Re: Demand Grows for Early Access to Promising Cancer Drugs

Providing patients with early access to new drugs for the treatment of cancer is a worthwhile objective. However, in reply to the news story by J. Baldwin published in a recent issue of the Journal (1), I would like to stress that there are real risks, both for patients and for physicians, associated with providing early access to promising cancer drugs outside of clinical trials. The rationale for requiring sufficient safety and efficacy data prior to allowing the commercial launch of a new chemical entity is that a “new drug” is not necessarily a more active and/or a less toxic drug than those currently available. Recent examples in oncology, which include new anti-angiogenic compounds and farnesyl transferase inhibitors, showed that an innovative drug may not live up to expectations (2). Gefitinib was also not as effective as expected when used in combination with conventional chemotherapy (2), and the initial enthusiasm raised by trastuzumab was counterbalanced by its cardiotoxicity (3). Thus, it is incumbent upon oncologists to explain to their patients that as long as the drug has not received authorization for licensing, data related to drug efficacy and toxicity are incomplete and inconclusive.

In France, health authorities can grant a temporary authorization to market compounds that might be active or beneficial for patients with a life-threatening condition. During this so-called temporary authorization of use (TAU) period, patients can have early access to new promising therapies, but, as mentioned by J. Baldwin (1), drug efficacy is not assessable within the TAU period and the patients benefiting from such early access cannot be included in clinical trials of the drug. Another risk associated with allowing very early access to a drug is that some pharmaceutical companies that provide this drug may take advantage of the situation by asking the patient-recipients of their products to put pressure on the health authorities for licensing approval. Moreover, during the TAU period, the price for the drug is generally fixed by the company at a high level and remains unchanged after the drug is licensed.

Caution is therefore needed before patients are offered a drug that is not licensed, because oncologists and patients may have an incomplete view of the drug’s efficacy and safety, which is sometimes conveyed by nonscientific journals or by the company that manufactured the drug. It would actually be more appropriate to let patients and physicians have access to the scientific information related to these promising compounds, i.e., the status of the clinical development of the drug, the title and status of the clinical studies performed with the drug, and data from the completed clinical trials of the drug. This data, instead of being kept secret by the health authorities, should be made accessible through a Web-based site so that oncologists and patients might form their own opinions about whether a drug is promising by considering information about the drug that is based on science, not on biased opinion. This information process would also encourage oncologists to include their patients in the clinical trials displayed on this Web site, thus shortening the time to complete the clinical development of the drug and speeding up its launch on the market.

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REFERENCES


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