Tezacaftor-ivacaftor, ivacaftor alone effective in CF

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(HealthDay)—Tezacaftor-ivacaftor or ivacaftor alone is effective for

patients with cystic fibrosis who are heterozygous for the Phe508del deletion, and tezacaftor-ivacaftor is effective for patients who are homozygous for the *CFTR* Phe508del mutation, according to two studies published online Nov. 3 in the *New England Journal of Medicine* to coincide with presentation at the North American Cystic Fibrosis Conference, being held Nov. 2 to 4 in Indianapolis.

Steven M. Rowe, M.D., M.S.P.H., from the University of Alabama at Birmingham, and colleagues conducted a randomized trial involving 248 patients aged 12 years or older with cystic fibrosis who were heterozygous for the Phe508del mutation and a *CFTR* mutation associated with residual CFTR function. Patients were randomized to tezacaftor-ivacaftor, ivacaftor monotherapy, or placebo. The researchers found that the least-squares mean difference with respect to the absolute change in the percentage of predicted forced expiratory volume in one second (FEV_1) from baseline was 6.8 and 4.7 percentage points for tezacaftor-ivacaftor and ivacaftor alone, respectively, versus placebo.

Jennifer L. Taylor-Cousar, M.D., from National Jewish Health in Denver, and colleagues conducted a trial involving 475 patients aged 12 years or older with cystic fibrosis who were homozygous for the *CFTR* Phe508del mutation. Participants were randomized to receive tezacaftor and ivacaftor or placebo for 24 weeks. The researchers found that the effects on the absolute and relative changes in the percentage of the predicted FEV_1 were 4 and 6.8 percent, respectively, in favor of tezacaftor-ivacaftor over placebo.

"The combination of tezacaftor and ivacaftor was efficacious and safe in patients 12 years of age or older who had cystic fibrosis and were homozygous for the *CFTR* Phe508del mutation," Taylor-Cousar and colleagues write.

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More information: Abstract/Full Text—Rowe
Abstract/Full Text—Taylor-Cousar
Editorial
More Information