

Orphan Drugs in Asia-Pacific: From designation to pricing, funding & market access

Donald Macarthur

www.justpharmareports.com

It is estimated that 6-10% of the world's population will suffer from a rare disease at one point in life. On this basis, the markets of Asia-Pacific should offer great potential to companies developing orphan drugs. The region's population is 4 billion, nearly two-thirds of the global total, so even the rarest disease will have many patients seeking treatment.

But clinical need rarely correlates with affordability, which dictates what is actually spent on healthcare. A major new report **Orphan Drugs in Asia-Pacific: From designation to pricing, funding and market access** focuses on five developed markets in the region: Japan, South Korea, Taiwan, Singapore and Australia. Each has passed specific measures to promote the development, registration and/or commercialisation of orphan drugs. Singapore's Medicines (Orphan Drug) Exemption Order of 1991 was the first legislation anywhere to follow the US Orphan Drug Act, with the other four Asia-Pacific countries also implementing orphan-promoting measures ahead of the EU.

The report describes the processes for obtaining orphan product designation, what can be omitted from the standard dossier to obtain marketing authorisation, and provides full details of the regulatory bodies and timelines involved. The main emphasis though is not on registration but on market access, which for products like orphan drugs that often carry premium prices, means eligibility for reimbursement or other sources of public funds.

Based on extensive research, including fieldwork in Tokyo and Seoul in November 2009 to capture detail, understanding and all the latest trends, the report includes:

- The incentives on offer to orphan drug sponsors.
- How P&R processes work specifically for orphan drugs.
- The role of HTA and risk sharing as levers to obtain reimbursement.
- Where special funding schemes operate and how to access them.
- 13 case studies on the P&R history of 12 different orphan drugs in 5 countries.
- How much patients are expected to contribute to the cost of their treatment.
- Listings of designated and approved orphan drugs.
- Comparisons between countries with orphan drug policies and those without.
- Contact details of the main rare disease patient organisations.

Learn which of the Asia-Pacific countries ...offers the most comprehensive programme for the management of rare disease...often enjoys higher orphan drug prices than the US...has the most orphan drugs on the market and reimbursed...promotes the development of rare disease medical devices as well as drugs...where 'named patient' imports are discouraged by rare disease patient representatives.

Buy or order your copy now at www.justpharmareports.com

Table of Contents

Executive Summary

1. Introduction

2. Japan

- 2.1 Healthcare and Health Insurance System
- 2.2 Orphan Drug Legislation
 - 2.2.1 Orphan Drug Development Programme, 1993
 - 2.2.2 Qualifying Criteria
 - 2.2.3 Procedure/Timetable
 - 2.2.4 Incentives
 - 2.2.4.1 R&D Support
 - 2.2.4.2 Protocol Assistance
 - 2.2.4.3 Expedited Regulatory Review
 - 2.2.4.4 Lower Regulatory Fees
 - 2.2.4.5 Market Exclusivity
- 2.3 Results
 - 2.3.1 Designation
 - 2.3.2 Marketing Approval
- 2.4 Pricing & Reimbursement
 - 2.4.1 Process for New Drugs
 - 2.4.1.1 Price Comparators
 - 2.4.1.2 Price Premiums
 - 2.4.1.3 'Me-toos'
 - 2.4.1.4 Cost Calculation
 - 2.4.1.5 Foreign Price Adjustment
 - 2.4.2 Health Economic Considerations
 - 2.4.3 Timetable
 - 2.4.4 Price Revision Process
 - 2.4.5 Outcome
- 2.5 Funding for Orphan Drugs
- 2.6 Prescribing Controls
- 2.7 Patient Co-payment
- 2.8 Distribution
- 2.9 Patient Access
 - 2.9.1 Unapproved Products
 - 2.9.2 Marketed Products
- 2.10 Case Studies
 - 2.10.1 Remicade
 - 2.10.2 Thaled
- 2.11 Rare Disease Organisations
- 2.12 Pending Reforms

3. Korea

- 3.1 Healthcare and Health Insurance System
- 3.2 Orphan Drug Legislation
 - 3.2.1 Qualifying Criteria
 - 3.2.2 Procedure/Timetable
 - 3.2.3 Incentives
 - 3.2.3.1 R&D Support
 - 3.2.3.2 Protocol Assistance
 - 3.2.3.3 Expedited Regulatory Review
 - 3.2.3.4 Market Exclusivity
- 3.3. Results
- 3.4 Pricing & Reimbursement
 - 3.4.1 Process for New Drugs
 - 3.4.2 Health Economic Considerations
 - 3.4.3 Timetable
 - 3.4.4. Price Revision Process
 - 3.4.5 Outcome
- 3.5 Funding for Orphan Drugs

