Tx response no different for migalastat, placebo in fabry's

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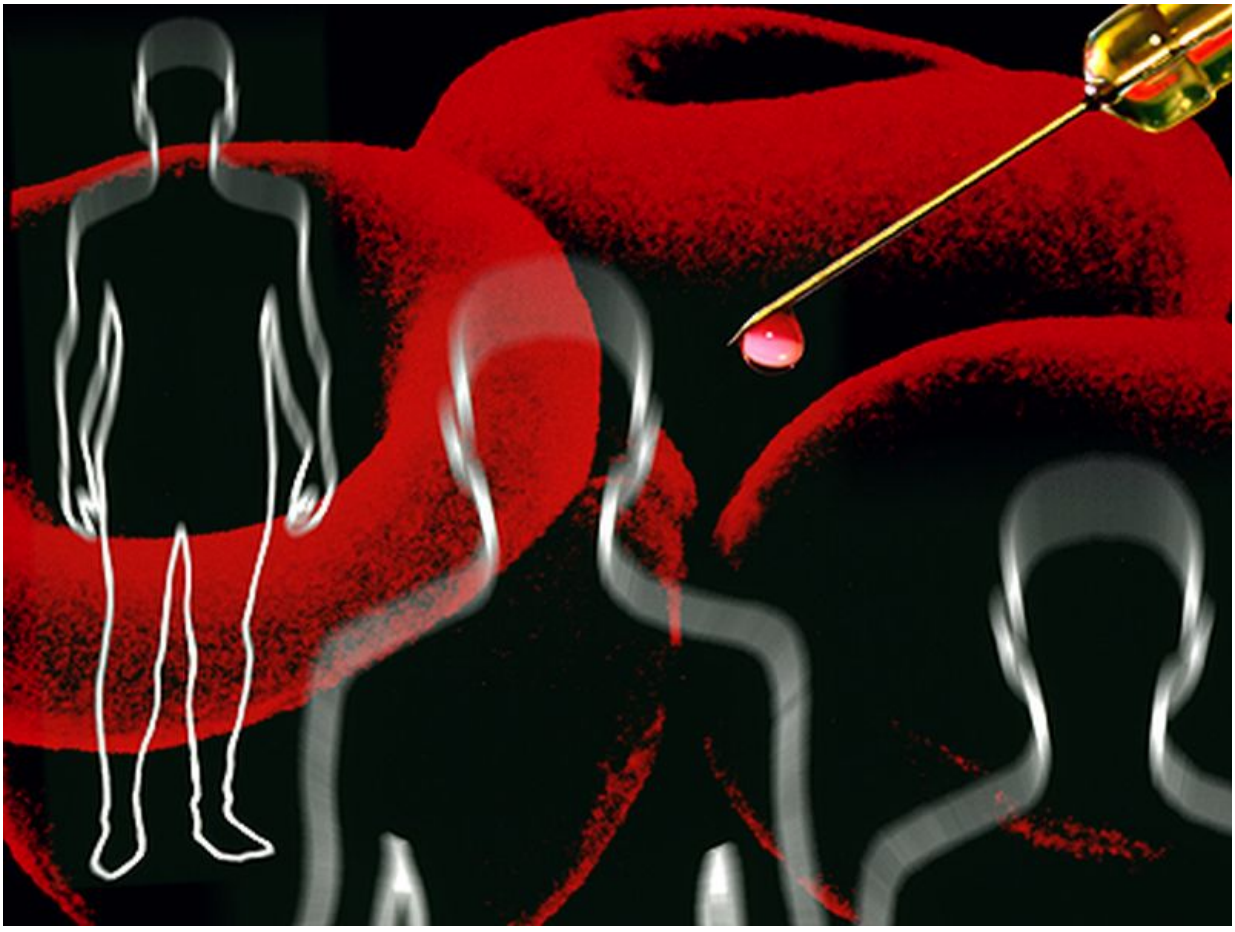

(HealthDay)—For patients with Fabry's disease, the percentage of

patients with response at six months does not differ for those treated with the oral pharmacologic chaperone migalastat or with placebo, according to a study published in the Aug. 11 issue of the *New England Journal of Medicine*.

Dominique P. Germain, M.D., Ph.D., from the University of Versailles in France, and colleagues used an initial assay of mutant α-galactosidase forms to categorize 67 patients with Fabry's disease for randomization to six months of double-blind migalastat or placebo (stage 1), followed by open-label migalastat from six to 12 months (stage 2) plus an additional year. Before unblinding, 50 of 67 participants were found to have mutant α-galactosidase forms suitable for migalastat targeting.

The researchers observed no significant treatment effect in the primary end point (percentage of patients with a response at six months) in analysis involving patients with mutant α-galactosidase forms that were suitable or not suitable for migalastat therapy (41 percent of those who received migalastat and 28 percent of those who received placebo had a response at six months; P = 0.30).

"Among all randomly assigned patients (with mutant α-galactosidase forms that were suitable or not suitable for migalastat therapy), the percentage of patients who had a response at six months did not differ significantly between the migalastat group and the placebo group," the authors write.

The study was funded by Amicus Therapeutics, which is developing migalastat.

More information: Full Text (subscription or payment may be required)