

Proper evaluation, for patients' sake

Time is of the essence for a seriously ill patient, especially if the disease progresses rapidly. There are many good reasons to conduct comparative trials of a treatment in this situation, such as physiological considerations, observed effects in vitro or in animals, or anecdotal evidence of efficacy. Even when time is short, certain questions must be answered before large numbers of patients are exposed to a treatment outside the clinical trial setting. Could the treatment worsen the condition of some patients? Are its known or foreseeable adverse effects acceptable given its uncertain efficacy?

Sometimes, after early, fragmentary results justify further research, more robust data then demonstrate the drug's efficacy. This was the case for example with *abemaciclib* in breast cancer. Use of the drug thus becomes justifiable (see "Abemaciclib added to fulvestrant in inoperable or metastatic breast cancer" p. 177). But in other cases, fragmented initial data that appeared "promising" are followed by higher-quality evidence showing the drug to be ineffective. It is realised belatedly that patients had been exposed to a drug with no efficacy but with serious adverse effects. This happened with *olaratumab* in soft-tissue sarcoma, and the drug was finally withdrawn from the market worldwide in 2019 (see *Prescrire Int* n° 213 p. 82).

Proper drug evaluation, based on trials designed to provide sufficiently high-level evidence and conclusive results, using patient-relevant clinical outcomes, is the best way of ensuring that drugs are used judiciously, without causing unnecessary harm.

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