

## Plain English Summary

# Eplontersen and vutrisiran for treating hereditary transthyretin amyloidosis with polyneuropathy

## What does the guidance say?

Eplontersen and vutrisiran are not recommended for government subsidy for patients with stage 1 or 2 hereditary transthyretin amyloidosis with polyneuropathy (hATTR-PN).

## What is hereditary transthyretin amyloidosis with polyneuropathy?

Hereditary transthyretin amyloidosis with polyneuropathy (hATTR-PN) is an inherited condition (passed down through families) caused by changes (mutations) in the *transthyretin* (*TTR*) gene. These genetic changes lead to abnormal transthyretin proteins that build up in the body, forming deposits that damage organs and nerves and prevent them from working properly. The condition usually develops in adulthood.

hATTR-PN can affect several parts of the body, including the eyes and digestive tract, but it most commonly affects the nerves in the hands and legs that control feeling and movement, as well as the nerves that control body functions such as digestion and blood pressure. In some people, the heart may also be affected. Nerve damage caused by hATTR-PN worsens over time. Doctors describe how advanced the condition is using four stages which range from stage 0 (no symptoms) to stage 3 (severe impairment). In stage 1, people can walk without help but usually have mild nerve problems, mainly in the lower limbs. In stage 2, people need help to walk and experience moderate nerve problems that have spread to the lower limbs, upper limbs, and trunk.

## What are eplontersen and vutrisiran?

Eplontersen and vutrisiran belong to a group of medicines called transthyretin silencers. They work by reducing the production of the abnormal transthyretin protein that causes nerve damage in hATTR-PN.

Eplontersen and vutrisiran are given as injections under the skin (subcutaneously) and can be self-administered at home.

## Why were they not recommended for subsidy?

ACE evaluates how well a treatment works in relation to how much it costs compared to other treatments. Eplontersen and vutrisiran were not recommended for government subsidy because they did not represent an acceptable use of healthcare resources. If you need eplontersen or vutrisiran for hATTR-PN, you can speak to a medical social worker to find out if there is financial assistance available to help with the cost of treatment.

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## Is this the right treatment for me?

Your doctor may recommend you have eplontersen or vutrisiran for hATTR-PN if they consider it is the best option based on your individual situation. They should give you clear information, listen to your views and concerns, and talk to you about your treatment options.

Some of the questions you may want to ask your doctor when making decisions about your care are:

- How will the treatment affect my day-to-day activities?
- How well does it work compared to other treatments?
- What are the side effects and risks of treatment, and how likely are they?
- How much does the treatment cost, and who can I approach if I want to find out whether there is financial assistance available to help with the cost of treatment?
- How long will I need to be on the treatment for?
- What happens if the treatment stops working?
- What happens if I do not want to have treatment?

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