

# Raxone- a guide for the Duchenne community

## About Raxone

### What is Raxone?

Raxone (also known as Idebenone) is a drug produced by Santhera Pharmaceuticals. It is used as a treatment for an eye condition called Leber's Hereditary Optic Neuropathy, and may have the potential to treat Duchenne muscular dystrophy.

### How does Raxone work?

Duchenne muscular dystrophy is a muscle-wasting condition caused by the lack of a protein called dystrophin. This leads to cell damage and reduced energy production in muscle cells.

The active substance in Raxone is called *idebenone*, which is believed to improve energy production in muscle cells.

Raxone is believed to help slow down the decline in respiratory function by slowing the rate at which the respiratory muscles weaken.

### Who is eligible?

Raxone isn't currently available for everyone with Duchenne muscular dystrophy but only for those meeting the agreed eligibility criteria.

To be eligible to receive Raxone, patients must be:

- Diagnosed with Duchenne muscular dystrophy;
- Over the age of 10 years old;
- Able to breathe without full time ventilation;
- Showing reduced breathing function. This is measured by forced vital capacity, or FVC, a test that measures the amount of air that can be forcibly exhaled from the lungs after taking the deepest breath possible. To be eligible, your FVC needs to be between 80 and 25%;
- Not currently being treated with steroids;
- Under the care of a neuromuscular doctor at a specialist centre.

Anyone who thinks they may be eligible should speak to their doctor about whether or not Raxone might be appropriate for them.

# Approval of Raxone for Duchenne muscular dystrophy

## Is Raxone approved?

Raxone is not approved as a treatment for people with Duchenne muscular dystrophy.

In 2017, Santhera applied to the European Medicines Agency for authorisation to market Raxone as a treatment for Duchenne muscular dystrophy. The application was refused by the Agency's Committee for Medicinal Products for Human Use (or CHMP), and following an appeal by Santhera, the refusal was confirmed in January 2018.

This means that Raxone cannot be marketed and prescribed for people with Duchenne muscular dystrophy like other drugs. However, Santhera are planning to re-apply for marketing authorisation and in the UK, the drug has been made available through the Early Access to Medicines Scheme.

## Raxone and the Early Access to Medicines Scheme

### What is the Early Access to Medicines Scheme?

The scheme allows patients with life-threatening or seriously debilitating conditions to access unproven medicines before marketing authorisation is given. Importantly, the MHRA (the UK medicines regulator) must believe that the potential benefits of the drug outweigh any potential risks or side effects.

Once part of a scheme, a drug can be prescribed by doctors, who will make a decision with the patient on whether to use a medicine before it is approved.

Under the scheme, Santhera are providing the drug to the NHS free of charge for patients who qualify for it. The company will continue to collect data on the effectiveness and safety of Raxone to support their application to the EMA.

*The Early Access Scheme for Raxone is due to be reviewed in June 2019.*

### Which Trusts are currently prescribing Raxone?

NHS England has a list of the paediatric and adult neuroscience centres that are currently approved to prescribe Raxone.

If you think you could be eligible to receive Raxone, then you should speak to your doctor about whether or not Raxone might be appropriate for you and whether your Trust currently prescribes the treatment.

## How we can help

**We are here to support you.** If you have questions about accessing Raxone or think you are eligible for the treatment but unable to access it then please get in touch with us.

### Action Duchenne

Call: 020 7250 8240

Email: [info@actionduchenne.org](mailto:info@actionduchenne.org)

Website: <https://www.actionduchenne.org>

### Duchenne UK

Call: 0203 096 7496

Email: [info@duchenneuk.org](mailto:info@duchenneuk.org)

Website: <https://www.duchenneuk.org>

### Duchenne Family Support Group

Call: 0800 121 4518

Email: [info@dfsg.org.uk](mailto:info@dfsg.org.uk)

Website: <https://www.dfsg.org.uk>

### Muscular Dystrophy UK

Call: 0800 652 6352

Email: [info@muscular dystrophyuk.org](mailto:info@muscular dystrophyuk.org)

Website: <https://www.muscular dystrophyuk.org>

### DMD Pathfinders

Call: 01273 252525

Email: [info@dmdpathfinders.org.uk](mailto:info@dmdpathfinders.org.uk)

Website: <https://www.dmdpathfinders.org.uk>

