THE MARKET ACCESS LANDSCAPE FOR ORPHAN DRUGS IN APAC: A COMPARISON OF POLICIES IN FIVE MAJOR MARKETS

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Introduction

- Manufacturers of orphan products have historically faced challenges in securing reimbursement due to (typically) high prices and insufficient clinical trial data.
- Despite this, however, the number of orphan drugs being approved continues to increase year-on-year in many of the world's largest markets.[1] For example, the US Food and Drug Administration (FDA's) Centre for Drug Evaluation and Research (CDER) reports to have approved twice as many orphan drugs in the eight years from 2012-2019 than in the previous eight years.
- Moreover, orphan drugs represent an increasing proportion of global prescription drug sales.[2]
- In this light, we sought to outline policies related to market access for orphan drugs in the Asia Pacific (APAC) region.

Objective

- We sought to identify the regulations and policies related to market access for orphan drugs in five major markets from the APAC Region, with the aim of providing an overview of the factors designed to support sponsors of orphan medicinal products. Specifically, we focused on policies in Australia, China, Japan, South Korea and Taiwan.

Methods

- We reviewed factors related to the approval, pricing and reimbursement of orphan drugs in Australia, China, Japan, South Korea and Taiwan.
- Relevant information was identified from the websites of the competent authorities in each market and from the academic and grey literature (published as at February 2020).

Results

All five markets have implemented measures to encourage the development and/or marketing of orphan drugs and support access, while four out of five countries (i.e. all but China) have implemented processes for designation of orphan drugs. The accepted definition of a rare disease varies from country-to-country. The legislative instruments regarding orphan drugs and the definitions of a rare disease applicable in each region can be found in Figure 1 (below).

Figure 1. Relevant legislation and definition of rare disease for Australia, China, Japan, South Korea and Taiwan



China. Legislation. Rare Disease List (2018). **Rare disease defined as that affecting:** less than 1 per 500,000 population.[3]*

South Korea. Legislation. Rare Disease Management Act (2015), amendment to Pharmaceutical Affairs Act (2001). **Rare disease defined as that affecting:** Less than 20,000 people in Korea (i.e. <4 per 10,000 population).[4]

Japan. Legislation: Amendment to Pharmaceutical Affairs Law (1993). **Rare disease defined as that affecting:** Less than 50,000 people in Japan (i.e. <4 per 10,000 population).[5]

Taiwan. Legislation: The Rare Disease and Orphan Drug Act (2000). **Rare disease defined as that affecting:** less than 1 per 10,000 population.[6]

Australia. Legislation: Orphan Drugs Programme (since 1997), via amendment to the Therapeutic Goods Act. **Rare disease defined as that affecting:** less than 5 per 10,000 population.[7]

*These are not official definitions of a rare disease in China, but rather a consensus reached by experts from the Genetics Branch of the Chinese Medical Association in 2010.[3] Under current official documentation related to China's Rare Diseases List, rare diseases include those for which "there is international and domestic evidence that the incidence and prevalence is low."[8] However, no quantitative criteria are provided.

Figure 2. Summary of types of supporting policies in place for each country

	Australia	China	Japan	South Korea	Taiwan
DATA EXCLUSIVITY	✗	✗	✓	✓	✓
PRICING	✗	✗	✓	✓	✓
HTA	✗	✗	✓	✓	✗
REIMBURSEMENT	✓	✓	✓	✓	✓

Data Exclusivity: Japan, South Korea and Taiwan provide extended data exclusivity for orphan drugs. Notably, in Japan and South this is provided in the form of an extended "re-examination period" of up to 10 years (in both countries) as there is no data exclusivity per se.[9,10] In Taiwan, orphan drugs may benefit from 10 years of data exclusivity (compared to 3-5 years for other new drugs).[4]

Pricing: In Japan, a marketability premium of 5%, or 10-20%, is available for orphan drugs,[11] while in Taiwan the price of an orphan drug can be set at the median of the prices in the countries used for international reference pricing (IRP) (compared to the lowest price among IRP countries for other products).[6] In South Korea, orphan products with a price equal to or lower than the weighted average price of alternative drugs are exempt from price negotiation, which is otherwise required for all new drugs.[12]

Health Technology Assessment (HTA): Japan and South Korea have implemented specific HTA arrangements for orphan products. In Japan, a higher QALY threshold applies for orphan drugs,[13] while in South Korea such drugs are eligible for a cost-effectiveness waiver, although a risk-sharing agreement may be required.[14]

Reimbursement: In all regions studied, specific reimbursement policies have been implemented for orphan/rare disease drugs to some degree. In Australia, orphan drugs that cannot be reimbursed under the Pharmaceutical Benefits Scheme (PBS) may be included in the Life Saving Drugs Programme (LSDP),[15] for which 100% reimbursement is provided. In China, additional reimbursement for specified orphan drugs may be available at the provincial level.[16] In Japan, while orphan drugs may be reimbursed under National Health Insurance (NHI), drugs for "intractable diseases" (though not necessarily orphan drugs) may be further subsidised.[17] In South Korea, orphan drugs incur a reduced co-payment (10%),[14] while in Taiwan, 80-100% of the cost of orphan drugs may be subsidized depending on the patient's status.[18]

Conclusions

- All five markets studied have implemented measures to encourage the development and/or marketing of orphan drugs and support access.
- However, the breadth and scope of such policies across markets varies substantially, presenting specific access challenges in each region.

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