# Trials supporting FDA approval of breakthrough drugs examined

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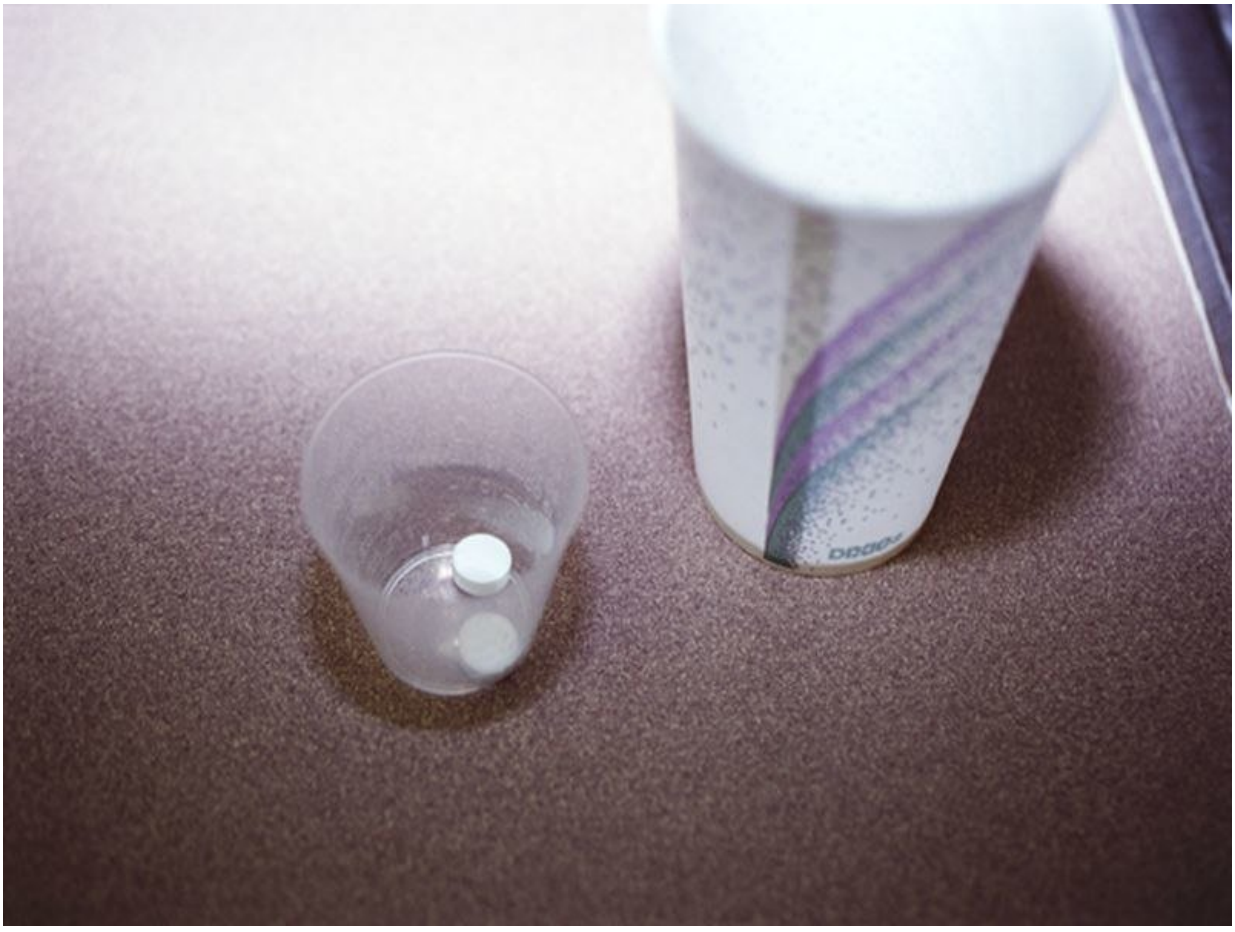

(HealthDay)—Pivotal trials supporting U.S. Food and Drug

Administration approvals granted Breakthrough Therapy designation often lack randomization, double-blinding, and control groups, according to a research letter published in the July 17 issue of the *Journal of the American Medical Association*.

Jeremy Puthumana, from the Yale University School of Medicine in New Haven. Conn., and colleagues abstracted FDA-determined regulatory and therapeutic characteristics and post-marketing requirements for all new drugs and biologics granted Breakthrough Therapy designation.

The researchers found that the FDA approved 46 therapeutics with Breakthrough Therapy designation on the basis of 89 pivotal trials from 2012 through 2017. Therapeutics were often designated as orphan products (65.2 percent) and qualified for FastTrack review and Accelerated Approval (52.2 and 39.1 percent, respectively). Per indication approval, the median number of pivotal trials was one, with a median of 222 patients enrolled among all pivotal trails supporting an indication approval. Among these approvals, 58.7, 45.7, 54.3, and 21.7 percent were made on the basis of pivotal trials using randomization, double-blind allocation, an active or placebo comparator, and a clinical primary end point, respectively. Pivotal trials that were used to support breakthrough approvals with Accelerated Approval status were less likely to be randomized, double-blinded, and include a control group than those without Accelerated Approval.

"FDA-required post-marketing studies will be critical to confirm the clinical benefit and safety of these promising, newly approved therapies," the authors write.

One author disclosed financial ties to the pharmaceutical industry.

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