

- Tafamidis is safe and effective in reducing the progress of neuropathy and in maintaining nutritional status and quality of life in Val30Met patients with stage 1 transthyretin familial amyloid polyneuropathy (TTR-FAP).
- To be most effective, treatment with tafamidis needs to be started as soon as possible once the diagnosis of TTR-FAP onset is suspected.
- The use of tafamidis in non-Val30Met TTR-FAP patients at early stage looks promising but is pending confirmation from long-term and placebo-controlled trials.
- Patients should be evaluated by multiple neurological and clinical assessments at referral centers at regular periods (every 6 months) to decide whether to continue or stop treatment, as it is now clear that different individual response profiles exist.

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