

# Effective Sales and Marketing Strategies for Orphan Drugs

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## Abstracts

### **The real potential for orphan drug pharma marketing**

With drug pipelines moving from essential products to niche therapies, large pharma companies are increasingly attracted to the prospective high demand and low competition of orphan drug markets. However, the 'sales and marketing' models for orphan drugs are distinctly different to conventional models for common medications. So the transition to this specialist field is a lot more challenging than it appears.

A new report from FirstWord Dossier – Effective Sales and Marketing Strategies for Orphan Drugs – examines the effectiveness of strategic business models that big pharma are using to market these products. It draws upon the in-depth opinions of 13 experts from the orphan drugs industry, patient organisations, and regulatory authorities in rare diseases.

### **Report Overview**

Effective Sales and Marketing Strategies for Orphan Drugs summarises the main issues, challenges and tactics involved in launching new pharma products for rare diseases. The report compares the critical differences between marketing orphan vs non-orphan drugs – from initial drug development through to commercialisation. It also discusses the huge potential of this field and the key factors driving new product awareness and approval.

### **Key Report Features**

Overview of pharma's current role in the rare diseases market

Orphan drug business models: specialist biotech vs big pharma

Distinct differences in orphan drug marketing vs common drugs

Clinical and medical challenges in developing orphan drugs

Practical ways to gain valuable patient organisation support

Rare disease indication strategies to maximise commercial return

Benefits of Managed Access Programmes (MAPS) for drug developers

Case studies of various pharma companies' orphan drug launches

## **Key Benefits**

Review the evolving rare diseases marketplace

Identify the potential of different business models

Manage the challenges specific to orphan drug marketing

Recognise the key factors that influence commercial success

Assess the pharmacoeconomic benefits of orphan drugs

Build key stakeholder alliances across the entire drug lifecycle

Use social media and websites as marketing tools

## **Key Questions Answered**

How do orphan drug business models differ from standard models?

What are the US and European incentives for orphan drug development?

What strategies did GSK use to enter the rare diseases market?

Which issues should you consider before orphan drug investment?

How can you initiate and encourage corporate-patient collaborations?

Can an orphan drug ever become a blockbuster?

What can MAPs offer orphan drug developers?

How can you effectively target physicians about rare diseases?

### **Who Would Benefit From This Report?**

This report will be of value to pharma directors and managers with responsibilities in the following areas:

Market access

Marketing management

Business development

Brand marketing

Sales management

Pharmacoeconomics

Clinical research development

Corporate communications

Key Opinion Leader Liaison teams

Patient Advocacy Liaison teams

Medical affairs

This report will also benefit patient group organisations and charities, as well as healthcare legislators and health policy researchers.

**Expert Views Include:**

Robert Derham, Founder of CheckOrphan

Abdul Mullick, Head of Global Marketing, Genetic Diseases, Genzyme

Craig Kephart, President, CEO of Centric Health Resources

Marc Dunoyer, Head of Rare Diseases Unit, GlaxoSmithKline

Steve Aselage, Executive VP and Chief Business Officer, BioMarin

Dirk Moritz, VP Global Strategic Marketing, Shire Human Genetic Therapies

Stephen C. Groft, Director, Office of Rare Diseases, National Institutes of Health

Hans Schikan, Chief Executive Officer of Prosensa

Nick Sireau, Chairman of the AKU Society

Simon Estcourt, Senior VP Strategy and Corporate, Idis

Maryze Schoneveld van der Linde, founder of Patient Centered Solutions

Theresa Heggie, Senior VP of Global Commercial Operations, Shire Human Genetic Therapies

Tony Hall, Chief Medical Officer at PSR

**Key Quotes**

“If you’re not doing the pre-marketing activity for orphan drug launches you’re going to fail. It will cost you a lot more and you’re not going to have a real defined patient

population,” – Robert Derham, founder of CheckOrphan.

“There’s an advantage to being first in your orphan drug market if you do it right. Other products may seem to work as well but the difference is in your programme,” – Craig Kephart, CEO of Centric Health Resources.

“Some big pharma are trying to make a business out of orphans but this isn’t something that comes naturally to them...they have to change their mind set to go into a market like this,” – Steve Aselage, executive VP, BioMarin.

“Orphan drugs appear superficially as a very lucrative space: rare diseases, few competitors ...but this takes time and persistence,” – Abdul Mullick, head of global marketing, genetic diseases, Genzyme.

“More than any other area, the advocacy that patient organisations bring to this area is really important, and these relationships start very early in drug development,” – Theresa Heggie, senior VP, global commercial operations, Shire Human Genetic Therapies

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Legislation and incentives

The rare diseases market

Orphan drug indications

Patient focus

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### **IS THE POTENTIAL OF THE ORPHAN DRUG MARKET ENOUGH TO ATTRACT INVESTMENT?**

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Market exclusivity in the EU

Protocol assistance

Fee reductions

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Development of drugs for rare diseases compared to drugs for more common diseases

Optimizing the orphan drug networks to maximize commercial return

Orphan drug business models: specialist biotech versus big pharma

### **WHAT ARE THE MAJOR CHALLENGES TO THE ORPHAN DRUG DEVELOPMENT PROCESS AND BUSINESS MODEL?**

Medical and clinical challenges to orphan drug development

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Clinical trial challenges
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- Harmonization of clinical trial processes between the EU and US
- Pricing and reimbursement
- Openness between pharma and patients

## **'SALES AND MARKETING' STRATEGY: WILL AN ORPHAN DRUG EVER BECOME A BLOCKBUSTER?**

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- Drug for a rare disease applied to a common disease
- Re-purposing

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- The interface between patient organizations and industry
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  - EURORDIS and NORD
  - Mutual benefits
  - Patient Registries

Natural history registry  
Product registry  
The Global Rare Disease Registry and Data Repository (GRDR)  
European Centres of Expertise and EUROPLAN  
How patients can drive research and drug development in rare diseases

## **WHAT CAN MANAGED ACCESS PROGRAMMES OFFER COMPANIES DEVELOPING ORPHAN DRUGS?**

When to use a MAP  
Benefits of a Managed Access Programme  
Access to medicines  
Data  
Stakeholder engagement  
Market development  
Market testing pre-approval (between post-Phase III and first submission)  
Ensuring continuity of patient supply  
Access in certain countries post-launch

## **REGULATIONS AND NATIONAL PLANS FOR MANAGED ACCESS PROGRAMMES**



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