Many new drugs did not have comparative effectiveness information available at time of FDA approval

May 3 2011

Only about half of new drugs approved in the last decade had comparative effectiveness data available at the time of their approval by the U.S. Food and Drug Administration, and approximately two-thirds of new drugs had this information available when alternative treatment options existed, according to a study in the May 4 issue of *JAMA*.

In 2009, Congress allocated $1.1 billion to comparative effectiveness research. According to the Institute of Medicine, such research is defined as the "generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care." Comparative effectiveness information on drugs is most useful to decision-makers shortly after marketing approval, when observational data from routine care and data from large head-to-head trials comparing multiple treatments are not yet available. "Comparative effectiveness is taking on an increasingly important role in U.S. health care, yet little is known about the availability of comparative efficacy data for drugs at the time of their approval in the United States," according to background information in the study.

Nikolas H. Goldberg, and colleagues from Brigham and Women's Hospital and Harvard Medical School, Boston, conducted a study to determine the proportion of recently approved drugs that had comparative efficacy data available at the time of market authorization

in the United States and to examine trends in availability of this information over time and by therapeutic indication. Data for the study were derived from approval packages publicly available through the online database of drug products (new molecular entities [NMEs]) approved by the U.S. Food and Drug Administration (FDA) between 2000 and 2010. The researchers analyzed whether eligible efficacy studies were head-to-head active controlled trials and whether the results of such studies were available in the approval packages.

The authors identified 197 eligible approved NMEs between 2000 and 2010, of which 100 (51 percent) had comparative efficacy data available at the time of market authorization. After excluding orphan products (n = 37; products or drugs that may be useful for common or rare diseases but which are not considered commercially viable) and other NMEs approved for indications for which no alternative treatments existed (n= 17), the proportion with available comparative efficacy data increased to 70 percent. On a yearly basis, the proportion of NMEs with comparative efficacy data (excluding orphan drugs and those for which no alternative treatment existed) varied between 50 percent in 2008 and 92 percent in 2010.

The researchers found that availability of comparative efficacy data was more common for some therapeutic indications, including diabetes mellitus (89 percent) and infectious diseases (73 percent), than others, such as hormones and contraceptives (33 percent), and cancer (35 percent). After excluding orphan drugs and products approved for indications for which no alternative treatments existed, the proportions by therapeutic indications were more similar. The authors also found that compared with those drugs that received standard review designations, NMEs that received priority review designations were much less likely to have comparative efficacy data.

The researchers note that although comparative efficacy data meeting

their minimal criteria were available for approximately half of all newly approved NMEs, they did not assess the extent to which the publicly available data are informative enough to provide a basis for prescribing and coverage decisions.

"In conclusion, we found that publicly available documents include results of at least 1 head-to-head trial with an approved alternative for approximately half of all newly approved NMEs. Strategies are needed to enhance the accessibility of, and ultimately the use of, this information, particularly in the early marketing experience, when comparative effectiveness data from other sources are scarce or nonexistent."

More information: *JAMA*. 2011;305[17]1786-1789.