

Medical terms

Intended for patients prescribed Duvyzat® ▼ (givinostat)
Including parents, guardians and caregivers

This medicine is subject to additional monitoring. Information about reporting side effects can be found on the back page.
If you have any questions about dosing or administering Duvyzat®, please speak with your healthcare team.

All photographic images
within this booklet
feature real patients.
They are shown for
illustrative purposes
only, and individual
experiences may vary

What do the medical words mean?

Explanations of important medical terms commonly used
when discussing Duchenne and how Duvyzat® works



Understanding scientific terms



Here are some words and definitions often used to explain how Duvyzat® works

Most of the key scientific terms are included, but if anything is unclear or you notice a term is missing, you can always ask the healthcare team for clarification.

ACETYL GROUPS

Tiny chemical tags that change how proteins behave.

ACETYLATION

Adding acetyl groups to a protein to change activity.

CONNECTIVE TISSUE

Strong material in the body that holds muscles and organs together.

CORTISONE

Medications that can inhibit inflammation in the body.

DEACETYLATION

Removing an acetyl group which can, depending on the target increase or decrease protein activity.

DNA

Instruction manual found in most cells, telling them how to work.

DUCHENNE MUSCULAR DYSTROPHY (DMD)

A rare muscle disease where the muscles gradually weaken over time. It almost only affects boys and usually begins in early childhood.

DYSTROPHIN

This is a protein that ensures the stability of muscle cells. In Duchenne, it's missing or doesn't work properly.

FAP CELLS

Fibro-adipogenic progenitors or 'FAP' cells help repair muscle but can form scar or fat.

FAT CELLS

Cells that store fat, and replaces muscle lost in Duchenne.

FIBROBLASTS

Cells that make connective tissue and help healing.

GIVINOSTAT

This is the active ingredient in Duvyzat®, which can help reduce inflammation in the muscles and slow down muscle breakdown.

GROWTH FACTORS

Signals that tell cells to grow, repair, or divide.

HAT ENZYMES

Proteins that add acetyl groups to other proteins.

HDAC (HISTONE DEACETYLASE) PROTEIN

A protein that removes acetyl groups from histones. In Duchenne HDAC is overactive, promoting inflammation and hindering muscle regeneration.

HISTONES

Proteins that DNA wraps around to stay organised.

IMMUNE CELLS

Cells that protect the body from germs and damage.

INFLAMMATION

The body's natural, protective response to injury, germs, or irritation. Often resulting in swelling and redness.

MUSCLE FIBRES

Long cells that make up your muscles, they contract and relax helping you move.

ORAL SUSPENSION

A liquid that has a medicine mixed into it, that is then swallowed. Duvyzat® is this kind of suspension – it is administered using a special syringe.

PROTEIN SKELETON

Inner support structure that keeps a cell's shape.

SORBITOL

A sugar substitute frequently used in food and medicine products.

STEM CELLS

Cells that divide into specialised cells (such as muscle cells) or make more stem cells.

THROMBOCYTES (PLATELETS)

Small pieces of cells that help wounds stop bleeding. If there are too few of them in the blood, bleeding and bruising will occur more easily.

THROMBOCYTOPENIA

This is where there is a reduced number of platelets in the blood. It can lead to increased tendency to bleed or small red spots on the skin.

TRIGLYCERIDES (BLOOD FATS)

These are a type of fat that are found within blood. If there are too many of them in the blood, this is referred to as "elevated blood fat levels". This is usually not noticeable, but it can be detected in the laboratory.

CHROMOSOME

A chromosome is a structure inside your cells that carries your DNA.

X CHROMOSOME

It is a sex-determination chromosome. Females have two copies (XX), and males have one X and one Y (XY). The genetic defect in Duchenne is located on the X chromosome, which is why almost only boys are affected.



MyEveryDay
Learning

These medical definitions have been worded for the intended audience and accurately reflect the current scientific information available.

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Reporting of side effects

If you get any side effects, talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. You can also report side effects directly via the Yellow Card Scheme at: <https://yellowcard.mhra.gov.uk>

By reporting side effects, you can help provide more information on the safety of this medicine. This medicine is subject to additional monitoring. This will allow quick identification of new safety information. You can help by reporting any side effects you may get. See <https://yellowcard.mhra.gov.uk> for how to report side effects.