

Givinostat Early Access Programme

This leaflet gives important information to help you decide if you want your child to join the Givinostat Early Access Programme at Evelina London Children's Hospital.

It is your choice whether or not you want your child to join this programme. You might want to talk to your family or friends before making your decision. Please take your time, and make a decision that is right for you and your child.

Early access programmes

Early access programmes are a way for people with serious medical conditions to access new medicines for treatment. Programmes like this can bridge the time between clinical trials and health services funding treatments. It is sometimes called 'compassionate use'.

This early access programme provides givinostat for the treatment of Duchenne muscular dystrophy (DMD). Givinostat will be provided free of charge by the manufacturer, for patients registered on this early access programme, until the National Institute for Health and Care Excellence (NICE) decides if givinostat should be made available through the NHS.

Givinostat

Givinostat is a medicine that might slow down the progression of muscle weakness for patients with DMD. In DMD, healthy muscle is replaced over time by fibrous tissue, and this is called fibrosis. Givinostat aims to reduce fibrosis and also to improve the regeneration of muscle.

How has givinostat been studied?

Givinostat has been tested in clinical trials. About 200 patients with DMD have received givinostat in clinical trials. In December 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) conditionally approved it to treat DMD in the UK. There is more information about this on the Government [website](https://www.gov.uk/government/news/givinostat-conditionally-approved-to-treat-patients-with-duchenne-muscular-dystrophy-dmd):

<https://www.gov.uk/government/news/givinostat-conditionally-approved-to-treat-patients-with-duchenne-muscular-dystrophy-dmd>

Results of the givinostat clinical studies

In clinical trials, boys who had DMD and could still walk were given givinostat, or a placebo (liquid that had no medicines in it). The boys given givinostat did not deteriorate as much as those given the placebo, and had higher scores on some assessments of muscle function.

Who qualifies for this early access programme

Your child is being invited to take part in this early access programme because they have DMD and they might benefit from treatment with givinostat. It might keep their muscles strong for longer, so they can do things like walking or getting up from the floor for longer than they otherwise would.

Givinostat is not suitable for everyone, so some patients will not be able to take part in this programme.

Other trials or studies

It is very important that you know that if your child starts taking givinostat through the early access programme they will not be eligible for any clinical study or trial of other new treatments.

Joining the programme

Before any treatment can start, you'll be asked to read this information and sign the consent form.

The treating doctor will arrange some tests (for example, blood tests) for your child to check if it is safe for them to take givinostat.

Your child's participation in this programme is voluntary. If you decide you do not want your child to take part in this programme, your child will continue with their current medical care.

How givinostat is given

Givinostat is a liquid that is taken by mouth (orally). It is usually taken with food, two times each day. It needs to be taken at about the same times every day.

You'll need to collect givinostat from the pharmacy at Evelina London Children's Hospital. It will not be available from your local pharmacy.

A review of medicines your child is already taking must be done before they can be given givinostat. This is to make sure they can be taken safely with givinostat.

If your child turns 16 while on the programme

In the UK, young people aged 16 and over are generally presumed to have the legal capacity to give consent for their own medical treatment, including participation in clinical programmes like this one.

If your child turns 16 while already in the programme, they will be asked to confirm if they want to continue. At that time they will:

- have to read this information about the programme
- be asked to sign a new consent form to confirm their decision to continue treatment

This makes sure that their legal right to make decisions about their own healthcare is respected.

Parents or legal guardians will still be encouraged to stay involved in discussions, and support their child's decision-making. The formal consent must come from the young person once they are 16, provided they are deemed competent to decide.

During the treatment programme

The frequency and type of clinical assessments your child needs will be determined by their doctor. This will be for your child's safety.

Certain safety assessments (such as blood tests) will need to happen at specific timepoints/frequency.

Before starting givinostat, your child will have an electrocardiogram (ECG). This measures their heart's electrical activity and is not painful. Your doctor will let you know if this needs to be repeated at any time during their treatment.

We will do blood tests:

- every 2 weeks for the first 2 months
- at 3 and 6 months
- every 6 months after that

If there are concerns about your child's health, more frequent blood tests may be needed.

Your child will continue to have their normal medical and physiotherapy clinic checks. These are usually every 6 months.

Side effects of taking givinostat

In addition to the known side effects listed below, there may be risks that are not yet known. This is because it is a new treatment and the side effects may be very rare. Sometimes even very serious side effects are not recognised until very large numbers of people have taken a new medicine.

As with any medicine, an allergic reaction is also possible.

After 222 patients with DMD were treated with givinostat in clinical treatment trials, these possible side effects were identified.

Very common (more than 1 in 10 patients)

- A drop in the normal number of platelets. This might result in easy bruising or bleeding
- Diarrhoea
- Vomiting
- Fever
- tummy (abdominal) pain
- Higher than normal level of a certain type of fat (triglycerides) in the blood
- A high blood triglyceride level. Triglycerides are a type of fat, and too much in your blood might increase the long-term risk for stroke and heart attack.

Common (up to 1 in 10 patients)

- Constipation
- Tiredness (fatigue)
- Decreased appetite
- Rash
- Joint pain
- Muscle pain (myalgia)

Other side effects reported include:

- too little thyroid hormone (sometimes called an underactive thyroid gland). Symptoms may include weight gain, constipation, dry skin, and sensitivity to the cold.

