

臨床開発におけるバイオマーカーに期待するもの**

—産から—

朝野芳郎*

Prospects for of Biomarkers in Clinical Development

—Industry Perspectives—

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Summary

In recent years, much attention has been paid to the use of biomarkers in the evaluation of clinical studies in order to develop new drugs efficiently, because it might be possible to use biomarkers as a basis for go/no-go decision in the early stage of clinical development. Therefore, the literature was searched for reports of clinical studies using biomarkers, especially in pharmacokinetic/pharmacodynamic (PK/PD) studies, and the results were compared with the recommendations by Sheiner, *et al.*¹⁾. The implications for clinical development strategy in Japan are considered to be as follows.

Clinical development in the 21st century should more effectively use the knowledge obtained from the planning stage of examination and early-stage clinical trials, to allow a go/no-go decision to be made as quickly as possible. Means to convert qualitative evaluations into quantitative evaluation by modeling using biomarkers are needed to improve the success rate of clinical development.

1. In the exploratory stage, it will be necessary to use not only established biomarkers, but also candidate biomarkers that lack thorough validation. However, the regulator recognizes that there are risks and uncertainties in this approach, and careful judgement will be needed.

2. Companies should select a flexible development plan, such as an adaptive design, in the exploratory stage before seeking to demonstrate clear efficacy in a confirmatory study.

It is expected that clinical development guided by biomarkers will be useful particularly in the area of anti-cancer drugs, such as molecular-targeting drugs.

Key words

Biomarker, Endpoint, Pharmacokinetic/pharmacodynamic (PK/PD), Surrogate marker, Validate, Molecular target agent, Drug development