HTA - - Health Technology Assessment Report

Denufosol for cystic fibrosis with mild lung disease

Code: HTA-32010000502 **Year:** 2009 **Date:** 05 May 2010 **Author:** National Horizon Scanning Centre

Study design (if review, criteria of inclusion for studies)

1) TIGER-1; NCT00357279; 08-108; denufosol vs placebo; phase III. Completed and published in abstract; 2) NCT00056147; 08-103; denufosol vs placebo; phase II. Complete and published; 3) TIGER-2; NCT00625612; 08-110; CF; denufosol vs placebo; phase III. Trials registry; 4) Extension of TIGER-2; NCT00846781; 08-114; denufosol; phase III. Trials registry.

List of included studies (4)

1) TIGER-1; NCT00357279; 2) NCT00056147; 3) TIGER-2; NCT00625612; 4) Extension of TIGER-2; NCT00846781

Participants

1) n=352; â‰¥5 years old; CF; FEV1â‰¥75% predicted; stable with no acute pulmonary exacerbations for at least 4 weeks. 2) n=89; 8-50 years old; CF; FEV1 â‰¥75% predicted; oxyhaemoglobin saturation â‰¥90%; clinically stable. 3) n=450; â‰¥5 years old; CF; FEV1 â‰¥75% â‰¤110% predicted; reproducible spirometry; stable with no acute pulmonary exacerbations for at least 4 weeks. 4) n=380; completed TIGER-2 study.

Interventions

1) Randomised to nebulised denufosol 60mg or placebo three times daily for 24 weeks. All enter 24 week open-label denufosol extension. 2) Randomised to nebulised denufosol 20, 40 or 60mg or placebo three times daily for 28 days. 3) Randomised to nebulised denufosol 60mg or placebo three times daily for 12 months. 4) All participants given nebulised denufosol 60mg three times daily for other 12 months.

Outcome measures

1) primary outcome: Lung function; secondary outcomes: Pulmonary exacerbation requirement for concomitant CF medications, quality of life (QoL). 2) primary outcome: Lung function (FEV1, FVCb, FEV1/FVC and FEF25%-75%); secondary outcomes: Respiratory symptoms, sputum weight, pulmonary exacerbations, high-resolution computed tomography (hyperinflation, mucus plugging, peribronchial thickening, bronchiectasis, ground glass, opacity and cysts/bullae. 3) primary outcome: Lung function; secondary outcomes: Pulmonary exacerbation, antibiotic use, incidence of hospitalisation or emergency room visits, health resource utilisation, QoL. 4) primary outcome: Lung function; secondary outcomes: Pulmonary exacerbation, antibiotic use, incidence of hospitalisation or emergency room visits.

Main results

1) At week 24: adjusted mean change in FEV1 of 48ml vs 3ml (p=0.047) for denufosol and placebo respectively. Difference compared to placebo in mean change in FEV1 of 41ml (p=0.101) in patients with no exacerbation vs 101 ml with exacerbation (p=0.062). Most common AEs for denufosol and placebo respectively: Cough: 55% vs 59%, nasal congestion 15% vs 19%, pharyngolaryngeal pain 19% vs 18%, rhinorrhea 10% (p

http://www.nhsc-healthhorizons.org.uk/topics/denufosol-ins37217-respiratory-for-cystic-fibrosis/

See also

Birmingham: National Horizon Scanning Centre (NHSC) YR: 2009

Keywords

Other drugs; denufosol; Inhalation OR nebulised; pharmacological_intervention;