If your child starts taking givinostat, their doctor will monitor for side effects by doing regular blood tests or an ECG.

You should tell their doctor if you notice anything new in your child that might be a side effect of the treatment. If you have any concerns about a possible side effect, you should seek medical advice urgently. They can contact us for more information if needed.

If a side effect relating to givinostat occurs, sometimes we can lower the amount (dose) given. For safety reasons, some children might need to stop taking givinostat. The treatment will also need to be stopped if the any safety assessments, such as the blood tests cannot be done.

Givinostat and other medicines

If your child is taking givinostat, some medicines should be avoided or used only when there is no alternative and the treatment is essential. This includes medicines that prolong a part of the heart rhythm, such as:

- some antibiotics (such as erythromycin, azithromycin and clarithromycin)
- some anti-nausea medicines (such as ondansetron)

Your child should continue taking their steroid (vamorolone) medicine if they are already on it.

Givinostat and other health conditions

If your child gets any new health problems while they are taking givinostat, you should tell their doctor immediately. The doctor will help work out if the problem is because of the givinostat, and change the treatment if necessary. They will also report any suspected side effects to the MHRA.

Pregnancy and birth control

If your child is sexually active with a partner who could become pregnant, both your child and their partner must use highly effective contraception. They must do this for the entire time your child is taking givinostat, and for 3 months after the final dose.

The treating doctor will discuss suitable birth control options with you and your child, and might also ask questions about pregnancy or whether your child or their partner could become pregnant, to keep everyone safe during the programme.

Sharing information with the manufacturer of givinostat

Italfarmaco make givinostat, and it is managed by ITF Pharma Ltd in the UK.

The only information your doctor will share with them is your child's diagnosis of DMD and their age. This limited information is necessary to confirm your child's eligibility for the programme and to make sure the medicine is provided appropriately. No personal details such as your child's name or contact information will be shared.

By signing the consent form, you are confirming that you agree to this specific information being shared with Italfarmaco for the purpose of accessing givinostat.

Leaving the programme

Even if your child joins the programme, they can leave it at any time. You will not need to give a reason why you are making this decision. However, if you decide that you want your child to stop taking givinostat, you must contact the treating doctor. This is so the doctor can explain to you and your child the safest way to stop the treatment.

Sometimes the treating doctor might need to end your child's participation in the programme, even if you or your child want to stay in it.

This can happen if:

- there is a change in your child's medical condition and continuing givinostat might be harmful
- you fail to follow the treating doctor's instructions, for example the safety blood tests
- your child no longer meets the programme's eligibility criteria
- the programme comes to an end

Length of the programme

Givinostat is being provided free of charge by the manufacturer, as part of this programme. This will continue until a national decision is made about whether the NHS will fund the medicine (for example, by NICE in England, or the Scottish Medicines Consortium [SMC] in Scotland).

- If NHS funding **is approved**, the programme will end and the medicine will be provided through the NHS instead.
- If NHS funding **is not approved**, the hospital will no longer be able to provide givinostat, unless it continues to be provided compassionately by the company.

Keeping you up to date with any changes in the programme

If new findings come up during the programme that could affect your child's safety, or your willingness for your child to take givinostat, we will tell you as soon as possible. This is so you can decide whether to continue or leave the programme.

Important

Do not sign the consent form unless you have been able to ask any questions, and received satisfactory answers.

Signing the consent form means that you voluntarily agree for your child to take part in this programme.

This agreement can be withdrawn at any time.

Contact us

If you have any questions about your child's givinostat treatment or the early access programme, please contact the neuromuscular team,

phone: 07785381995, Monday to Friday 9am to 5pm,

email: gstt.evelinaneurocns-smb@nhs.net

If it is an emergency, please call NHS 111 or 999, who can reach the neurology team if needed.

For more information on conditions, procedures, treatments and services offered at our hospitals, please visit **web** www.evelinalondon.nhs.uk/leaflets

Evelina London medicines helpline

If you have any questions or concerns about your child's medicines, please speak to the staff caring for them or contact our helpline, **phone** 020 7188 3003, Monday to Friday, 10am to 5pm

email letstalkmedicines@gstt.nhs.uk

Your comments and concerns

For advice, support or to raise a concern, contact our Patient Advice and Liaison Service (PALS), **phone** 020 7188 8801 **email** pals@gstt.nhs.uk. To make a complaint, contact the resolution department **phone** 020 7188 3514 **email** complaints2@gstt.nhs.uk

Language and accessible support services

If you need an interpreter or information about your child's care in a different language or format, please contact the department where they have the appointment.

NHS 111

This service offers medical help and advice from fully trained advisers. They are supported by experienced nurses and paramedics. The service is available by phone 24 hours a day, **phone** 111 **web** www.111.nhs.uk (111 online is for people aged 5 or over, and you need to call 111 to get help for a child under 5)

Get involved and have your say: become a member of the Trust

Members of Guy's and St Thomas' NHS Foundation Trust contribute to the organisation voluntarily. We rely on them for feedback, local knowledge and support. Membership is free and you can decide how much to get involved. To find out more, please get in touch,

phone 0800 731 0319 **email** members@gstt.nhs.uk

web www.guysandstthomas.nhs.uk/membership



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