

Orphan Drugs in Europe: Pricing, Reimbursement, Funding and Market Access Issues

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In order to raise public awareness, 29 February 2008 was dedicated the first European rare disease day ('a rare day for very special people'), but European payers need no reminding of the significance of rare disease treatments, generally known as orphan drugs.

Eight years earlier, EU Regulation 141/2000 was enacted to encourage R&D in rare diseases and increase orphan drug output. By any standard the Regulation has been a major success. Almost 800 designation applications have been made, with 44 orphan medicines from 31 sponsoring companies receiving pan-European marketing approval. An estimated 1.6 million patients with 38 different rare diseases have potentially benefited, and the surge of interest in the area has offered hope to many more. Orphan drugs accounted for 23% and 12% of all new marketing applications to the EMEA in 2006 and 2007 respectively..

Rare diseases and their treatments may still be emotive territory, with a strong patient voice and genuine unmet need, but the pendulum is swinging from a guarantee of 'special status' when it comes to public reimbursement to issues of budgetary impact and cost effectiveness – not only because of the high cost of orphan drugs themselves, but the growing number of target diseases and treatments, associated new costs of medical education and diagnosis, and the need for chronic disease management.

Questions answered by this report include:

- What is the actual in-market status of all EU designated orphan drugs with marketing authorisations up to the beginning of 2008?
- Which drugs are covered by reimbursement, which are not?
- Are special funds available for orphan drugs?
- Where is health technology assessment required?
- How large is the European price corridor for orphan drugs?
- Is there a relationship between target patient population and price?
- How do orphan drug policies differ across the leading European countries?
- Do payers view all orphan drugs as unique?

This is what one highly informed reviewer thought of Donald Macarthur's earlier report on orphan drugs, published by *Scrip Reports* in 2000:

"The author's previous orphan drug report was an extremely useful instrument for Swedish Orphan to fully understand the global opportunities within the orphan disease and orphan drug area, and did serve as a stimulus to get the company started. I am convinced that this latest report equally will serve as a very useful

document for academia with interest in drug development, small start-ups as well as medium-sized corporations with interest in orphan diseases and their treatment.”

Lars-Uno Larsson, CEO, Swedish Orphan

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Table of contents

Executive Summary

1. Introduction

1.1 What are Orphan Drugs?

1.2 What are the P&R Issues with Orphan Drugs?

2. EU Orphan Drug Regulation

2.1 Objectives

2.2. Qualifying Criteria

2.3 Procedure/Timetable

2.4 Incentives

2.4.1 Information

2.4.2 R&D

2.4.3 Protocol Assistance

2.4.4 Marketing Approval Assistance

2.4.4.1 Priority review/fast track

2.4.4.2 Lower regulatory fees

2.4.5 Market Exclusivity

2.4.5.1 Exclusivity withdrawn?

2.5 Comparison with US Orphan Drug Act

2.6 Results

2.6.1 Designation

2.6.2 Marketing Approval

2.6.3 Medical Benefit

3. Compassionate Use

4. Paediatric Regulation

5. Market Access

6. Pricing and Sales

6.1 Public Prices

6.2 Manufacturer Selling Prices

6.3 Sales

7. Distribution

8. Advantages and Disadvantages of Orphan Designation

8.1 Pros

8.1.1 Incentives

8.2 Cons

8.2.1 Most National Incentives Still Apply

8.2.2 Some Help from EU Still Available

8.2.3 Limitation to Market Exclusivity

8.2.4 Initial fee Waivers May Not be Permanent

8.2.5 Information Disclosure to Authorities

8.2.6 Information Disclosure to Competitors

8.2.7 No Regulatory Flexibility

9. Future EU Developments

9.1 New Communication

9.2 Common HTA, Single EU Price for Orphan Drugs?

10. National Situation

10.1 EU-5

10.1.1 Germany

10.1.1.1 Orphan incentives

10.1.1.2 Patient access

10.1.1.3 Funding provisions

10.1.1.4 Impact on P&R

10.1.1.5 Health economic considerations

10.1.2 France

10.1.2.1 Orphan incentives

10.1.2.2 Patient access

10.1.2.3 Funding provisions

10.1.2.4 Impact on P&R

10.1.2.5 Health economic considerations

10.1.3 UK

10.1.3.1 Orphan incentives

10.1.3.2 Patient access

10.1.3.3 Funding provisions

10.1.3.4 Impact on P&R

10.1.3.5 Health economic considerations

10.1.4 Italy

10.1.4.1 Orphan incentives

10.1.4.2 Patient access

10.1.4.3 Funding provisions

10.1.4.4 Impact on P&R

10.1.4.5 Health economic considerations

10.1.5 Spain

10.1.5.1 Orphan incentives

10.1.5.2 Patient access

10.1.5.3 Funding provisions

10.1.5.4 Impact on P&R

10.1.5.5 Health economic considerations

10.2 Other European Countries

10.2.1 Austria

10.2.3 Belgium

10.2.4 Bulgaria

10.2.5 Czech Republic

10.2.6 Ireland

10.2.7 Netherlands

10.2.8 Poland

10.2.9 Portugal

10.2.10 Slovak Republic

10.2.11 Sweden

10.2.12 Other Nordic Countries

10.2.13 Switzerland

11. Pricing Case Studies

11.1 Chronic Myeloid Leukaemia

- 11.2 Pompe Disease
- 11.3 Pulmonary Arterial Hypertension
- 12. Conclusions
- 13. Strategic Implications

List of tables

- 1.1 Level of evidence in selected orphan drug appraisal dossiers
- 2.1 Examples of orphan medicines predating the orphan drug Regulation
- 2.2 Advice to orphan drug sponsors from EMEA
- 2.3 Orphan drug regulations compared
- 2.4 Overview of EU procedures for orphan drug designation and approval
- 2.5 EU designated orphan drugs with centralised marketing authorisations
- 2.6 National marketing authorisations granted for EU-designated orphan drugs
- 2.7 Main therapeutic classification of approved orphan drugs
- 2.8 EU designated orphan drugs with previous non-orphan use
- 4.1 Marketed orphan drugs with supporting data in paediatric populations
- 5.1 European availability of orphan drugs by product
- 5.2 European availability of orphan drugs by country
- 5.3 First launch quarters for centrally authorised orphan drugs
- 5.4 Reimbursement status of Aldurazyme across EU
- 6.1 Public prices of EU orphan drugs
- 6.2 Estimated manufacturer selling prices of orphan drugs (EU-5, Swiss & US)
- 7.1 Main distribution channels for orphan drugs
- 10.1 ZE rates for Glivec in Germany
- 10.2 ASMR scores for orphan drugs in France
- 10.3 Status of orphan drugs in Scotland after SMC review
- 10.4 Status of orphan drugs in Wales after AWMSG review
- 10.5 Costs per QALY for ultra orphan drugs in UK
- 10.6 Certified rare disease centres in Italy
- 10.7 Treatment costs with selected orphan drugs in Italy
- 10.8 Reimbursement status of orphan drugs in Belgium
- 10.9 Actual reimbursed expenditure on orphan drugs in Belgium
- 10.10 Intramural and extramural orphan drugs reimbursed in the Netherlands
- 10.11 NFZ points value and MSP of reimbursed orphan drugs in Poland
- 10.12 Swedish cost effectiveness estimates for orphan drugs
- 10.13 Swiss orphan drugs with simplified marketing authorisations

List of figures

- 6.1 Estimated manufacturer selling prices across Europe for Glivec
- 6.2 Orphan target population vs monthly adult treatment cost