

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Pentosan polysulfate sodium for treating bladder pain syndrome

Final scope

Remit/appraisal objective

To appraise the clinical and cost effectiveness of pentosan polysulfate sodium within its marketing authorisation for treating bladder pain syndrome.

Background

Bladder pain syndrome (also known as interstitial cystitis) is a chronic bladder condition characterised by pain, urinary urgency, frequency and nocturia. Symptoms often resemble those of patients with overactive bladder. It is often associated with negative cognitive, behavioural, sexual or emotional consequences, as well as with symptoms suggestive of lower urinary tract and sexual dysfunction. It is not clear what causes bladder pain syndrome. In some people with the condition the bladder is inflamed, ulcerated, scarred or stiff. Bladder pain syndrome that is characterised by 'Hunner's lesions' (distinctive inflammatory lesions that rupture the bladder lining) is sometimes known as 'classic interstitial cystitis'.¹ The condition can also be characterised by glomerulations (haemorrhages in the bladder wall), although these are not specific to bladder pain syndrome. These characteristics can be detected by cystoscopy with hydrodistension. In clinical practice the diagnosis of bladder pain syndrome is often made once specific causes such as infection and malignancy have been ruled out.

Bladder pain syndrome may affect approximately 400,000 people in the UK². It is more common in women than men; 90% of people with the condition are women in their fifth and sixth decades of life. Up to 50% of patients with symptoms of bladder pain syndrome will have spontaneous resolution in time.³ Estimates of the prevalence of bladder pain syndrome associated with inflammation in the bladder (for example, characterised by Hunner's lesions or glomerulations) range from 0.3 to 10.2 per 10,000 patients.

There is no NICE technology appraisal guidance for bladder pain syndrome. Treatments are generally aimed at controlling the symptoms, as there is no cure for the condition. Lifestyle changes such as avoiding certain foods and drinks, reducing stress and stopping smoking may help to reduce symptoms. Antihistamine tablets may be prescribed to reduce the inflammation, and painkillers can also be taken.^{4:5} Some medicines can be passed directly into the bladder through a catheter to relieve symptoms (bladder instillations).⁶ Before the marketing authorisation was granted, pentosan polysulfate sodium was sometimes used off-licence to treat bladder pain syndrome with glomerulations or Hunner's lesions.⁷ There are no other medicines with a marketing authorisation in the UK for treating bladder pain syndrome.

The technology

Pentosan polysulfate sodium (Elmiron, Consilient Health) has a marketing authorisation in the UK for treating 'bladder pain syndrome characterized by either glomerulations or Hunner's lesions in adults with moderate to severe pain, urgency and frequency of micturition'. Pentosan polysulfate sodium is taken orally.

Pentosan polysulfate sodium is a semi-synthetic heparin-like substance that resembles glycosaminoglycans. It is thought to work by binding to and repairing the glycosaminoglycan layer in the deficient mucous of the bladder.

Intervention	Pentosan polysulfate sodium
Population	Adults with bladder pain syndrome characterised by either glomerulations or Hunner's lesions with moderate to severe pain, urgency, and frequency of micturition
Comparators	<ul style="list-style-type: none">• Bladder instillations• For people for whom bladder instillations are inappropriate, cannot be tolerated or are unsuccessful: established clinical management without pentosan polysulfate sodium or bladder instillations (including medicines that do not currently have a marketing authorisation in the UK for this indication)
Outcomes	The outcome measures to be considered include: <ul style="list-style-type: none">• bladder pain• urinary urgency• urinary frequency• nocturia• adverse effects of treatment• health-related quality of life.
Economic analysis	<p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p> <p>The use of pentosan polysulfate sodium is conditional</p>

	on the presence of either glomerulations or Hunner's lesions. The economic modelling should include the costs associated with diagnostic testing for glomerulations or Hunner's lesions in people with bladder pain syndrome who would not otherwise have been tested. A sensitivity analysis should be provided without the cost of the diagnostic test. See section 5.9 of the Guide to the Methods of Technology Appraisals .
Other considerations	Guidance will only be issued in accordance with the marketing authorisation of pentosan polysulfate sodium. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations and NICE Pathways	<p>Related NICE evidence summary:</p> <p>Interstitial cystitis: oral pentosan polysulfate sodium (2015) NICE evidence summary ESUOM43.</p> <p>Interstitial cystitis: dimethyl sulfoxide bladder instillation (2014). NICE evidence summary ESUOM26.</p> <p>Related NICE Pathways:</p> <p>Urogenital conditions (2017) NICE pathway.</p>
Related National Policy	<p>NHS England (2018) Manual for Prescribed Specialised Services 2018/19.</p> <p>Department of Health (2016) NHS Outcomes Framework 2016-2017 Domains 2-5.</p>

References

- ¹ International Society for the Study of Bladder Pain Syndrome [Hunner Lesion](#) (accessed February 2018)
- ² The Urology Foundation [Bladder related statistics](#) (accessed May 2018)
- ³ European Public Assessment Report. [Elmiron \(2017\)](#) (accessed February 2018)
- ⁴ European Association of Urology (2015) [Chronic Pelvic Pain guidelines](#) (accessed February 2018)
- ⁵ Royal College of Obstetricians and Gynaecologists/British Society of Urogynaecology (2016) [Management of Bladder Pain Syndrome](#) Green top guideline No. 70 (accessed February 2018)
- ⁶ NHS Choices [Interstitial Cystitis](#) (accessed February 2018)

⁷ UK Medicines Information (2011) [Oral Pentosan for Painful Bladder Syndrome/Interstitial Cystitis: Unlicensed and Off-label Medicines Reports Number 2](#) (accessed February 2018)