

Cochrane Database of Systematic Reviews - - Cochrane Review

Lumacaftor/ivacaftor - assessment according to §35a (para. 1, sentence 10) Social Code Book V (Structured abstract)

Code: HTA-32016000718 **Year:** 2016 **Date:** 2015 - updated: 1 NOV 2019

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

Randomised and quasi-randomised controlled trials in people with cystic fibrosis who have breathlessness. Studies comparing any drugs used for easing breathlessness to another drug administered by any route (inhaled (nebulised), intravenous, oral, subcutaneous, transmucosal (including buccal, sublingual and intra-nasal) and transdermal).

Participants

Terminally-ill people with CF, of either sex and of any age group with breathlessness.

Interventions

1. inhaled (nebulised) drugs; 2. intravenous (IV) drugs; 3. oral drugs; 4. subcutaneous drugs; 5. transmucosal drugs including buccal, sublingual and intranasal; and 6. transdermal drugs. Comparisons of two different doses of one drug will be excluded. Inhaled drugs with drugs given systemically will be compared. However, there are likely to be many ways of giving a drug systemically e.g. oral, IV, buccal, transdermal. Within these two main groupings (inhaled versus systemic), individual classes of drugs will be compared both between groups (e.g. opiates versus benzodiazepines) and within groups (e.g. morphine versus fentanyl - both being opiates).

Outcome measures

Primary outcomes 1. Breathlessness (dyspnoea) score (for severity of breathlessness) as measured by a validated scoring system, e.g. modified Borg score, a visual analogue scale (VAS), numerical rating scales (NRS), the Chronic Respiratory Disease Questionnaire (CRQ), Medical Research Council Scale (MRC) 2. Frequency of episodes of breathlessness 3. Duration of period free from feeling breathless. Secondary outcomes 1. Quality of life (QoL) 2. Need for change of therapy 3. Hospitalization 4. Adverse events 5. Blood oxygen saturation (SpO₂) levels 6. Increased tolerance to activity (daily activities).

Main results

The new searches in 2020 yielded two ongoing studies that were not relevant to the review question. Previous searches had found only one study (cross-over in design), which did not fulfil the inclusion criteria as no data were available from the first treatment period alone.

Authors' conclusions

Due to the lack of available evidence, this review cannot provide any information for clinical practice. The authors call for specific research in this area after taking into account relevant ethical considerations. The research should focus on the efficacy and safety of the drugs with efficacy being measured in terms of improvement in quality of life, dyspnoea scores and hospital stay.

<http://onlinelibrary.wiley.com/doi/10.1002/hta.10007>

See also

Health Technology Assessment Database

Keywords

Adult; Aged; pharmacological_intervention; Respiratory Tract Diseases; Lung Transplantation; Respiratory Insufficiency; transplantation;