NHSEED - - Economic Study or Review

Efficacy and safety of elexacaftor plus tezacaftor plus ivacaftor versus tezacaftor plus ivacaftor in people with cystic fibrosis homozygous for F508del-CFTR: a 24-week, multicentre, randomised, double-blind, active-controlled, phase 3b trial.

Code: PM34942085 **Year:** 2021 **Date:** 2013 **Author:** Sutharsan S

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

State transition model based on transitions between three strata of lung function. The model structure was informed by systematic reviews of evidence concerning the plausibility of potential relationships between intermediate endpoints and final outcomes. The model assumes that treatment impacts on FEV1 trajectory, which manifest as changes in health-related quality of life. No survival benefit is assumed due to the absence of robust quantifiable evidence. Model parameters were informed by patient-level and aggregate data from two randomised controlled trials together with the best available

Participants

Chronic P. aeruginosa lung infection in patients with CF

Interventions

(i) colistimethate sodium dry powder for inhalation (DPI) and (ii) tobramycin DPI versus nebulised tobramycin

Outcome measures

Cost-effectiveness from the perspective of the National Health Service (NHS) and Personal Social Services (PSS). Forced expiratory volume in 1 second (FEV1) % predicted. Additional health states representing post-lung transplantation and dead are also modelled. Resource use and costs associated with drug acquisition, the management of exacerbations and reduced nebuliser maintenance were drawn from reference sources and expert opinion. Costs were valued at 2011/2012 prices. Costs and health outcomes were discounted at a rate of 3.5 %. Simple and probabilistic sensitivity analyses were undertaken, including additional analyses of Patient Access Scheme (PAS) price discounts offered by the manufacturers of both DPI products.

Main results

Colistimethate sodium DPI is expected to produce fewer quality-adjusted life-years (QALYs) than nebulised tobramycin. Based on its list price, colistimethate sodium DPI is expected to be dominated by nebulised tobramycin. When the PAS is incorporated, the incremental cost-effectiveness ratio (ICER) for colistimethate sodium DPI versus nebulised tobramycin is expected to be approximately £288,600 saved per QALY lost. Based on its current list price, the ICER for tobramycin DPI versus nebulised tobramycin is expected to be approximately £124,000 per QALY gained. When the proposed PAS is included, tobramycin DPI is expected to dominate nebulised tobramycin.

Authors' conclusions

Under their list prices, neither DPI product is likely to represent good value for money for the NHS given current cost-effectiveness thresholds. The PAS discounts have a significant impact upon the economic attractiveness of both DPI products compared against nebulised tobramycin. The clinical effectiveness and cost effectiveness of the DPIs against other nebulised antibiotics, such as aztreonam and inhaled colistimethate sodium, remains unclear.

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See also

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Keywords

Anti-Bacterial Agents; Bacterial Infections; colistimethate; Colistin; Infection; Inhalation OR nebulised; nebuliser; non pharmacological intervention - devices OR physiotherapy; pharmacological_intervention; Powders; Pseudomonas aeruginosa; Pseudomonas; Respiratory Tract Diseases; Respiratory Tract Infections; other anti-bacterial agents; Tobramycin; Aminoglycosides;