

# Medicine: patisiran (brand name: Onpattro®) for hereditary transthyretin-mediated amyloidosis

Alnylam Pharmaceuticals

## What is patisiran for?

Patisiran is for the treatment of hereditary transthyretin-mediated amyloidosis (hATTR-amyloidosis). hATTR-amyloidosis is a very rare life threatening genetic disease caused by mutations (genetic changes) in the transthyretin (TTR) gene. This leads to the production of abnormal TTR protein which forms build-ups called amyloid deposits in the body's tissues and organs (referred to as amyloidosis). If the amyloid deposits build up around the nerves this causes nerve damage (polyneuropathy).

Patisiran is used to treat adult patients in the first two stages of nerve damage due to hATTR (stage 1, when the patient is able to walk unaided, and stage 2, when the patient can still walk but needs help).

## How does patisiran work?

Patisiran is a medicine made of a small strand of synthetic genetic material called small interfering RNA (siRNA). It binds to the genetic material of the cells that make the faulty TTR protein and block its production. This reduces the formation of amyloid deposits and helps to relieve the symptoms of hATTR amyloidosis.

### What has SMC said?

SMC has accepted patisiran for the treatment of adults with hATTR-amyloidosis and nerve damage as described above.



## Why has SMC said this?

SMC looks at how well new medicines work compared with current treatments available in Scotland and in relation to how much they will cost to buy and administer (for example, if the medicine has to be given at a clinic or side effects have to be monitored).

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland. When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not just those who may be treated with the medicine under consideration.

To do this SMC consider the following:

- Clinical trial and economic evidence from the company that makes the medicine.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.
- Information from patient groups about the potential impact of the medicine on patients and carers.

After careful consideration, applying extra flexibility because patisiran is a medicine for a very rare condition, and after the company applied a confidential discount to the cost of the medicine, SMC was able to accept it as a possible treatment within NHSScotland.

### What does SMC's decision mean for me?

If your healthcare professional thinks that patisiran for use as described above is the right medicine for you, you should be able to have the treatment on the NHS in Scotland.

For further information see:

Medicines in Scotland: What's the right treatment for me?

[www.healthcareimprovementscotland.org/medicinesbooklet.aspx](http://www.healthcareimprovementscotland.org/medicinesbooklet.aspx)



### More information

The organisation below can provide more information and support for people with amyloidosis and their families. SMC is not responsible for the content of any information provided by external organisations.

#### Amyloidosis Research Consortium UK



<http://www.arci.org.uk/> - UK web-site with contact form



0131 550 3866



<https://www.arci.org/> – American web-site for further information

You can find out more about patisiran in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



<http://www.ema.europa.eu>

**Date advice published:** 10 June 2019

**SMC No:** (SMC2157)