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Market Access for Orphan Drugs: assessing the global landscape

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Abstracts

Their development costs can be high and the market is relatively small, yet orphan drugs represent a significant contribution to public health—and to pharmaceutical market values.

In fact, analysts predict that orphan drugs will account for 15 percent of the global pharmaceutical market by 2018. Yet getting there will not be easy. While governments offer incentives for development, payers must also balance the needs of the majority with the high cost of orphan drugs. International and regional regulations are also hurdles to be overcome, despite harmonisations efforts by agencies.

In Market Access for Orphan Drugs: Assessing the Global Landscape, FirstWord goes around the world to lay bare the market access issues affecting orphan drug developers in the US, Asia and Europe. Based on thorough research and European national drug policies translated specifically for FirstWord, the report reviews the challenges, offers insight into strategies being used and includes invaluable comparative tables. Filled with information on trends, patient access schemes and the role of patient organisations, the report is a must-read for orphan drug developers and manufacturers.

Key Report Features

Comparative tables for orphan drug polices and incentives in the major markets

A look at the role of patient organisations and registries in orphan drug development

Pricing and reimbursement information for key markets

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Overview of orphan drug trends by year, company and therapeutic focus

Key Facts

By 2020, the recently-formed International Rare Disease Research Consortium aims to be able to diagnose most rare diseases and to have developed 200 new therapies

Biomarkers and improved clinical diagnostics are increasing the possibility of developing targeted rare disease therapies

Currently, there are 6,000 to 8,000 rare diseases, with genetic research identifying more

Although the prevalence of rare disease is extremely low, they affect 30 million people in the EU

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