Orphan Drugs: Pricing and Market Access Strategies
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Orphan Drugs: Pricing and Market Access Strategies

Orphan drug market access and pricing: strategies for success

When it comes to securing market access and drug pricing for orphan drugs, a one size approach won’t work.

Driven by increasing competition and cost pressures, securing market access now requires developers to take an increasingly sophisticated approach when dealing with payers and HTA organisation. By combining disease information, patient input, real world data and innovative low risk contracting with the wider social and health benefits companies can – and must – build robust value propositions to justify pricing.

Orphan Drugs: Pricing and Market Access Strategies is an expert report that reveals the latest thinking on the proven strategic approaches that will deliver market access and pricing success in the increasingly complex and challenging US and EU5 orphan drug sector.

Key Features

In-depth interviews which reveal the knowledgeable insights of leading executives from companies such as Pfizer, Shire and AstraZeneca

SWOT of orphan drug market access

Graphs defining orphan drug market drivers

Taxonomy of companies in the orphan drug space from pioneers to ultra-rare disease pure play companies

Case studies including how Alexion gained support from NICE for Soliris and PTC Therapeutics Managed Access Agreement for its nmDMD treatment Translarna

Summary of key criteria underlying HTA decisions for access to orphan drugs

At-a-glance summaries of all the key insights.

Key Benefits

Understand the internal and external drivers for orphan drug developers

Review models defining the approach orphan drug developers are taking

Harness the influence of patients for clinical research design and proving value to payers

Develop solid pricing strategies by establishing key stakeholder benefits and contextualising prices to meet local conditions and budgets

Learn how to develop enhanced dossiers that go beyond clinical data to demonstrate undeniable cost effectiveness and value

Understand the benefits of orphan drug market exclusivity regulations.
Key Questions Answered By This Report:

Focus: Why is it critical for companies to really understand rare diseases and associated patient or sub patient groups when planning orphan drug development?

How Rare? Why is understanding the opportunities and challenges in rare verses ultra-rare disease critical to developers?

Inventive Dossiers: How can pharma enhance often limited clinical evidence with RWD and patient-reported outcomes to create persuasive value dossiers for payers?

Understanding Payers: What do payers really want and how can an appreciation of budget impact and cost pressures influence pharma’s pricing policy and strategy?

Fragmented EU: There may be centralised drug approvals in the EU but pricing and reimbursement are national issues – what do you need to know to secure market access?

Pricing Principles: What pricing principles do Celgene and Aegerion apply when establishing an orphan drug price strategy?

Sharing Risk: How – and when – might risk sharing schemes such as Managed Access, Managed Entry, Conditional Approval and Patient Access Programmes secure market access for orphan drugs?

Expert Views

Juan Ambriz: Access Manager and Orphan Drugs at Pfizer
Nicola Bonner: Associate Director at Adelphi Values
Tom Croce: Head of Global Patient Advocacy at Shire Pharmaceuticals
Usman Iqbal: Senior Medical Affairs Leader—Neuroscience, Global Medical Affairs at AstraZeneca
David Jakouloff: Head of Global Market Access at Aegerion Pharmaceuticals (Views made in this report are his personal opinion and do not represent the views of Aegerion Pharmaceuticals.)
Ramya Logendra: Senior Consultant, Global Supplier & Association Relations at IMS Health
Angela McFarlane: Market Development Director at IMS Health UK and Ireland
Sheela Upadhyaya: Associate Director of Highly Specialised Technologies at the National Institute for Health and Care Excellence (NICE)
Michael Zaiac: Head of Medical Affairs Haematology/Oncology EMEA at Celgene
Anonymous: The personal opinions of a recent FDA employee

Key Quotes

“Evidence of post-marketing patient benefits, payer re-evaluation and commercial reward for the company should be a continuum. It cannot be just a one-shot assessment at the time of marketing authorisation given the
significant payer budgets involved.”

David Jakouloff
Head of Global Market Access at Aegerion

“One way to address payer pushback is to assess and demonstrate some benefit enrichment criteria within your patient population – where you are able to provide some sub-group of interest that has the highest unmet need and would benefit the most from the drug both in terms of efficacy as well as economics.”

Usman Iqbal
Senior Medical Affairs, AstraZeneca

“I think where it gets tricky is when you go to other countries and look at the payment system and at the level of challenge for getting coverage for products. It becomes a question mark or a potential obstacle.”

Tom Croce
Head of Global Patient Advocacy at Shire Pharmaceuticals

Who will benefit from this report?

Market access professionals needing to secure orphan drug market entry
HEOR teams building value and evidence portfolios for orphan drugs
Brand/KAM teams charged with presenting commercial propositions to payers for orphan drugs
Regulatory professionals needing to secure orphan drug designation and market exclusivity
Payers having to negotiate pricing and reimbursement with companies
HTA experts reviewing the safety, efficacy and value of orphan drugs
Patient groups wanting to widen market access and availability.

Content Highlights

Drivers of Growth in Orphan Drugs
The Orphan Drug Environment
Internal and external drivers of the orphan drug market
The burden of ultra-rare diseases
The key players in the growing orphan drug market
SWOT analysis
Key success factors

The Challenges in Commercialising Orphan Drugs
The Orphan Drug Pricing and Market Access Landscapes in the US and the EU

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Recruitment issues and sparse clinical trial data
Market uptake and treatment management issues
Proving cost-effectiveness
The Health Technology Assessment challenge
The orphan drug market in the EU
The orphan drug market in the US
Country-specific decision-making
Exclusivity strategies in the US and EU
Pricing Considerations and Models
Pricing principles
Pricing and trust
Risk sharing
A focus on budget impact
Opportunities to Ensure Orphan Drug Market Access
Offering high clinical benefits in a niche segment
Finding evidence in real world data
Deeper payer engagement and better value propositions
Enhanced patient engagement
Engaging other stakeholders
Ensuring the Positive Future of Orphan Drugs
More innovations in preventive medicine
Clear illustration of expandable value
Price rationalisation and future financing
2017 and beyond

Need more information? Contact a consultant for an executive summary and sample pages from the report.

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Publisher : FirstWord Pharma
Reference :
Number of Pages : 0
Report Format : PDF
Publisher Information : 

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