Think of the losers

Recommend the broad use of a cytotoxic drug with no selection criteria other than the site and stage of cancer is often an unreasonable option: serious adverse effects are more likely than tangible clinical benefits.

Certain drugs, such as gefitinib and crizotinib (examined on pages 261 and 264), are designed for patients who are selected on the basis of a genetic trait. These personalised treatments sometimes have a more favourable harm-benefit balance. But often, cancer progression halts for a few weeks and then resumes, without resulting in prolonged overall survival.

Occasionally one hears of spectacular, sustained responses after initiation of the treatment. These winners are rare, but seem to justify the use of the drug that appears to have induced this outcome.

But beware of observation bias. Let’s not forget the losers. If we observe remission, or even improved survival, in one patient, how can we tell that there are not also other patients who died sooner because of the drug’s adverse effects?

Comparative randomised trials allow us to go beyond empirical data and anecdotal reports, and to evaluate the overall harm-benefit balance, taking into account a diverse patient population. The aim of carrying out a clinical trial is to avoid many forms of bias.

When a treatment does not have a favourable harm-benefit balance in any group of patients, and we cannot identify with sufficient accuracy which patients are likely to benefit, they should be recommended to avoid this treatment. By not taking the gamble, a few patients will perhaps have lost an opportunity, but overall, there will be far more winners.