

## 希少疾病用医薬品の開発経験に関する アンケート調査に基づくその開発促進策に関する検討

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Consideration of Ways of Encouraging  
Development of Orphan Drugs Based on a Questionnaire Survey

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### Summary

In order to consider ways of encouraging development of orphan drugs, including so-called ultra-orphan drugs, a questionnaire survey addressed to pharmaceutical companies that had recently obtained marketing approval for orphan drugs in Japan was conducted. Responses were received for 105 drugs. Concerning the timing of orphan drug designation, several drugs were designated before the commencement of clinical trial or during Phase 1 in Japan, but they were already under development in the US or European countries in most cases. Many responses pointed out the benefits of "priority review" and "longer re-examination (data protection) period" among several possible measures for encouraging development of orphan drugs. Good communication with the regulatory body, flexible clinical development plan, and improvement of the company's image were also cited as benefits. Review of the designation criteria and their operation, a flexible mechanism for the clinical data package/review process, and consideration of appropriate drug pricing were pointed out as necessary matters for promoting development of orphan drugs. The survey results should serve as useful reference data when considering ways of encouraging development of orphan drugs in the future.

### Key words

Orphan drugs, Orphan drug development, Rare diseases