

Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene

Information for the public

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What has NICE said?

Ataluren (Translarna) is recommended as a possible treatment for people with Duchenne muscular dystrophy (DMD) with a nonsense mutation in the dystrophin gene, if:

- they are 5 years and older and
- they can walk and
- there is sign up to the managed patient access agreement in the [managed access agreement](#).

The managed access agreement includes rules for starting and stopping treatment with ataluren, and for assessing how well the treatment is working. NICE will take the information about how well the treatment works into account when the guidance on ataluren is reviewed.

What does this mean for me?

If you (or your) child has Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene, and your doctor thinks that ataluren is the right treatment, you (or your child) should be able to have the treatment on the NHS, providing you sign up to the managed patient access agreement.

Ataluren should be available on the NHS within 3 months of the guidance being issued. Your doctor should ask you if you are happy for details of your (or your child's) treatment to be collected.

However, if NICE does not recommend ataluren for NHS funding when a review of the guidance is published after 5 years, it will no longer be available for any patient.

The condition and the treatment

Duchenne muscular dystrophy is a severe muscle-wasting condition. It is caused by a defective gene (nonsense mutation) that affects the production of dystrophin, a protein needed to build and repair muscle.

Ataluren (Translarna) restores the production of dystrophin.

[NHS Choices](#) may be a good place to find out more.

These organisations can give you advice and support:

- [Action Duchenne](#), 0800 652 6532
- [Muscular Dystrophy UK](#), 0800 652 6532
- [Genetic Alliance UK](#), 0207 704 3141

NICE is not responsible for the quality or accuracy of any information or advice provided by these organisations.

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Accreditation

