NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Appraisal

Prolonged-release potassium bicarbonate and potassium citrate for treating distal renal tubular acidosis

Final scope

Scope remit/appraisal objective

To appraise the clinical and cost effectiveness of prolonged-release potassium bicarbonate and potassium citrate within its marketing authorisation for treating distal renal tubular acidosis.

Background

Distal renal tubular acidosis (DRTA) is a disorder of impaired acid removal from the blood. The kidneys filter acids from the blood and remove them from the body in urine, which prevents the build-up of acids in the blood. DRTA occurs when the kidneys do not properly remove acids from the blood into the urine, which causes a person's blood to become too acidic. DRTA may be hereditary (primary) caused by mutations in genes or acquired (secondary) due to other conditions like Sjögren syndrome, sickle cell anaemia, systemic lupus erythematosus, chronic obstructive uropathy, or post-renal transplantation.[1]

DRTA is a rare condition with a prevalence in the UK of between 0.46 to 1.6 per 10,000 people.[2] It is estimated that between 2,589 and 9,005 people are currently living with the condition in England. The incidence of the disease seems to be unknown.

Primary DRTA is a highly variable disorder and can affect people differently. Some people have slightly elevated acid levels and no accompanying symptoms while others can experience kidney stones, deafness, growth failure or rickets (bowing of the bones).[1]

There is no NICE guidance on the treatment of DRTA. Standard management includes the use of off-label alkalinizing replacement therapy to correct metabolic acidosis and to maintain serum potassium levels in the normal range. The dose and exact treatment is dependent on the form of DRTA.

The technology

Potassium citrate and potassium bicarbonate (Sibnayal, Advicenne) is a fixed-dose prolonged-release formulation, designed to maintain sustained release over a twelve-hour period. It is expected to both neutralise excess acid and restore levels of potassium in the blood. It is administered orally.

Prolonged-release potassium citrate and potassium bicarbonate does not have marketing authorisation in the UK for DRTA. The Committee for Medicinal Products for Human Use (CHMP) has issued a positive opinion recommending the granting of a marketing authorisation for the treatment of DRTA.

Intervention(s)	Prolonged-release potassium citrate and potassium bicarbonate (Sibnayal)
Population(s)	People with distal renal tubular acidosis aged 1 year and older
Comparators	Established clinical management without prolonged-release potassium citrate and potassium bicarbonate (Sibnayal), which may include alkalinising treatments alone or in combination with one another
Outcomes	The outcome measures to be considered include: • Bicarbonate level in the blood • Potassium level in the blood • Calcium level in the urine • Citrate level in the urine • Renal function • Measures of impaired growth • Bone mineral density • 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. Any commercial arrangements will 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 and NICE Pathways	None
Related National Policy	NHS England (2019) The NHS long term plan

	NHS England (2018) NHS England Funding and Resource 2018/19: Supporting 'Next Steps for the NHS Five Year Forward View' NHS England (2017) Next steps on the five year forward view NHS England (2014) NHS Five year forward view **National Service Frameworks** Renal Services - archived **Other policies** Department of health (2016) NHS outcomes framework 2016 to 2017

References

1. National Institute of Diabetes and Digestive and Kidney Diseases (2008) Renal tubular acidosis (pdf version available on NIDDK website). Accessed 30/03/2021

2. Advicenne (2019) Advicenne confirms prevalence of distal renal tubular acidosis (dRTA) and cystinuria at ISPOR conference. Accessed 30/03/2021