Characteristics of Drugs Approved in Japan without Conducting Confirmatory Clinical Trials

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Abstract

Aims: The aim of this study was to investigate the characteristics of drugs for which the requirement of confirmatory clinical trials for approval was waived in Japan. We also aimed to identify factors and formulae to predict the waiver of confirmatory clinical trials. Methods: Data on approved drugs and their characteristics were mainly extracted from the Japan Pharmaceuticals and Medical Device Agency database. The seriousness of the disease, existence of available treatments, and number of patients were considered as candidate factors. The influence of each factor on receiving a waiver was determined using logistic regression analysis comparing drugs approved with and without confirmatory clinical trials as the binary response variable. The predictive formula was derived from the results of the logistic regression analysis. A receiver operating characteristic curve was used to evaluate the accuracy of the prediction. Results: The drugs categorised as anti-neoplastic agents, use of the cost accounting method in the drug pricing system, 'orphan' designation, and Accelerated Approval designation in the United States emerged as significant factors in the logistic regression analysis, predicting a waiver for confirmatory clinical trials ($P < 0.001$). These factors were then used to establish a predictive model to ascertain whether confirmatory clinical trials would be necessary for a new drug, exhibiting good sensitivity (0.754) and specificity (0.785), and high accuracy for newly approved drugs. Conclusion: The identification of key factors that can predict waivers of confirmatory clinical trials may accelerate the development of clinically important drugs and improve patient access globally.

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