Efficacy

Diflunisal is a non-steroidal anti-inflammatory (NSAID) that has been shown to stabilise transthyretin (TTR) to stop disaggregation into the monomers that could otherwise subsequently re-aggregate into amyloid fibrils.

A Phase III placebo controlled randomised controlled trial* with 130 patients with familial TTR neuropathy showed that at a dose of 250mg po bd, 29.7% of the diflunisal group vs 9.4% of the placebo group achieved a reduced rate of neurologic progression and improved quality of life.

The side effects are the same as for other NSAIDs, namely renal impairment and gastric inflammation.

Cost and funding

Diflunisal is not Medicare rebated. The cost is determined by the importer and distributor. The cost per month is approximately $70 as of April 2019. The cost does not include pharmacy handling fees or GST.

Administrative requirements for obtaining Diflunisal

1. TGA SAS Category B approval
2. Patient consent for SAS drug prescription (standardised form)
   Hardcopy to pharmacy
3. Script
   500mg dose tablets, ½ tablet bd, 60 x 500mg dose tablets with repeats

Clinical monitoring

We suggest monitoring the creatinine every 2 weeks for the first month then monthly for 2 months.

Start proton pump inhibitor as prophylaxis or if upper gastrointestinal side effects occur.

Monitor and manage fluid retention.

References