How to recognize at an early stage whether a new cancer therapy prolongs life

May 11 2011

Great hopes are always placed in new cancer therapies. However, whether new surgical techniques, drugs or radiation therapies actually prolong the life of cancer patients, or even cure them, can often only be reliably assessed after several years' of testing. Researchers are therefore looking for proxy markers ("surrogate endpoints") that after initiation of cancer therapy can reliably predict as quickly as possible whether the treatment has a benefit.

A rapid report published by the Institute for Quality and Efficiency in Health Care (IQWiG) summarizes the findings of a search for literature on the validation of surrogate endpoints in breast and bowel cancer. The results are ambivalent. On the one hand, operational scientific methods are available to assess the validity of surrogate endpoints (surrogate=substitute). "We propose to apply these methods in the early benefit assessment of drugs," says Stefan Lange, IQWiG's Deputy Director.

However, on the other hand it has been shown that these methods have so far not been applied systematically, at least not in breast and bowel cancer. "For these common types of cancer it is therefore currently unclear which surrogate endpoints are suitable to predict a benefit of an intervention," says Lange. Consequently, patients often do not know what prospects of success a specific cancer treatment offers.

Predictions for the future

The ideal situation is a different one: cancer therapies should have proven in studies that they can prolong life and at the same time improve (or at least not seriously impair) quality of life. However, as such studies often last for years, cancer drugs are currently approved mostly on the basis of surrogate endpoints. In this context, for example, it is assessed in how many patients a new treatment stops the growth of a cancer or actually causes it to shrink.

"It is a good sign that a cancer is being contained in this way," says Lange, "but this is no guarantee that a treatment has a benefit." For example, in some women the drug Avastin® can contain breast cancer for a certain period of time. However, researchers now strongly doubt that this prolongs the life of the women. In view of the side effects that cancer therapies often have, patients could be spared therapies that have no benefit.

Impact on the early benefit assessment of cancer drugs

IQWiG assumes that for the early benefit assessment of new cancer drugs often only studies on surrogate endpoints will be available. "Consequently, conclusions on the benefit and harm of new drugs will be characterized by some degree of uncertainty" says Lange. "However, we now propose a way as to how this uncertainty can be detected and dealt with."

In order to apply the methods for assessing surrogate endpoints successfully, however, the Institute is dependent on the preparatory work of other researchers. Many long-completed studies on cancer therapies could easily be used to examine systematically the association between surrogate endpoints and the benefit for patients. "It would be ideal if companies and researchers rigorously analysed the surrogate endpoint

data currently lying in their drawers and published the results," says Lange.

Provided by Institute for Quality and Efficiency in Health Care