in study design and the availability of evidence for value demonstration. Systematic reviews and retrospective database studies investigating the efficacy and safety of orphan drugs are supportive care, which is less expected against the new therapy, were generally expected by most of the respondents. Cost-effectiveness analysis was required in some of the countries, with the rest expecting only a budget-impact analysis based on local epidemiological data. Findings were similar for therapies for diseases with low prevalence but without orphan drug designation. CONCLUSIONS: Unmet needs in rare diseases are high, and effective new therapies are welcomed and valued by payers in these key reimbursed markets in Asia. Decision makers are willing to show a degree of flexibility in their evidence requirements for these kinds of products.

METHODS: We reviewed literature using the Ichushi database (Japanese medical literature database) and the database on government-funded research projects, and also contacted investigators and experts for related information. RESULTS: Four guidelines were identified: Shiragami (2004) and Kamea (2007) groups, funded by the Ministry of Health and Welfare, Japan, proposed two guidelines on pharmaceutical pricing. The task force of the Ministry of Economy, Trade and Industry, Japan (2007) proposed one guideline on medical device policy. The Hiota group (2011), funded by the Ministry of Health and Welfare, Japan, proposed one guideline on vaccination strategy. Although the headings and structures of all guidelines were almost similar, significant differences were identified among them. For example, two guidelines recommended a societal perspective, while the other two recommended a consumer’s perspective. In terms of outcome measures, QALYs were preferred in three guidelines, whereas one recommended “the proportions of patients who achieved target clinical results within 2 years.” Trial use was not conducted to determine the feasibility of any guidelines, except for the Hiota guideline for vaccination policy. In addition, some recommendations had a serious problem in terms of scientific rationality. CONCLUSIONS: There are significant variations in the key features among all four abovementioned guidelines, and even between the two sets of guidelines for pharmaceutical pricing decisions. To use an economic evaluation to aid rational resource allocation, official guidelines should be established with scientific rigor and integrity, and future discussions about feasibility are needed among various representatives from government, academia and industry.

HEALTH CARE USE & POLICY STUDIES - Health Care Research & Education

METHODS: We reviewed literature using the Ichushi database (Japanese medical literature database) and the database on government-funded research projects, and also contacted investigators and experts for related information. RESULTS: Four guidelines were identified: Shiragami (2004) and Kamea (2007) groups, funded by the Ministry of Health and Welfare, Japan, proposed two guidelines on pharmaceutical pricing. The task force of the Ministry of Economy, Trade and Industry, Japan (2007) proposed one guideline on medical device policy. The Hiota group (2011), funded by the Ministry of Health and Welfare, Japan, proposed one guideline on vaccination strategy. Although the headings and structures of all guidelines were almost similar, significant differences were identified among them. For example, two guidelines recommended a societal perspective, while the other two recommended a consumer’s perspective. In terms of outcome measures, QALYs were preferred in three guidelines, whereas one recommended “the proportions of patients who achieved target clinical results within 2 years.” Trial use was not conducted to determine the feasibility of any guidelines, except for the Hiota guideline for vaccination policy. In addition, some recommendations had a serious problem in terms of scientific rationality. CONCLUSIONS: There are significant variations in the key features among all four abovementioned guidelines, and even between the two sets of guidelines for pharmaceutical pricing decisions. To use an economic evaluation to aid rational resource allocation, official guidelines should be established with scientific rigor and integrity, and future discussions about feasibility are needed among various representatives from government, academia and industry.