Prescrire International Editorial

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Mirage or reality?

Patients with serious diseases, particularly certain types of cancer, are often offered burdensome and aggressive treatment protocols, in the hope that these will be effective or have a high probability of being effective. In reality, these burdensome treatments are generally only beneficial in a minority of cases.

Patients differ, their cancers differ, and their responses to treatment also differ. How can we identify the handful of patients for whom an "aggressive treatment" will be more beneficial than harmful? And how can we avoid exposing the other patients to unjustified toxicity? Satisfactory solutions are hard to come by.

This state of affairs encourages the promotion of so-called personalised therapies based on pharmacogenetics (see pages 161-163). These therapies are claimed to target patients via genetic criteria to determine which patients are most likely to benefit from the treatment.

But beware of confusing tempting hypotheses with established facts, of confusing mirages with reality.

The genetic tests used cannot yet reliably identify which patients will benefit substantially from a given treatment. And personalised therapies target of course the tumour, but healthy organs as well, leading to specific adverse effects. The regulation of gene expression is a highly complex process. Overall, it is far from guaranteed that the benefits of "personalised" treatment will clearly outweigh its harms.

In practice, as of early 2013, when therapies of this type have been subjected to comparative clinical trials, the results have often been disappointing.

In the domain of so-called personalised medicine, just as in medicine in general, research, tests and medicines are ultimately paid for by society: patients and health professionals are therefore entitled to demand that such research be subject to regulation. With rigour and transparency, at every stage, and with access to the results. Choosing this path will encourage the arrival on the market of treatments, be they personalised or not, that are supported by solid arguments.

Pie in the sky, perhaps, but firmly grounded in reality.

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