

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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EVOLUTION OF THE ORPHAN DRUG MARKET

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

Medical and clinical challenges to orphan drug development Diagnosis Disease understanding

Effective Sales and Marketing Strategies for Orphan Drugs



Case study 1: Fabry Outcome Survey Clinical trial challenges Case study 2: Endpoints stall AKU clinical trial

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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?

Optimizing the company-physician relationship in the orphan drug space Case study 3: Prolastin - An integrated healthcare management model of sales and marketing Disease and product familiarization amongst physicians and patients Diagnosis

'Sales and marketing' infrastructures

Targeting physicians in rare diseases

Competition in the orphan drug market

Social media in communications and the marketing mix

RARE DISEASE INDICATION STRATEGIES

New molecular entity (NME) for unmet medical need Drug for a rare disease applied to a common disease Re-purposing

HOW CAN COMPANIES COLLABORATE WITH PATIENT ORGANIZATIONS ACROSS THE ORPHAN DRUG LIFECYCLE TO THE MUTUAL BENEFIT OF BOTH?

Motivated patients and patient organizations The interface between patient organizations and industry Patient organizations 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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