

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

Information for the public

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Ataluren (Translarna) is available on the NHS. It is a possible treatment for Duchenne muscular dystrophy resulting from a nonsense mutation in children over 2 and adults, if they can still walk.

Is this treatment right for me?

Your healthcare professionals should give you clear information, talk with you about your options and listen carefully to your views and concerns. Your family can be involved too, if you wish. Read more about [making decisions about your care](#).

Questions to think about

- How well does it work at different stages of the disease?
- What are the risks or side effects? How likely are they?
- How will the treatment affect my day-to-day life?
- What happens if the treatment does not work?
- What happens if I do not want to have treatment? Are there other treatments available?

Information and support

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

These organisations can give you advice and support:

- [Action Duchenne](#), info@actionduchenne.org
- [Duchenne UK](#), info@duchenneuk.org
- [Muscular Dystrophy UK](#), 0800 652 6352
- [Joining Jack](#), info@joiningjack.org

You can also get support from your local [Healthwatch](#).

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

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