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Cost of Clinical Trials For New Drug FDA Approval Are Fraction of Total Tab

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Clinical trials to obtain FDA approval typically account for small proportion of total drug research and development costs, study suggests.

Clinical trials that support FDA approvals of new drugs have a median cost of \$19 million, according to a new study by a team including researchers from Johns Hopkins Bloomberg School of Public Health.

The study, published Sept. 24 in *JAMA Internal Medicine*, is the most comprehensive analysis of key drug trial costs to date, and suggests that these costs contribute only modestly to the overall costs of developing new drugs.

The \$19 million median figure represents less than one percent of the average total cost of developing a new drug, which in recent years has been [estimated](#)  at between \$2 to \$3 billion.

“The cost of generating this fundamental scientific information is surprisingly low given the total cost of drug development and the high price tags on many drugs,” says study senior author G. Caleb Alexander, MD, MS, associate professor of epidemiology and medicine at the Bloomberg School.

Pharmaceutical companies in the U.S. routinely explain the high prices they set for drugs by citing the high costs of drug development, including the costs of the “pivotal” clinical trials that are the basis for FDA approvals of new drugs. However, few studies have objectively analyzed these costs.

These pivotal trials are often large “Phase 3” trials, but for some indications, such as rare incurable diseases, the FDA will accept much smaller, shorter trials.

To get a better picture of pivotal trial costs, Alexander and colleagues, including first author Thomas Moore, AB, of the Milken Institute of Public Health at George Washington University, examined the key clinical trials that led to FDA approvals of new therapeutic drugs during 2015 to 2016. The researchers examined 138 trials, covering 59 different drugs—some of which were tested in multiple trials for multiple disorders. For each trial, the team calculated the likely trial cost with standard cost-estimation software used by contract research organizations and others in the pharmaceutical industry.

The researchers found that the pivotal trials from 2015 to 2016 had an estimated median cost of \$19 million. The trials’ estimated costs clustered relatively closely around this figure—half were between \$12 million and \$33 million. The lowest estimated trial cost was \$2 million for a four-patient trial of a treatment for a rare metabolic disorder; the highest, \$347 million, was for a large study of a heart-failure drug.

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The scientists also looked at how trial costs varied depending on the type of clinical trial. Trials that test a drug’s ability to prevent a clinically meaningful outcome, such as a heart attack, tended to have a much higher median cost (\$65 million) than trials using a surrogate outcome such as high serum cholesterol (median \$24 million). Costs were also higher for trials comparing a new drug to an existing standard rather than a placebo. “You generally need fewer people to demonstrate that a drug is better than a sugar pill than you do to show it works better than the current standard of care,” says Alexander.

Costs tended to be higher when a trial lasted longer or enrolled more patients. For example, the trials with fewer than 100 patients had an average cost of just \$6 million, while the trials with more than 1,000 patients had an average cost of \$77 million. Trial costs also varied across treatment areas, reflecting the varying difficulty of measuring and demonstrating efficacy from one disorder to another. Pivotal cardiovascular drug trials, for example, had a mean cost of \$157 million, versus just \$21 million for pivotal trials in endocrine and metabolic disease patients.

Alexander and colleagues now plan further studies of the characteristics of drug trials and how they affect costs. “We now have a foundation for a much broader set of inquiries into what it would cost to build better trials—trials that are more informative about the outcomes of greatest interest to patients and their providers,” Alexander says.

“Estimated Costs of Pivotal Trials for Novel Therapeutic Agents Approved by the US Food and Drug Administration, 2015-2016,” was written by Thomas Moore, Hanzhe Zhang, Gerard Anderson, and G. Caleb Alexander.

Support was provided by The Laura and John Arnold Foundation and by The Johns Hopkins Center of Excellence in Regulatory Science and Innovation, a collaborative research initiative with the FDA.

Alexander serves as Chair of the US Food and Drug Administration’s Peripheral and Central Nervous System Advisory Committee; is a paid advisor to IQVIA; serves on the advisory board of MesaRx Innovations, is a member of OptumRx’s National P&T Committee, and is a founder and principal holding equity in Monument Analytics, a consultancy that provides services to the life sciences industry and to plaintiffs in opioid litigation. These arrangements have been reviewed and approved by Johns Hopkins University in accordance with its conflict of interest policies.

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