

## **Early-stage drug trials often launched without solid evidence of clinical promise, researchers say**

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Pills. Credit: Public Domain

Standards for authorizing first-time trials of drugs in humans are lax, and should be strengthened in several ways, McGill University researchers argue in a paper published today in *Nature*.

While regulators in North America and Europe evaluate safety before human trials can proceed, they do not currently demand meaningful evidence for potential efficacy, write McGill bioethicist Jonathan Kimmelman and PhD student Carole Federico in a commentary article. "We believe that many (first-in-human) studies are launched on the basis of flimsy, under-scrutinized evidence."

Trials of ineffective therapies place burdens on society even if research participants aren't harmed directly, the researchers argue. Drug development soaks up financial and research resources; patients and healthy volunteers involved in testing a dud treatment miss out on more promising ones; and expenses wasted on ineffective therapies are often passed on to healthcare systems in the form of higher drug prices. The argument for better scrutiny of animal studies may be especially timely, since the incoming U.S. president has indicated he intends to weaken requirements for [clinical evidence](#) of efficacy before drugs are approved.

A clinical trial in France that led to the death of one person last year and hospitalization of five others has drawn intense scrutiny into how the drug's toxicity could have been anticipated, the researchers note. Yet ethical review boards—bodies at research institutions and universities that are set up to protect patients in clinical trials—seldom recognize that

they have a duty to evaluate whether an experimental treatment is promising enough to warrant human testing.

"Commercial interests and hope, alone, cannot be trusted to ensure that human trials launch only when the case for clinical potential is robust," says Kimmelman. "Ethics requires a clear-eyed evaluation of a drug's potential."

The McGill researchers propose several measures to reinforce standards, including:

- Require drugs sponsors to include negative results from animal studies in documents submitted to investigators and ethics committees;
- Allow trials to proceed only after careful vetting of the preclinical evidence by independent experts;
- Encourage reviewers to consider a broad base of evidence in assessing the probability that a drug will prove clinically useful: for example, how have other drugs in the same class performed in trials?

Critics of the proposal may object that this approach could increase costs and time for [drug development](#), the researchers note. But more-thorough assessments of clinical potential before [trials](#) could reduce failure rates, they say, and thereby offset development costs.

**More information:** Jonathan Kimmelman et al, Consider drug efficacy before first-in-human trials, *Nature* (2017). [DOI: 10.1038/542025a](#)

Provided by McGill University

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