NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Oxybutynin hydrochloride for managing neurogenic detrusor overactivity in people 6 years and over with spinal cord injury or spina bifida

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of oxybutynin hydrochloride (Vesoxx) within its marketing authorisation for managing neurogenic detrusor overactivity in people 6 years and over with spinal cord injury or spina bifida.

Background

Neurogenic detrusor overactivity (NDO) occurs when the wall muscle of the bladder is overactive, which means that it sporadically contract resulting in increased pressure within the bladder. This can lead to harm of the upper urinary tract, urinary urgency and incontinence. NDO can result from inherited (congenital) conditions, such as spina bifida, or can occur after disease or injury of the spinal cord.

Symptoms of neurogenic lower urinary tract dysfunction may relate to impaired urine storage and/or bladder emptying difficulties. Symptoms of impaired storage include increased frequency of urination and urinary incontinence. Urinary tract symptoms have a significant impact on quality of life, for example, they can cause embarrassment, lead to social isolation and impair activities of daily living.

It is estimated that there are 50,000 people in the UK with spinal cord injury, out of which approximately 22,400 have NDO.[1,2] The incidence of spina bifida is 0.06% of live births.[3] It estimated that over 95% of people with spina bifida also experience neurogenic bladder issues but the data might be insufficient.[1,4]

For people with neurogenic urinary tract dysfunction, CG148 recommends antimuscarinic drugs (such as oxybutynin hydrochloride, trospium chloride or propiverine) for people with spinal cord disease and symptoms of an overactive bladder such as frequency, urgency and incontinence. CG148 recommends bladder wall injection with botulinum toxin type A in children, young people and adults with spinal cord disease and symptoms of overactive bladder or impaired bladder storage where antimuscarinic drugs have proved to be ineffective or poorly tolerated.

The technology

Intravesical oxybutynin hydrochloride (Vesoxx, Farco-Pharma) has a marketing authorisation for the suppression of detrusor overactivity due to spinal cord injury or myelomeningocele (spina bifida) in children from 6 years of age and adults, who are managing bladder emptying by clean intermittent catheterisation, not adequately managed with oral anticholinergics.

Intervention(s)	Intravesical oxybutynin hydrochloride
Population(s)	Children from 6 years of age and adults, who are managing bladder emptying by clean intermittent catheterisation, not adequately managed with oral anticholinergics.
Comparators	People with spinal cord disease, symptoms of an overactive bladder and for whom antimuscarinic drugs have proved to be ineffective or poorly tolerated • Botulinum toxin type A Adults with symptoms of overactive bladder • Mirabegron
Outcomes	The outcome measures to be considered include: • Symptoms of urgency • Urinary frequency • Incontinence episodes • adverse effects of treatment • health-related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. 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. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account. The availability and cost of biosimilar and generic products should be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. 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	**Related Technology Appraisals:**

	'Mirabegron for treating symptoms of overactive bladder' (2013). NICE Technology appraisal guidance TA290. Review date TBC. **Related Guidelines:** 'Urinary incontinence in neurological disease: assessment and management' (2012). NICE guideline CG148. Review date TBC.
Related National Policy	The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018/2019), Chapters 58 and 136, NHS manual for prescribed specialist services (2018/2019)

Questions for consultation

Where do you consider intravesical oxybutynin hydrochloride will fit into the existing care pathway for neurogenic detrusor overactivity?

Have all relevant comparators for intravesical oxybutynin hydrochloride been included in the scope?

Which treatments are considered to be established clinical practice in the NHS for people with spinal cord disease, symptoms of an overactive bladder and whose symptoms are not adequately managed by oral anticholinergics?

Are the outcomes listed appropriate?

Are there any subgroups of people in whom intravesical oxybutynin hydrochloride is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Would intravesical oxybutynin hydrochloride be a candidate for managed access?

Do you consider intravesical oxybutynin hydrochloride to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?

Do you consider that the use of intravesical oxybutynin hydrochloride can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Would you consider intravesical oxybutynin hydrochloride significantly more beneficial than oral oxybutynin hydrochloride?

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which intravesical oxybutynin hydrochloride will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. We welcome comments on the appropriateness of appraising this topic through this process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

NICE's health technology evaluations: the manual states the methods to be used where a cost comparison case is made.

- Would it be appropriate to use the cost-comparison methodology for this topic?

- Is the new technology likely to be similar in its clinical efficacy and resource use to any of the comparators?

- Is the primary outcome that was measured in the trial or used to drive the model for the comparator(s) still clinically relevant?

- Is there any substantial new evidence for the comparator technology/ies that has not been considered? Are there any important ongoing trials reporting in the next year?

References

1 Cameron AP. Medical management of neurogenic bladder with oral therapy. *Transl Androl Urol* 2016; **5**: 51–62.

2 The Back-Up Trust. What is spinal cord injury? https://www.backuptrust.org.uk/. 2022. https://www.backuptrust.org.uk/spinal-cord-injury/what-is-spinal-cord-injury (accessed May 25, 2022).

3 England PH. Spina bifida: information for parents. Promot. Mater. 2020. https://www.gov.uk/government/publications/spina-bifida-information-for-parents/spina-bifida-information-for-parents (accessed May 25, 2022).

4 Ruffion A, Castro-Diaz D, Patel H, *et al.* Systematic review of the epidemiology of urinary incontinence and detrusor overactivity among patients with neurogenic overactive bladder. *Neuroepidemiology* 2013; **41**: 146–55.

Appendix B