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The BMJ Press Release

Experts raise concerns over FDA's increasing use of expedited development and approval pathways

New data have important implications for patient care

Two studies carried out by US researchers and published by **The BMJ** today raise questions about whether most new drugs are any more effective than existing products or whether they have been adequately assessed before approval.

They describe how the US Food and Drug Administration (FDA) is increasingly using its expedited development and approval pathways for what it deems to be important new drugs and assess the level of evidence used to support supplemental approvals for existing drugs.

Yet they also point out that Congress is poised to advocate for still faster reviews based on even less evidence.

Before a new prescription drug can be sold widely in the United States, it must be approved by the US Food and Drug Administration. The FDA also offers four special programs to expedite the development and approval of promising new drugs for treating serious or life threatening conditions.

These programs were designed as exceptions to the standard approval process and are supposed to be strictly limited to drugs meeting unmet medical needs or providing noticeable clinical advances.

But they are controversial because, while they have enabled approval of numerous beneficial drugs, they also rely on early stage trials, which produce less robust data.

So a team of researchers at Brigham and Women's Hospital and Harvard Medical School decided to evaluate the use of special expedited development and review pathways for new drugs at the FDA between 1987 and 2014.

They show that over the past 20 years, there has been a statistically significant increase of 2.6% per year in the number of drugs qualifying for the FDA's expedited drug development and approval programs.

They also point out that "this trend is being driven by drugs that are not first in class and thus potentially less innovative."

By the end of the study period, they found that "a majority of newly approved drugs were associated with at least one of these special programs, meaning that the exceptions had become more common than the rule."

In a second study, they show wide variations in the quality of evidence underpinning FDA approval of supplemental indications (uses beyond a drug's original indications) between 2005 and 2014. This was especially the case among supplements that expanded the drugs' approved patient populations, the results show.

They say their findings "have important implications for patient care" and "underscore the need for a robust system of post-approval drug monitoring for efficacy and safety, timely confirmatory studies, and re-examination of existing legislative incentives to promote the optimal delivery of evidence based medicine."

In an accompanying editorial, two experts from the US and Canada say these studies "give cause for concern about whether most new drugs are any more effective than existing

products or whether their safety has been adequately assessed.”

Do patients and doctors really want medicines for cancer and other life threatening conditions approved this way - quickly, with marginal evidence of real benefit, they ask?

Do they know that faster reviews are associated with a significant increase in serious safety problems and the risk of patients being admitted to hospital with or dying from adverse reactions?

“Effectively, the FDA has been granting most supplemental approvals without evidence of meaningful clinical benefit,” they argue.

And they call for an alternative paradigm, “in which research focuses on better medicines for patients rather than for profits, where clinical trials with low risk of bias look for real benefits and faithfully report harms.”

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Notes to Editors:

Research: Trends in utilization of FDA expedited drug development and approval programs, 1987-2014: cohort study
<http://www.bmj.com/cgi/doi/10.1136/bmj.h4633>

Research: Characteristics of efficacy evidence supporting approval of supplemental indications for prescription drugs in United States, 2005-14: systematic review
<http://www.bmj.com/cgi/doi/10.1136/bmj.h4679>

Editorial: The FDA’s new clothes
<http://www.bmj.com/cgi/doi/10.1136/bmj.h4897>

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[Experts raise questions about FDA's use of expedited development, approval pathways for new drugs](#) - News Medical
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