

Orphan Drug Market Access: Payer Insights on the Present and Future

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Abstracts

Orphan Drug Market Access: Payer Insights on the Present and Future is a report that examines the regulation, pricing, reimbursement, players and stakeholder/payer interests that are shaping the current and future direction of the orphan drug sector.

The number of orphan drug approvals is growing and access for patients is wide. There is little by way of price constraint with cost per patient year exceeding US\$300,000 in some cases. Orphan drugs bring benefits for critically ill patients and profit for companies.

Can the good times last?

There is fear from healthcare payers that the rising number of products, treatment cost and unrestricted access is unsustainable. Many orphan drugs are label extensions of mainline treatments while better understanding of the genetic basis of disease is naturally creating ever smaller patient populations for highly-targeted therapies. As genetic research advances, it is possible to imagine a time when the majority of new drugs would meet orphan drug criteria. Orphan Drugs are part of the clinical landscape and all stakeholders have valid interests which have to be addressed now.

Orphan Drug Market Access: Payer Insights on the Present and Future offers insights from US and European healthcare payers and includes illustrative case studies that highlight key aspects of the orphan drug market.

Key Benefits

Profile leading markets in respect to your orphan drug projects and know the

points of difference in their pricing/reimbursement mechanisms and regulatory requirements.

Identify which therapy areas are driving growth in the orphan drug space and what that means for your business and plans.

Competitively evaluate the companies most active in the field in terms of revenue performance and research.

Address the rising and legitimate concerns of healthcare payers in US and Europe about cost, pricing policies, value for money, indication expansion and the lack of positive engagement with industry.

Practically plan for likely strategic challenges in orphan drugs such as funding caps and HTA assessments.

Get Answers to Critical Questions

What does the regulatory environment in the US and Europe look like?

What is the current and future market value?

What therapy areas are of importance?

What is the pricing and reimbursement structure for orphan drugs in US, France, Germany, Italy, Spain and the UK?

What are the payers' views and how can industry positively respond to rising concerns around cost, clinical data and involvement in the research process?

Which future market developments may pose challenges? (ie: risk sharing, HTA assessment, a less benign view of funding, post marketing cost benefit justification and the influence of patient advocacy groups.)

Experts Interviewed

UK payer – Advisor to NHS organisations (e.g. CCGs)

UK payer - Head of medicines optimisation for a large CCG, UK

Germany payer – Pharmacist

Italy payer – Pharmacist on Hospital Formulary Committee and Regional Formulary Committee

Spain payer – Director of Hospital Pharmacies, Member of Medication of High Impact Committee, Chief of the Pharmacy, Chief of the Pharmacy Therapeutic Committee

France payer – Pharmacist

US payer – Executive vice-president of pharmacy for national US PBM

US payer – Professor of Medicine, health policy researcher and lawyer with a special interest in orphan drugs

Key Quotes

“There’s a central committee with a president and a vice-president, and groups of medical experts for oncology and other fields. As soon as new drugs are released, we first ask the experts for their opinion. They tell us what they think about the new products compared to the existing treatments. This opinion is then sent to the central committee that then discusses whether or not to add the product to our formulary.” Pharmacist, France

“We’re going to have to start looking at different risk scenarios with pharmaceutical manufacturers,” Executive Vice President of pharmacy for national PBM, US

“We get very little notification from them. We get very little detail about what’s coming to market. It’s generally a reactive process rather than something we seek out proactively.” Head of medicines optimisation, CCG, UK

“It’s always helpful if we see data or we see summaries where it’s clear this new drug is working ... In my eyes that is very critical in the discussion at the moment.

Nobody really talks about how much money can we save and how many people are cured by this new drug”. Pharmacist, Germany

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7. CONTRIBUTORS

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