Raxone – a guide for clinicians

About the treatment

What is Raxone?

Raxone (also known as Idebenone) is a drug produced by Santhera Pharmaceuticals. It treats several conditions and has the potential to treat Duchenne muscular dystrophy.

How does the treatment work?

Duchenne muscular dystrophy is a muscle-wasting condition caused by the lack of a protein called dystrophin. This leads to cell damage, impaired calcium homeostasis, elevated oxidative stress, 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. *Idebenone* is an anti-oxidant that acts on mitochondria to improve their functioning. Mitochondria are the structures inside cells that produce energy for the cells to function. Where these are not working properly, they produce toxic forms of oxygen called 'free radicals', and *idebenone* acts against these, which helps improve the mitochondrial functioning.

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

Raxone is not believed to be able to repair fibrosed muscle at a late stage of disease.

Who is eligible?

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

To be eligible to receive Raxone, patients must:

- Have a diagnosis of Duchenne muscular dystrophy;
- Be over the age of 10 years old;
- Show a decline in respiratory function (FVC% predicted 80-25%);
- Not currently be taking glucocorticoids (a type of steroid hormones).
- Be under the care of a neuromuscular doctor at a specialist centre.

About approval of the treatment

About the approval process

The European Medicines Agency (EMA) evaluates medicines based on their safety and efficacy, before deciding whether to approve them. The evaluation is done by the Committee for Medicinal Products for Human Use (CHMP), which looks at the scientific evidence to decide whether a medicine meets quality, safety and efficacy requirements and whether the benefits outweigh the risks. It then approves or denies marketing authorisation for the medicine across the European Union.

Is Raxone approved?

Raxone is currently approved as a treatment for a condition called Leber's optic neuropathy. Santhera Pharmaceuticals are seeking approval for its use in treating people with Duchenne muscular dystrophy.

In September 2017, the CHMP refused marketing authorisation for Raxone as a treatment for Duchenne muscular dystrophy. This decision was appealed by Santhera but the CHMP confirmed the refusal in January 2018.

This means that Raxone has not had European Union approval to be marketed and prescribed for people with Duchenne muscular dystrophy. However, Santhera are planning to re-apply for marketing authorisation.

About getting access

What is the Early Access to Medicines Scheme?

Raxone is available in the UK through the Early Access to Medicines Scheme. The scheme allows patients with life-threatening or seriously debilitating conditions access to medicines which have not yet received marketing authorisation from the European Medicines Agency.

An Early Access to Medicines Scheme is put in place when a medicine hasn't necessarily been proven to be either safe or effective, but that overall, the potential benefits of the medication are believed to outweigh the potential risks of it.

Under the scheme, Santhera are providing the treatment to the NHS free of charge for patients who qualify for it whilst they also continue to collect data on the treatment's effects.

How does the Early Access to Medicines Scheme work?

1. Pharmaceutical companies can apply for a medicine to be designated as a "Promising Innovative Medicine" based on early clinical data. This is where the early data suggests that it may be an effective treatment, but before there is enough evidence to be certain.

2. Once designated a "Promising Innovative Medicine", the Medicines and Healthcare products Regulatory Agency (MHRA) can issue a 'scientific opinion' on the medicine. The scientific opinion summarises the risks and benefits of the medicine based on data gathered from the research already done on the medicine.

3. If the MHRA believes that the benefits of the medicine outweigh the risks of the medicine, it can approve the medicine under the Early Access to Medicines Scheme. This allows prescribers to prescribe it under their own authority, and lets them make a decision with the patient on whether to use a medicine before its license is approved.

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

Which Trusts are currently prescribing Raxone?

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

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
Website: https://www.actionduchenne.org

Duchenne UK
Call: 0203 096 7496
Email: info@duchenneuk.org
Website: https://www.duchenneuk.org

Duchenne Family Support Group
Call: 0800 121 4518
Email: info@dfsg.org.uk
Website: https://www.dfsg.org.uk

Muscular Dystrophy UK
Call: 0800 652 6352
Email: info@musculardystrophyuk.org
Website: https://www.musculardystrophyuk.org

DMD Pathfinders
Call: 01273 252525
Email: info@dmdpathfinders.org.uk
Website: https://www.dmdpathfinders.org.uk