.

If side effects occur, your healthcare provider can adjust the dose to help reduce them.

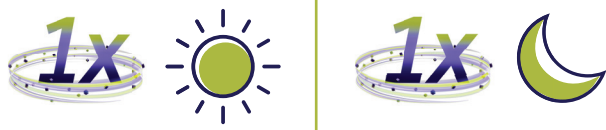


Ryan, taking DUVYZAT for 4+ years.

DUVYZAT study results

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)

Tell your healthcare provider 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 healthcare provider.

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Establish a daily routine with DUVYZAT

The three S's of success:



Same time (morning and night)



Set dose reminders



Snack 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.

Your healthcare provider will frequently test your or your loved one's blood counts throughout treatment to watch for side effects.

Important Safety Information (cont'd)

DUVYZAT can cause serious side effects, including:

- See "What is the most important information I should know about DUVYZAT?"
- **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 healthcare provider right away if you feel faint, have an irregular heartbeat, feel dizzy, or lose consciousness.

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I want to be able to preserve the muscle in him so he's able to continue being more independent in his life and keep doing the things that he likes to do.

– Susan, Ryan's mom



Susan, Ryan's mom.

Not an actual doctor.

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The most common side effects of DUVYZAT included diarrhea, nausea, vomiting, stomach pain, low platelet counts in the blood, increased fat level in the blood and fever.

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

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

Please see full Prescribing Information and Medication Guide.



Ethan, taking DUVYZAT for 4+ years.

DEDICATED SUPPORT THROUGH ITF ARC

ITF ARC [Access, Resources, Care] 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
- Educational materials about DMD and how DUVYZAT may be able to help
- Coordination between families and healthcare providers, including refill reminders

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.



For more information, visit 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

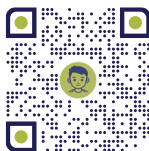
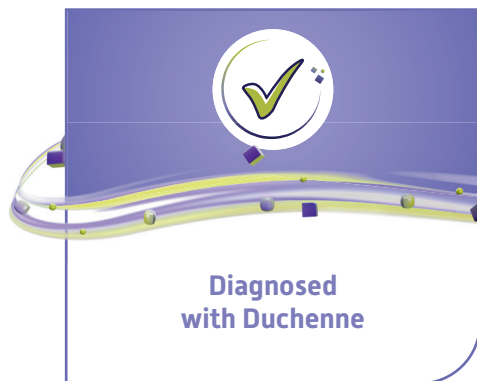
*Each patient's eligibility for access programs is evaluated on an individual case-by-case basis. To be eligible, use of DUVYZAT must be consistent with the FDA-approved indication. All programs are designed to comply with state and federal regulations. All programs may be modified or discontinued at 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/ITFARC.

Is DUVYZAT right for you or your loved one?

In a clinical trial, DUVYZAT and steroids were more effective at preserving 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.

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