

HTA - - Health Technology Assessment Report

# Lumacaftor and ivacaftor combination therapy for cystic fibrosis ? first line (Structured abstract)

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

Review, HTA report

## List of included studies (3)

TRANSPORT, NCT01807949, VX12-809-104; lumacaftor and ivacaftor vs placebo; phase III. TRAFFIC, NCT01807923, VX12-809-103; lumacaftor and ivacaftor vs placebo; phase III. NCT01931839, VX12-809-105; phase III extension.

## **Participants**

patients aged 12 years and older with cystic fibrosis (CF) who are homozygous for the F508del-CFTR mutation.

#### Interventions

Lumacaftor is a cystic fibrosis transmembrane conductance regulator (CFTR) corrector and ivacafor is a CTFR potentiator.

#### **Outcome measures**

Primary: Relative change in percent predicted forced expiratory volume in 1 second (FEV1); safety. Secondary: Absolute change in percent predicted FEV1, change in body mass index (BMI), number of pulmonary exacerbations, cystic fibrosis questionnaire (CFQ-R) score, safety.

http://www.hsc.nihr.ac.uk/topics/lumacaftor-and-ivacaftor-combination-therapy-for-c/

#### See also

Health Technology Assessment Database

## Keywords

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological\_intervention; placebo; VX-770; VX-809; ivacaftor; Aminophenols; lumacaftor;