- 3.6 Prescribing Controls
- 3.7 Patient Co-payment
- 3.8 Patient Access
- 3.9 Korea Orphan Drug Center
- 3.10 Case Studies
 - 3.10.1 Erbitux
 - 3.10.2 Fuzeon
 - 3.10.3 Glivec
- 3.11 Rare Disease Organisations
 - 3.11.1 Genetic and Rare Diseases Center
 - 3.11.2 Korean Organisation for Rare Diseases

4. Taiwan

- 4.1 Healthcare and Health Insurance System
- 4.2 Orphan Drug Legislation
 - 4.2.1 Qualifying Criteria
- 4.3 Incentives
 - 4.3.1 R&D Support
 - 4.3.2 Protocol Assistance
 - 4.3.3 Expedited Regulatory Review
 - 4.3.4 Lower Regulatory Fees
 - 4.3.5 Market Exclusivity
- 4.4. Results
- 4.5 Pricing & Reimbursement
 - 4.5.1 Process for New Drugs
 - 4.5.2 Health Economic Considerations
 - 4.5.3 Price Revision Process
- 4.6 Funding for Orphan Drugs
- 4.7 Prescribing Controls
- 4.8 Patient Co-payment
- 4.9 Patient Access
- 4.10 Case Study: Zavesca in Niemann-Pick Disease Type C
- 4.11 Rare Disease Organisations

5. Singapore

- 5.1 Healthcare and Health Insurance System
- 5.2 Orphan Drug Legislation
 - 5.2.1 Qualifying Criteria
 - 5.2.2 Procedure/Timetable
- 5.3 Incentives
- 5.4 Pricing & Reimbursement
- 5.5 Distribution
- 5.6 Compassionate Use

6. Other Asian Countries

- 6.1 Philippines
- 6.2 Thailand
 - 6.2.1 Case Study: Glivec

7. Australia

- 7.1 Healthcare and Health Insurance System
- 7.2 Orphan Drug Legislation
 - 7.2.1 Qualifying Criteria
 - 7.2.2 Procedure/Timetable
- 7.3 Incentives
 - 7.3.1 R&D Support
 - 7.3.2 Protocol Assistance

- 7.3.3 Expedited Regulatory Review
- 7.3.4 Lower Regulatory Fees
- 7.3.5 Market Exclusivity
- 7.4 Results
- 7.5 Pricing & Reimbursement
 - 7.5.1 Process for New Drugs
 - 7.5.2 Criteria Employed
 - 7.5.3 Health Economic Considerations
 - 7.5.4 Price Revision Process
- 7.6 Funding for Orphan Drugs
 - 7.6.1 Highly Specialised Drugs Programme
 - 7.6.2 Life Saving Drug Programme
- 7.7. Patient Co-payment
- 7.8 Patient Access
- 7.9 Case Studies
 - 7.9.1 Agrylin
 - 7.9.2 Duodopa
 - 7.9.3 Tracleer
- 7.10 Rare Disease Organisations

8. New Zealand

- 8.1 Healthcare and Health Insurance System
- 8.2 Orphan Drug Provisions
- 8.3 Pricing & Reimbursement
- 8.4 Funding for Orphan Drugs
- 8.5 Patient Co-payment
- 8.6 Patient Access
- 8.7 Rare Disease Organisations

List of Tables

- 1.1 Basic statistics on selected countries in Asia-Pacific
- 1.2 Results of first five years of orphan drug legislation by country/region
- 2.1 Orphan drug/device designations and approvals in Japan (1993-2008)
- 2.2 Orphan drug designation in Japan by origin of sponsor
- 2.3 Orphan drug designation in Japan by main disease area target
- 2.4 Authorised orphan drugs by leading therapeutic area, US, EU
- 2.5 Orphan drugs granted marketing approval in Japan, FY 2005-08
- 2.6 Average time from first marketing in world to local marketing
- 2.7 Regulatory approval dates with orphan drugs, Japan, US, EU
- 2.8 Price premiums for new drugs in Japan
- 2.9 Best-selling orphan drugs in Japan, 2008
- 2.10 Diseases in Japan with patient co-payment ceiling
- 3.1 Reimbursement listing success rates in Korea, pre- and post-DERP
- 3.2 Patient co-payments in Korea, 2009
- 3.3 Products procured/supplied by KODC, first half 2009
- 3.4 P&R history of Glivec in Korea, 2001-2009

Glossary of Abbreviations and Acronyms

Appendices

- 1. Cross-national comparison of orphan drug policies
- 2. Korean-designated orphan drugs with marketing authorisations
- 3. Taiwan-designated orphan drugs with marketing authorisations
- 4. Australian-designated orphan drugs and those with marketing authorisations
- 5. International comparison of manufacturer selling prices of specimen orphan drugs