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IMPACT OF NATIONAL ORPHAN DRUG POLICY AND REIMBURSEMENT MECHANISMS OVER THE IMPLEMENTATION OF MANAGED ENTRY AGREEMENTS IN SELECT ASIA-PACIFIC COUNTRIES

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INTRODUCTION

- Orphan drug legislation has been passed in several Asia-Pacific countries, including Australia, Japan, South Korea, Singapore and Taiwan, outlining the pathway for drug registration and approval, and enabling reimbursement through specific funding mechanisms
- Given that the clinical evidence of orphan drugs is often uncertain at product launch and that these treatments generally have high costs, innovative reimbursement approaches such as managed entry agreements (MEAs) may be of interest to national healthcare payers
- The objective of this study is to evaluate whether current national orphan drug policy and existing reimbursement mechanisms in these countries support the implementation of MEAs for orphan drugs

METHODOLOGY

- Non-systematic review of published literature, from 2010 onwards, performing a web-based search as well as PubMed, for key words including: "orphan drug policies" and "managed entry agreements" in Asia-Pacific countries
- Reviewed articles were in languages including English, Japanese, Korean and Mandarin
- Revision of approval and funding regulations for orphan drugs in publically available health authority and patient association websites: PBS and TGA in Australia, PMDA and MHLW in Japan; HIRA and KORD in South Korea; HSA in Singapore and NHIA in Taiwan

RESULTS

- Orphan drug legislation and independent market authorization for orphan drugs are in place in the geographies within scope (figure 1); there are similar financial and non-financial incentives to encourage the availability and access to orphan drugs in these countries
- Reimbursement pathways allow for partial or full funding for treatment at present; current orphan drug legislation and reimbursement schemes in place do not explicitly contemplate access through MEAs in most countries, except Australia

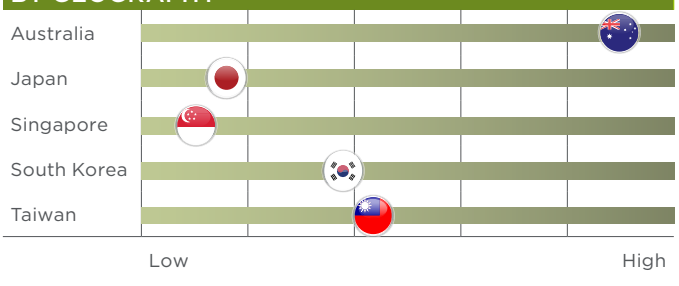
FIGURE 1: SUMMARY OF ORPHAN DRUG POLICY AND REIMBURSEMENT MECHANISMS

Country	Orphan Drug Policy	Orphan Drug Pricing & Reimbursement
Australia	• Prevalence threshold for orphan disease designation: 0.9 in 10,000 • FI to ensure access to orphan drugs: fee reduction for marketing authorization approval • NFI: pre-licensing access, regulatory assistance	• Fixed pricing • Funding under the highly specialized drugs program for orphan drugs • Reimbursement under the life-saving drug program for ultra-orphan drugs; no co-payment applies
Japan	• Prevalence threshold for orphan disease designation: <3.9 in 10,000 • FI: subsidies, tax credits, 10 year marketing exclusivity • NFI: priority review, fast track approval, free protocol assistance	• Fixed pricing • 100% funding (30% from insurance companies, 70% from national/regional governments)
Singapore	• Prevalence threshold: 37.7 in 10,000 • FIs: 10 year marketing exclusivity • NFI: priority review and fast track approval	• Free pricing – orphan drug legislation remains to be activated • Reimbursement decisions made by the Centre for Drug Administration
South Korea	• Prevalence threshold: <4.0 in 10,000 • FI: funding for national research programs, exclusive marketing rights for 6 years • NFI: exemption of certain orphan drugs from economic evaluations	• Fixed pricing • Full reimbursement available for select orphan drugs
Taiwan	• Prevalence threshold for orphan disease designation: <1 in 10,000 • FI: grants, fee reductions, 10 year marketing exclusivity • NFI: regulatory assistance	• Fixed pricing • 70% to 100% (for low income families) reimbursement

Abbreviations: FI: Financial incentives; NFI: Non-financial incentives

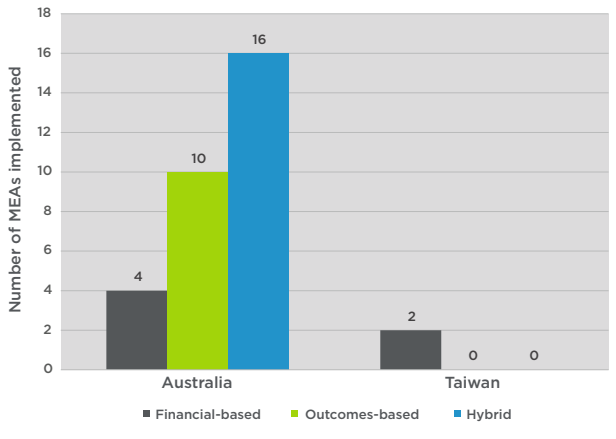
- Experience with MEA implementation varies across countries (figure 2), with Australia being the most experienced where MEAs have been in place for years, including financial and outcomes-based agreements (particularly "conditional treatment continuation schemes")

FIGURE 2: LEVEL OF EXPERIENCE WITH MEAs BY GEOGRAPHY



- In Japan, financial-based MEAs include price cuts that are attached to forecasted spending, but rare diseases are exempt from this; outcomes-based agreements have not been implemented for orphan drugs
- In Singapore, MEAs for orphan drugs have not been implemented at present, and healthcare authorities would rather wait to assess the experience in other geographies prior to implementing locally
- In South Korea, there is experience with price-volume agreements for a limited number of drugs in indications with large populations (e.g. hypertension). Conditional treatment continuation schemes are in place for select oncology treatments, but not for orphan drugs; concerns remain around the high administration costs of outcomes-based MEAs and how to address failure (e.g. delist drug or cut its price)
- In Taiwan, there is experience with price-volume agreements; outcomes-based MEAs have also been of interest, but no agreement of this type has been implemented for orphan drugs
- Regarding implementation of MEAs for orphan drugs (figure 3), Australia is the country with the highest number of agreements implemented to date, with 30 agreements in place. These are mainly hybrid agreements, with a financial and outcomes-based component
- In Taiwan, two price volume agreements have been implemented for orphan drugs; no MEAs for orphan drugs were identified in Japan, Singapore and South Korea

FIGURE 3: ORPHAN DRUG MEAs IDENTIFIED IN COUNTRIES WITHIN SCOPE



CONCLUSIONS

- Experience with and formal recognition by healthcare authorities appear to incentivize the implementation of MEAs in the context of orphan drugs. This is evidenced by Australia, where a high level of experience with MEAs has translated into a considerably higher number of agreements for orphan drugs
- In Japan, a favorable reimbursement mechanism in place for orphan drug access, limited MEA experience and a preference for basic financial agreements (e.g. price cuts) as opposed to outcomes-based agreements, may be pre-empting the need to consider MEAs for orphan drugs at present
- In countries such as Singapore, South Korea, and Taiwan where funding for orphan drugs is partial or limited to select drugs, there is an interest in considering MEAs, but health authorities remain hesitant to implement outcomes-based schemes due to high administration costs, conflicts of interest and uncertainty on how to address failure; authorities would prefer to watch and learn from experiences abroad with such schemes prior to implementing locally

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