

# UPDATE ON MEDICINES USED IN HEREDITARY TRANSTHYRETIN AMYLOIDOSIS WITH POLYNEUROPATHY

**HSE** Medicines used in hereditary transthyretin amyloidosis in adult patients with Stage 1 or Stage 2 polyneuropathy

**Reimbursed indication:**  
**Patisiran (Onpattro®)** and **vutrisiran (Amvuttra®)** are indicated for the treatment of hereditary transthyretin-mediated amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy.  
**Inotersen (Tegsedi®)** is indicated for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis.

| Medicine   | Patient population | Route of administration | Dose                                               |
|------------|--------------------|-------------------------|----------------------------------------------------|
| Patisiran  | Adults < 100 kg    | IV infusion             | 300 mcg per kg body weight once every three weeks  |
| Patisiran  | Adults ≥ 100 kg    | IV infusion             | 30 mg once every three weeks                       |
| Vutrisiran | Adult patients     | SC injection            | 25 mg once every three months                      |
| Inotersen  | Adult patients     | SC injection            | 284 mg once every week (on the same day each week) |

IV: Intravenous, kg: kilogram, mcg: microgram, mg: milligram, sc: subcutaneous


| Medicinal Product                                                      | Ex-Factory price       |
|------------------------------------------------------------------------|------------------------|
| Onpattro® 2mg/ml concentrate for solution for infusion (1 x 5 ml vial) | €8,074.94 <sup>^</sup> |
| Amvuttra® 25mg solution for injection (1 x 25 mg/0.5 ml PFS)           | €107,357.08*           |

Mg: milligram, ml: millilitre, PFS: pre-filled syringe


| Medicinal Product                                       | Reimbursement price <sup>^</sup> |
|---------------------------------------------------------|----------------------------------|
| Tegsedi® 284 mg solution for injection (4 x 1.5 ml PFS) | €23,641.42                       |

Mg: milligram, ml: millilitre, PFS: pre-filled syringe


Data used: HSE Medicines Management Programme Managed Access Protocols, reimbursement applications data.  
<sup>^</sup> Information correct as at March 2024. \*Information correct as at May 2024. Please refer to the HSE managed access protocol for medicines used in hereditary transthyretin amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy for more information, available at [www.hse.ie/mmp](http://www.hse.ie/mmp).



**4**  
Total number of approved prescribers



**66 years**  
Mean age of applicants



**20**  
Total number of applications by May 2024

**Medicines Management Programme**

The HSE reimburse three medicines for hereditary transthyretin amyloidosis (hATTR) in adults with stage 1 or stage 2 polyneuropathy subject to a managed access protocol (MAP). The first MAP for a medicine for the treatment of hATTR with polyneuropathy was for patisiran, introduced in October 2021. This was followed by a MAP for inotersen in October 2022. A combined MAP for all medicines used in hATTR with polyneuropathy was developed with the reimbursement of vutrisiran in May 2024. The aim of this MAP is to provide patients with hATTR with polyneuropathy with access to these high-cost treatments.

Patisiran (Onpattro®) and vutrisiran (Amvuttra®) are available under hospital pricing approval and inotersen (Tegsedi®) is available under the High Tech Arrangement.

To date, the HSE have approved four prescribers under these protocols. By May 2024, 20 applications for these medicines were submitted to the HSE Medicines Management Programme. The mean age of applicants was 66 years at date of application.

**June 2024**

**HSE Medicines Management Programme.**