

primary studies - published RCT

## Effects of probiotics on quality of life in children with cystic fibrosis; a randomized controlled trial.

Code: PM24910746

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

RCT

### Participants

Patients heterozygous for F508del-CFTR, aged 18 years or older with a confirmed CF diagnosis and percent predicted FEV1 (ppFEV1) of 40 to 90

### Interventions

Patients were randomized to lumacaftor/ivacaftor (400 mg/250 mg every 12 h) or placebo daily for 56 days.

### Outcome measures

Primary outcomes were change in ppFEV1 at Day 56 and safety.

### Main results

Of 126 patients, 119 (94.4%) completed the study. Lumacaftor/ivacaftor was well tolerated, although chest tightness and dyspnea occurred more frequently with active treatment than with placebo (27.4% vs. 14.3% and 14.5% vs. 6.3%, respectively). Mean (SD) ppFEV1 values at baseline were 62.9 (14.3) in the active treatment group and 60.1 (14.0) in the placebo group. Absolute changes in ppFEV1 (least squares mean [SE]) at Day 56 were -0.6 (0.8) percentage points in the active treatment group and -1.2 (0.8) percentage points in the placebo group (P = 0.60). CF respiratory symptom scores in the active treatment group improved by a mean of 5.7 points versus a decrease of -0.8 in the placebo group (P

### Authors' conclusions

Sweat chloride and respiratory symptom scores improved with lumacaftor/ivacaftor, though no meaningful benefit was seen in ppFEV1 or body mass index in patients heterozygous for F508del-CFTR.

### See also

Iran J Pediatr. 2013 Dec;23(6):669-74.

### Keywords

Child; Adult; Adolescent; Aminophenols; CFTR Modulators; Genetic Predisposition to Disease; Orkambi; pharmacological\_intervention; VX-770; ivacaftor; lumacaftor; VX-809;