Validity of cost-effectiveness models based on randomized clinical trials

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Cost-effectiveness studies are widely used to guide prescribing policy in many countries, as part of health technology assessment programmes. However, a new study published this week in *PLoS Medicine* by Tjeerd-Pieter van Staa and colleagues suggests that cost-effectiveness analyses based on data from randomized controlled trials may not be realistic enough to accurately inform policy.

In the study the researchers (affiliated with the General Practice Research Database, and London School of Hygiene & Tropical Medicine in the UK, and Utrecht University in the Netherlands) specifically examine the relative cost-effectiveness of two commonly used groups of painkillers: selective cyclooxygenase-2 inhibitors (cox-2 inhibitors), versus conventional nonsteroidal anti-inflammatory drugs (NSAIDs). One type of cox-2 inhibitor has now been withdrawn from use, and recommendations regarding the use of others have changed as a result of data on cardiovascular harms associated with these drugs. The researchers collected data on the risk of upper gastrointestinal effects associated with use of either type of drug, and calculated the cost-effectiveness of the drugs in relation to these events using either data from large randomized controlled trials or from routine clinical practice - using the UK General Practice Research Database.

These analyses showed that the average cost of preventing an upper gastrointestinal effect by switching NSAIDs for a cox-2 inhibitor was calculated at roughly US$104K, using the data from routine clinical practice. However, the cost effectiveness was calculated as around

US$20K using randomized trial data.

The researchers suggest that in the specific scenario evaluated here, patients in the randomized trials experienced more gastrointestinal events than patients in routine practice, and that they used drugs differently - commonly involving higher daily doses - leading to a different estimate of cost-effectiveness. A more realistic assessment might have led to differences in prescribing guidelines, according to the authors. Tjeerd-Pieter van Staa says:

"Real-world questions on what to prescribe to whom should consider the varied group of patients in actual clinical practice rather than just using data from highly selective randomized trials that have narrow inclusion criteria."

However, one limitation of the study is that it involves an assessment of cost-effectiveness of two specific types of painkillers, and it isn't clear whether the observations will apply to cost-effectiveness for other drug classes.

More information: van Staa T-P, Leufkens HG, Zhang B, Smeeth L (2009) A Comparison of Cost Effectiveness Using Data from Randomized Trials or Actual Clinical Practice: Selective Cox-2 Inhibitors as an Example. PLoS Med 6(12): e1000194. doi:10.1371/journal.pmed.1000194

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