Lumacaftor, ivacaftor linked to improved lung function in CF

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(HealthDay)—For patients aged 6 to 11 years with cystic fibrosis

homozygous for *F508del-cystic fibrosis transmembrane conductance regulator* (*CFTR*), lumacaftor and ivacaftor treatment is associated with significant improvement in lung function, according to a study published online June 9 in *The Lancet Respiratory Medicine*.

Felix Ratjen, M.D., from the University of Toronto, and colleagues conducted a phase 3 randomized multicenter study involving patients enrolled in nine countries to examine the efficacy and safety of lumacaftor and ivacaftor in patients age 6 to 11 years with cystic fibrosis homozygous for *F508del-CFTR*. Patients were randomized to receive lumacaftor and ivacaftor every 12 hours (103 patients) or placebo (101 patients) for 24 weeks.

The researchers found that the average absolute change in lung clearance index$_{2-5}$ from baseline over all study visits least squares mean difference was −1.09 units for lumacaftor and ivacaftor versus placebo (95 percent confidence interval [CI], −1.43 to −0.75; P placebo in absolute change in percent predicted forced expiratory volume in one second was 2.4 (95 percent CI, 0.4 to 4.4; P = 0.0182). Ninety-six percent of the patients reported adverse events, which were mainly mild or moderate (43 and 48 percent, respectively).

"Treatment with lumacaftor and ivacaftor was associated with statistically significant improvements in lung function," the authors write.

Several authors disclosed financial ties to pharmaceutical companies, including Vertex Pharmaceuticals, which manufactures lumacaftor-ivacaftor and funded the study.

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