The FDA is undertaking a project to evaluate potential endpoints for cancer drug approval. The FDA will hold public workshops to identify important issues, and these issues will be discussed in meetings of the Oncologic Drugs Advisory Committee (ODAC). This site provides agendas, background materials, meeting transcripts, and links providing additional information on workshops and ODAC meetings. Workshop planning will be guided by a steering committee that includes representation from the FDA, the National Cancer Institute, the American Society of Clinical Oncology, and the American Association for Cancer Research. Workshop participants will include oncology experts, radiation oncologists, statisticians, industry representatives, and patient advocates. Workshops were held for three major cancers, lung cancer, colorectal cancer, and prostate cancer.

Each workshop summary includes general instructions on the FDA approval system (e.g., the definitions of regular approval and accelerated approval), general principles and FDA considerations concerning major endpoints for drug marketing approval, such as overall survival, time to progression, tumor response, tumor-related symptoms, and patient-reported outcomes (global Quality of Life).

There is a consensus that overall survival is the most suitable endpoint for drug approval for each cancer despite cross-over issues, however, discussion on other surrogate endpoints, such as time to progression and QOL, has not reached a consensus between cancer types. The FDA seems to be more reluctant to use time to progression as an endpoint for approval in lung cancer than it does in colorectal cancer. For colorectal cancer, in an adjuvant setting, the possibility of using 3-year disease-free survival as a surrogate for 5-year overall survival, based on data highlighting a close correlation between the two, has been intensively discussed, although a consensus has yet to be formulated. Conversely, QOL was more intensively discussed in the lung cancer workshop, while the discussion in the colorectal cancer workshop was completely negative.

It is noteworthy that the FDA leads the way in this kind of scientific discussion among academic oncologists, statisticians, and FDA officers in the USA, and that such discussion is publicly available at the FDA website. The Japanese regulatory agency does not currently conduct this kind of scientific activity.

Additionally, you can find a very interesting topic in the Lung Cancer Endpoints Workshop Summary. The comparison of approved drugs for non-small cell lung cancer (NSCLC) and small cell lung cancer (SCLC) from the USA, the European Union (EU) and Japan was discussed. Many people in Japan believe that fewer oncology drugs are commercially available in Japan compared with Western countries, however, it can be seen, from Tables 1 and 2 of the Workshop Summary, that in fact the opposite is true, at least for lung cancer. In Japan, eleven drugs have been approved for use in the treatment of NSCLC, five in the EU and six in the USA. Dr Puzdur, of the FDA, commented on the reason for this difference: "The Japanese drug regulatory system has many unique features including a lack of infrastructure to perform randomized studies, and a reluctance to accept foreign studies as relevant to the Japanese population. The Japanese medical care system is also very different from the US system. For example, in Japan oncology drugs are commonly administered by primary care physicians, internists, and other non-oncology specialists. For this reason, the Japanese system tends to focus more on establishing safety than on establishing efficacy." Anyone engaged in oncology drug development in Japan, including regulatory bodies, should be aware that the backwardness of Japan, with regard to cancer drug therapy, is being publicly discussed in the USA.

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